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Strategies To Enhance NIH-Funded Pediatric Research

Optimizing Child Health

Phyllis Dennery, Frederick Rivara, Alexis Wojtowicz, and Udara Perera, *Editors*

Committee on Strategies to Enhance Pediatric Health Research Funded by NIH

Center for Health, People, and Places

Consensus Study Report

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This Consensus Study Report was reviewed in draft form by individuals chosen for their diverse perspectives and technical expertise. The purpose of this independent review is to provide candid and critical comments that will assist the National Academies of Sciences, Engineering, and Medicine in making each published report as sound as possible and to ensure that it meets the institutional standards for quality, objectivity, evidence, and responsiveness to the study charge. The review comments and draft manuscript remain confidential to protect the integrity of the deliberative process.

We thank the following individuals for their review of this report:

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Although the reviewers listed above provided many constructive comments and suggestions, they were not asked to endorse the conclusions or recommendations of this report nor did they see the final draft before its release. The review of this report was overseen by **RONALD KLEINMAN,** Massachusetts General Hospital for Children and **DAN G. BLAZER,** Duke University. They were responsible for making certain that an independent examination of this report was carried out in accordance with the standards of the National Academies and that all review comments were carefully considered. Responsibility for the final content rests entirely with the authoring committee and the National Academies.

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Preface

Thanks in no small part to research funded by the National Institutes of Health (NIH), and particularly the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), the mortality of infants, children, and adolescents in the United States has decreased dramatically since the turn of the 20th century. Beyond this, since the turn of the 20th century we have witnessed myriad other remarkable advancements in health and medical research that have dramatically altered the quality and length of the lives of infants, children, and adolescents. The numbers speak for themselves: Since 1900, the percentage of children born alive who die before their first birthday has decreased by nearly half in the United States. Over the last 75 years, children with cystic fibrosis have gone from rarely surviving past age 5 to having a median life expectancy of 61 years. Fewer than 10 percent of children with acute lymphocytic leukemia survived 60 years ago; now, at least 90 percent will survive for 5 years or more. More recently, infant mortality decreased in the United States nearly 25 percent between 1999 and 2022.

Advances in sanitation, nutrition, public health, health care, and more have occurred thanks to the rigorous and incremental march of scientific discoveries, building one on another through sharing of knowledge and through collaboration across disciplines and nations. This scientific research would have been impossible to conduct without the support of the NIH, which since 1887 has in some form funded research ranging from basic molecular insights to research that impacts whole communities. As the world's largest funder of health and basic biomedical research, it has involved hundreds of thousands of scientists and staff at NIH and in our

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nation's academic centers, children's hospitals, research organizations, and industry while engaging millions of individuals as research participants.

Yet, there is so much more to be done, particularly for our nation's youth. In the United States, while declines in child and adolescent mortality have occurred across all races, the rate in Black youth remains twice that of white youth; firearm-related deaths of all kinds among adolescents are increasing; and mental, emotional and behavioral disorders among children and adolescents are more prevalent than ever. In addition, many chronic diseases of adulthood have antecedents in infancy or childhood. Adverse childhood experiences, exposure to chronic and toxic stress, and childhood hypertension and dyslipidemia are just a few antecedents linked with the onset of cancer, cardiovascular disease, depression, diabetes, and hypertension in adulthood. Pediatric research provides cornerstone knowledge and understanding about the causes of these issues, as well as their prevention and treatment.

The current scrutiny that NIH is undergoing may result in the most substantial changes in the organization since its official establishment in 1930. In preparing this report, which was mandated by Congress and commissioned by NIH before these current considerations, the committee took seriously its charge to examine NIH's current pediatric research portfolio and structure and make recommendations focused on improving NIH's overall support of pediatric research. The committee strived to balance celebrating the progress made in pediatric research supported by NIH by identifying where meaningful changes could be made in its approach. The committee also recognized the importance of acknowledging the reality of NIH's financial and staff capacity while still identifying longer-term goals and possibilities. As such, some of the committee's recommendations can be implemented with relatively quick actions, while others may take more time and a wider range of stakeholders to fully implement.

The committee is grateful to the U.S. Congress and NICHD for supporting this work. This report lays out a framework for how pediatric research should be situated and prioritized within NIH, emphasizing a life course approach, so as best to return dividends in health for our future generations of youth and adults. Pediatric research must continue, building on the enormous knowledge gained from the last century of research. This is necessary, not just for the health of our nation's youth, but for everyone.

Fredrick P. Rivara, *Co-Chair*
Phyllis Dennery, *Co-Chair*
Committee on Strategies to Enhance
Pediatric Health Research Funded by NIH
December 2025

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Acronyms and Abbreviations

ABCD	Adolescent Brain Cognitive Development
ACE	adverse childhood experience
ALPS	Antenatal Late Preterm Steroid
BRDPI	Biomedical Research and Development Price Index
CC	NIH Clinical Center
CHRCDA	Child Health Research Career Development Award
CFP	call for perspective
CIT	Center for Information Technology
CKID	Chronic Kidney Disease in Children study
CRIC	Chronic Renal Insufficiency Cohort
CSR	Center for Scientific Review
CTSA	Clinical and Translational Science Awards
ECHO	Environmental Influences on Child Health Outcomes
ENRICH	Early Intervention to Promote Cardiovascular Health of Mothers and Children
eRA	Electronic Research Administration
FDA	Food and Drug Administration
FIC	Fogarty International Center
FY	fiscal year

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GMS	grant management specialist
GMO	grant management officer
HBCD	healthy brain and child development
HEAL	NIH Helping to End Addiction Long Term
ICs	institutes and centers (at NIH)
IDDRC	Intellectual and Developmental Disabilities Research Center
IOM	Institute of Medicine
IRB	institutional review board
IRP	NIH Intramural Research Program
NCATS	National Center for Advancing Translational Sciences
NCCIH	National Center for Complementary and Integrative Health
NCI	National Cancer Institute
NEI	National Eye Institute
NHGRI	National Human Genome Research Institute
NHLBI	National Heart, Lung, and Blood Institute
NIA	National Institute on Aging
NIAAA	National Institute on Alcohol Abuse and Alcoholism
NIAID	National Institute of Allergy and Infectious Diseases
NIAMS	National Institute of Arthritis and Musculoskeletal and Skin Diseases
NIBIB	National Institute of Biomedical Imaging and Bioengineering
NICHD	Eunice Kennedy Shriver National Institute of Child Health and Human Development
NIDA	National Institute on Drug Abuse
NIDCD	National Institute on Deafness and Other Communication Disorders
NIDCR	National Institute of Dental and Craniofacial Research
NIDDK	National Institute of Diabetes and Digestive and Kidney Diseases
NIEHS	National Institute of Environmental Health Sciences
NIGMS	National Institute of General Medical Sciences
NIH	National Institutes of Health
NIMH	National Institute of Mental Health
NIMHD	National Institute on Minority Health and Health Disparities
NINDS	National Institute of Neurological Disorders and Stroke
NINR	National Institute of Nursing Research
NLM	National Library of Medicine
NOSI	notice of special interests
NPeRC	NIH Pediatric Research Consortium

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OD	NIH Office of the Director
PCTRC	Pediatric Clinical and Translational Research Center
PEHSU	Pediatric Environmental Health Specialty Unit
PFAC	patient and family advisory council
PHN	Pediatric Heart Network
PHACS	Pediatric HIV/AIDS Cohort Study
PI	principal investigator
PO	program officer
PRO	patient-reported outcome
PROMIS	Patient-Reported Outcomes Measurement Information System
PRSP	Pediatric Research Strategic Plan
RADx	Rapid Acceleration of Diagnostics
RCDC	Research, Condition, and Disease Categorization
RECOVER	Researching COVID to Enhance Recovery
RePORT	NIH Research Portfolio Online Reporting Tools
RePORTER	RePORT Expenditures and Results
RFA	request for application
SRO	scientific review officer
UDP	NIH Undiagnosed Diseases Program

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Summary[1]

Pediatric health is the cornerstone of the nation's well-being. Increases in the U.S. average lifespan during the 20th century were in great part due to declines in child mortality from infectious diseases, public health, prematurity, trauma, cancer, sanitation, and other health issues. These advances would never have happened without the significant and consistent federal investment in pediatric research, supported as a whole or in part by the National Institutes of Health (NIH).

Children today have more opportunities for health and longevity than at any point in history. Yet rising rates of chronic conditions, including obesity, anxiety, and other health challenges, signal that this progress is uneven and fragile. As a leader in supporting pediatric research, NIH has an opportunity to illuminate how rapidly evolving exposures and experiences shape child health trajectories. Through pediatric research investment, NIH can generate actionable knowledge that empowers families, clinicians, and communities to foster resilience, prevent disease, and optimize lifelong health. This report offers recommendations for how NIH, with the support of Congress, can best support research in pediatric health and improve the health of American children and individuals throughout the life course.

Children who grow up without the burden of chronic disease are more likely to thrive academically, socially, and professionally. Improving the health of all our children and adolescents will contribute to the development of the next generation of scientists, engineers, teachers, artists, and

[1] This summary does not include references. Citations to support the text and recommendations herein are provided in the main text.

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leaders who will drive the next wave of American progress. A healthy generation of children is the foundation of a resilient, innovative, and secure nation, directly influencing the state of the nation's future workforce, military readiness, economic productivity, and societal stability.

STUDY TASK AND APPROACH

NIH tasked the National Academies of Sciences, Engineering, and Medicine (the National Academies) with convening an ad hoc consensus committee to examine NIH's pediatric research portfolio and structure, as was mandated by Congress in 2024, along with examining:

- the methods and rationale used in categorizing projects as pediatric research according to the NIH's Research, Condition, and Disease Categorization (RCDC) process;
- how pediatric components have been included or excluded from larger NIH initiatives;
- structural or process impediments to pediatric research applicants;
- how pediatric research priorities are established within and across NIH institutes and centers (ICs);
- how pediatric research activity is coordinated across ICs; and
- ways in which the NIH Clinical Center could be used to advance innovative pediatric research.[2]

The health of children is foundational to having a healthy, thriving society composed of healthy individuals across the life course. Health status changes across the life course, molded by biological, psychological, and social transitions, life's turning points, physical and social exposures, and lived experiences. During the arc of the human life course, health is acquired, developed, optimized, sustained, and ultimately lost. Thus, health is emergent, relational, and ever-changing, rather than a fixed state. This perspective is at the core of the life course developmental approach. The committee adopted this approach as a lens to understand child health and pediatric research and to shape the committee's vision and goals for pediatric health research in the United States and for developing the recommendations for improving the NIH's support of pediatric research.

[2] The complete Statement of Task can be found in Chapter 1.

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PEDIATRIC HEALTH RESEARCH AT NIH: PAST AND PRESENT

NIH is the world's largest public funder of biomedical research. Nearly all ICs led by The Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) fund pediatric research. The NIH Intramural Research Program (IRP), which accounts for 11 percent of its budget, also conducts pediatric research.

For nearly 100 years, consistent investment by the federal government has made NIH a global leader in scientific discovery and medical breakthroughs. NIH funding has supported numerous advances, including the development of the polio vaccine and tests for congenital disorders resulting from chromosomal abnormalities and genetic mutations. These breakthroughs created a groundswell of national interest in understanding and improving the health of children and led to the formation of NICHD in 1962. One of NICHD's early priorities was to understand the impact of health care, the environment, family and community systems on the healthy development of children, leading to advances in the prevention and treatment of neonatal conditions, novel treatments for childhood cancers, effective vaccines against infectious diseases, and more.

THE CURRENT CRISIS IN CHILD HEALTH

Despite advances in health and medicine, there is broad consensus among scientists, clinicians, and policy makers that child health is in crisis. Children in the United States are experiencing high incidences of chronic diseases and poor mental, emotional, and behavioral health. U.S. children are 80 percent more likely to die than their peers in European nations. The impacts of negative or positive exposures in early life are cumulative, so adults who experience poor health or adversity in childhood have substantially higher rates of a variety of costly chronic health conditions. Social and economic factors such as poverty, food insecurity, and poor access to health care also result in health inequities that imperil the health and future of many American children. A robust and coordinated NIH pediatric research portfolio is needed to address this multifaceted child health crisis.

THE CURRENT PEDIATRIC RESEARCH PORTFOLIO AT NIH

The committee used NIH Office of Budget data from fiscal year (FY) 2015 to FY 2024 to determine recent trends in pediatric research funding. In FY 2024, NIH spent $6.19 billion on pediatric research, of which 10 percent was intramural and 90 percent extramural. From FY 2015 to FY 2024, pediatric research expenditures increased by 70 percent, while NIH-wide total research expenditures increased by 62 percent. However, adjusted for inflation using the Biomedical Research and Development Price Index, growth in pediatric research and NIH-wide research expenditures flattened

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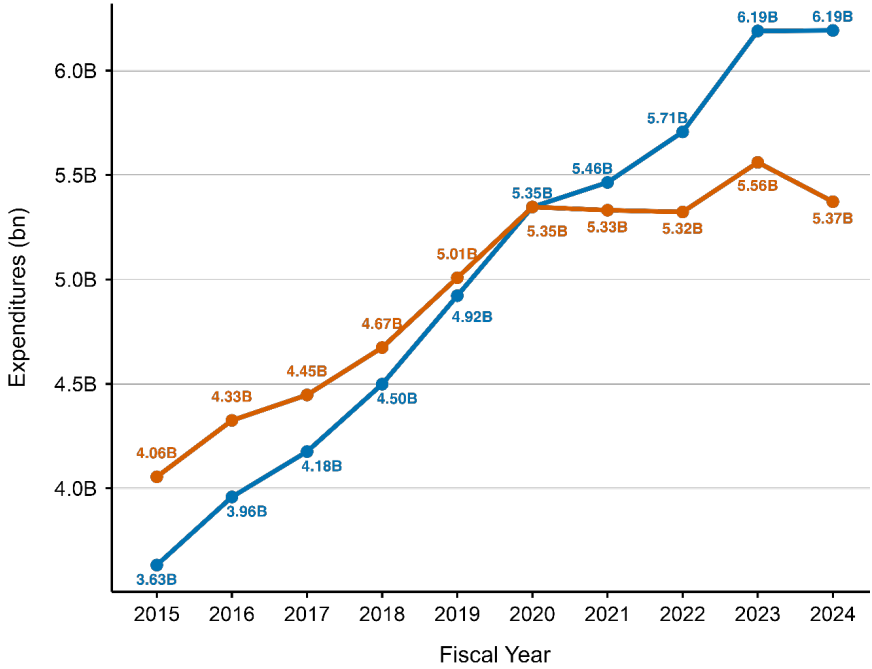

FIGURE S-1 Pediatric research expenditures, FY 2015–FY 2024.
NOTE: RCDC = Research, Condition, and Disease Categorization.
SOURCE: Committee's analysis of data from the RCDC system, using the Biomedical Research and Development Price Index for inflation adjustment.

from FY 2020 through FY 2024 (see Figure S-1). Pediatric research has consistently accounted for 13 percent of the non-administrative NIH budget over the past decade (see Figure S-2), a smaller share than the 22 percent of the U.S. population represented by children.

All NIH ICs fund some amount of pediatric research, but five ICs—NICHD, National Cancer Institute (NCI), National Heart, Lung, and Blood Institute (NHLBI), National Institute of Mental Health (NIMH), and National Institute of Allergy and Infectious Diseases (NIAID)— account for the majority of those expenditures, with NICHD accounting for 17 percent of total pediatric expenditures across NIH. Across the 10-year period, the five health condition categories with the largest pediatric research expenditures were genetics, neurology, mental health, infectious diseases, and neonatal/perinatal. No broad pediatric health condition category was funded exclusively by a single IC, although a significant majority of research on childhood cancers and craniofacial/oral conditions were funded by NCI and the National Institute of Dental and Craniofacial Research, respectively.

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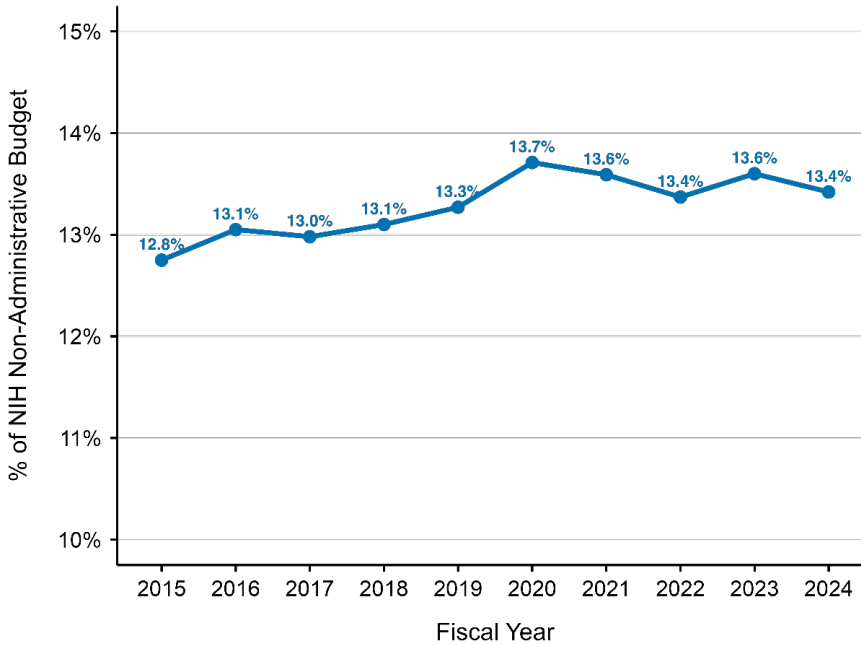

FIGURE S-2 Pediatric research expenditures as a proportion of NIH non-administrative expenditures, FY 2015–FY 2024.
NOTE: NIH = National Institutes of Health; RCDC = Research, Condition, and Disease Categorization.
SOURCE: Committee's analysis of data from the RCDC system and data from NIH Office of Budget.

Although every IC funded some pediatric research, there was wide variation in the level of funding (see Figure S-3). Aggregating the total pediatric research funding across FY 2015–FY 2024, ICs were categorized into five tiers (bars). Tier 1 included a single institute, NICHD, which funded the plurality of research, accounting for 17 percent of NIH's pediatric research expenditures. Tier 2 ICs included NCI (11 percent), NHLBI (10 percent), NIMH (10 percent), and NIAID (9 percent). Combined, Tier 1 and Tier 2 ICs accounted for the majority (57 percent) of pediatric research funding. The proportion of each IC's total research budget spent on pediatric research varied widely, and the diversity of ICs that fund pediatric research suggests a continued need for NICHD leadership, which houses a substantial amount of pediatric expertise that other ICs can use.

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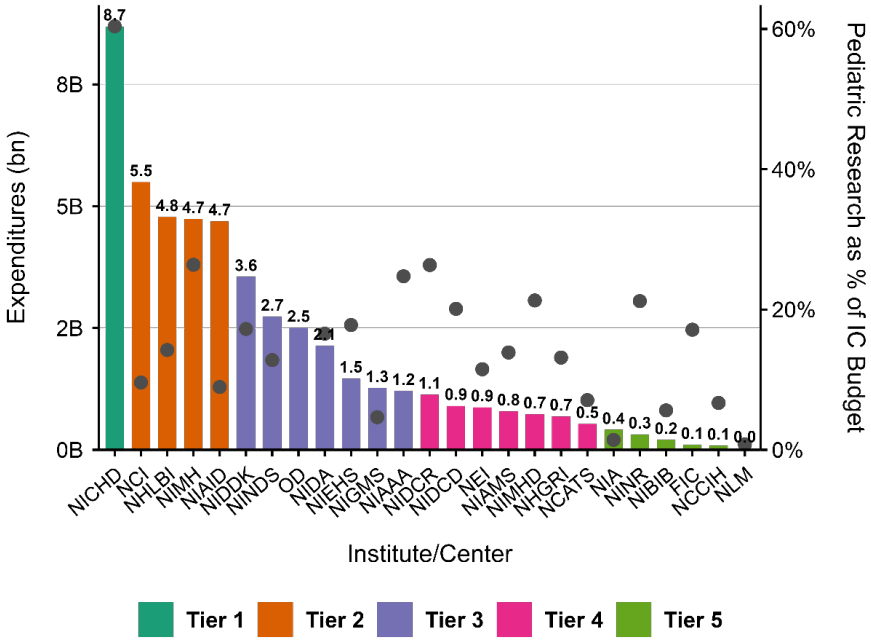

FIGURE S-3 Pediatric research expenditures by IC, FY 2015–FY 2024.
NOTE: RCDC = Research, Condition, and Disease Categorization. The bars represent expenditures by the billion corresponding with the left y-axis. The dots represent the proportion of each IC total research budget that went to pediatric research corresponding with the right y-axis.
SOURCE: Committee's analysis of data from the RCDC system.

The NIH RCDC System for Cataloging Pediatric Research

The Research, Condition, and Disease Categorization system is used to place NIH-funded projects into spending categories for public reporting and ensures uniform coding of research grants and activities across NIH ICs. The RCDC system defines the Pediatric category as research "on health conditions that originate during the embryonic period through age 20." The committee closely examined the methods and rationale used by the RCDC system in categorizing projects as Pediatric from FY 2015 through FY 2024. In FY 2024 there were 310 spending categories assigned to projects that were also assigned to the Pediatric category.

In a survey of 200 randomly sampled projects (20 projects per year from FY 2015 through FY 2024), the committee found that a small percentage of them were classified by the RCDC system as pediatric research even though the project presented no publicly accessible evidence of being

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pediatric research. Using the data available through the RCDC system, it was difficult to ascertain with any level of confidence which research studies were basic science and which were translational research.

Recommendation 3-2[3]: The National Institutes of Health (NIH) should improve the Research, Condition, and Disease Categorization (RCDC) system to better assess funding for pediatric research by (a) adding the new spending categories and (b) improving the Categorical Spending webpage in RePORT.

The following new spending categories should be updated:

a. **Add pediatric developmental stages: during pregnancy, neonate (≤28 days), infant (<1 year old), early childhood: age 1–2 years (1- to 2-year-olds), age 3–5 years (3- to 5-year-olds), middle childhood (6- to 11-year-olds), adolescence (12- to 17-year-olds), and young adults (18- to 24-year-olds).**
b. **Add a Basic Science spending category and ensure its validity.**
c. **Add new terms for pediatric health conditions to better reflect the breadth of pediatric health conditions. The process to determine these new terms should include child health expertise from each institute and center (IC).**
d. **To improve specificity of diagnostic labeling, consider switching eponyms to indicate diseases—e.g., change Cooley's Anemia to Beta Thalassemia Major."**

To improve the Categorical Spending (RCDC) webpage in RePORT, NIH should:

a. **Publish a reference paper that clearly and comprehensively describes the RCDC classification methodology with sufficient detail that it can be replicated or a similar assessment can be done by the scientific community. Update the methodology report whenever the methodology changes.**
b. **Evaluate the validity of key RCDC terms, such as "pediatric" and update the classification to improve accuracy and remove possible biases.**
c. **Allow more than one RCDC term to be exported in a specific project. (This functionality currently exists in RePORTER, but not RCDC.)**

[3] The recommendations in this summary are numbered based on how they present in the chapters of the report.

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d. Enable download of all projects funded by the NIH for a given year.
e. Add a flag to clearly identify extramural, intramural, and, specifically, Clinical Center research and ensure that this information is available for all research projects.
f. Add a search field that links projects to their respective notice of funding opportunity, program announcement, program announcement with special review, request for application, notice of special interest, or request for proposal.

PEDIATRIC RESEARCH AT THE CLINICAL CENTER

The Clinical Center (CC) plays a critical role in supporting pediatric research, particularly for children with rare, complex, or currently untreatable diseases, and makes up most of the IRP's research budget. The CC hosts clinical trials and natural history studies for many pediatric diseases and offers highly specialized wraparound care tailored for children in research protocols. Pediatric research at the CC includes gene, stem cell, and cellular therapies with an emphasis on deep phenotyping, genotyping, and novel therapeutic strategies. As of the first quarter of 2025, approximately one-third of the 1,500 active research protocols included children under the age of 18, and 80 protocols included children under 2 years old.

Recommendation 3-3: The National Institutes of Health (NIH) Clinical Center (CC) should continue to prioritize the current level of pediatric research, particularly on topics and using methods that are difficult to pursue in extramural environments (e.g., longitudinal studies of rare diseases and early-stage interventional trials) as well as on the long-term outcomes of interventions. Potential mechanisms include:

a. Increasing capacity while ensuring safety by overcoming operational constraints at the CC. Younger and more acute patient populations should be supported in early-phase interventional and observational protocols.
b. Continued, expanded, or new partnerships with children's hospitals, academic medical centers, schools, public health departments, and other large health centers across the country to best support pediatric populations.
c. Offering pediatric education and training opportunities at the CC to continue to develop the specialized workforce needed to support this research.
d. Collecting and presenting data in the annual report to the NIH Pediatric Research Consortium (or its equivalent) on the proportion of pediatric research studies, support, and usage by each institute and center at the CC.

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PEDIATRIC RESEARCH PRIORITIZATION AND INCLUSION

The committee evaluated efforts to incorporate pediatric research priorities within and across NIH ICs, facilitators to this incorporation, and how challenges can be addressed, mitigated, or eliminated.

Two critical pediatric research facilitators are IC strategic plans and the NIH Pediatric Research Consortium.

NIH IC Strategic Plans

Each IC periodically creates a strategic plan to identify goals and priorities to drive its scientific research, build research capacity, and facilitate effective management of its intramural and extramural research. The committee evaluated current IC strategic plans and found that many research projects discussed in the plans emphasize the importance of treating the condition as early as possible to yield the best return on investment. In addition, most ICs prioritize research on chronic conditions and diseases that arise during childhood and continue into adulthood.

Though all ICs support pediatric research, only about half of IC strategic plans explicitly mentioned "pediatrics" or "children." Within those plans, there were limited details about the strategic direction for research on child health; more often. pediatric research was embedded within larger strategic priorities, implicitly included in research "across the life course". Importantly, some strategic plans do include specific pediatric research priorities. For example, NICHD mentions a focus on improving child and adolescent health and the transition to adulthood. Few IC strategic plans articulate exactly how success in achieving their goals is measured or monitored.

Recommendation 4-1: All directors of National Institutes of Health institutes and centers that fund research should explicitly incorporate pediatric health and potential downstream life course impacts into their strategic plans, as childhood chronic conditions, exposures, and experiences may have disproportionate health impacts into adulthood. Include metrics such as those focused on investigators (e.g., funding rates and career advancement), scientific discovery (i.e., publications and innovations), impact on child health (e.g., morbidity, mortality), and downstream life course impacts (e.g., premature cardiovascular disease) to measure success in meeting their goals.

In addition, the committee stresses that adopting a common definition for "pediatric research" will facilitate the integration of pediatric research into IC prioritization and strategic plans.

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Recommendation 3-1: The National Institutes of Health should adopt and apply a consistent agency-wide definition of pediatric research. The definition should recognize that pediatric research may include studies that: (1) involve data or specimens from or about infants, children, adolescents, or young adults; (2) inform the prevention, diagnosis, or treatment of conditions affecting these populations; (3) advance the health, well-being, or resilience of children, adolescents, or young adults; or (4) investigate childhood origins of adult health conditions.

NIH Pediatric Research Consortium

The NIH Pediatric Research Consortium (NPeRC) was created in 2018 to improve child health research collaboration and coordination by harmonizing pediatric research endeavors at NIH, identifying gaps in the pediatric research portfolio, and capitalizing on existing resources, infrastructure, and expertise to facilitate and promote pediatric health research across NIH. All work performed through NPeRC is done without explicit funding or resources. NPeRC members have historically represented 25 ICs, two research programs, and five offices and meet several times a year to develop workshops, review research programs and IC portfolios, discuss scientific opportunities and areas for collaboration, and promote research training for the next generation of pediatric researchers. NPeRC is an invaluable entity within NIH for facilitating and coordinating pediatric research. The committee believes that NPeRC is an excellent model for enhancing NIH-funded pediatric research but that it will benefit from having the financial resources and staff expertise to enable it to grow and have the capacity to continue its important work.

Recommendation 4-2: The National Institutes of Health (NIH) director should:

- **a. Elevate the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to the Office of the Director, with membership including senior leaders within each institute or center (IC) with pediatric health and life course expertise, who systematically review all relevant components of strategic plans, including progress and impact made in the past year. Each senior leader should then report back to their IC and to the NIH director.**
- **b. Provide appropriate infrastructure funding and adequate resources (including personnel) to enable NPeRC (or its equivalent) to accomplish its goals and for its members to participate in its work, which includes (1) identifying and addressing research gaps, (2) enabling a nimble response to emerging threats to**

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pediatric health, (3) promoting greater efficiency and reach of ongoing initiatives through data harmonization, (4) spearheading novel partnerships, (5) providing consultation to ambitious inter-IC initiatives, and (6) ensuring representation and visibility at national scientific meetings and societies.

c. Have NPeRC's membership (or its equivalent) include expertise in pediatric bioethics. NPeRC should also partner with patient advocacy or community representatives and develop a communications plan to disseminate pediatric research findings to both the scientific community and the general public. NPeRC (or its equivalent) should be prepared to coordinate the NIH-wide response to any emerging pediatric research needs.

FACILITATORS OF SUCCESSFUL INTER-IC INITIATIVES

Inter-IC pediatric health research initiatives are supported by two or more ICs and/or the Common Fund within the Office of the Director. Inter-IC initiatives have had success in addressing several significant pediatric health problems. In some cases, these initiatives have relied on NPeRC for support and expertise. Successful initiatives include adolescent brain development, environmental influences on child health, and pediatric participant-reported outcome measures, among others. Facilitators enabling the success of cross-IC pediatric research initiatives include: the originating research questions were developed collaboratively, by multiple ICs; those scientific questions raise issues inherently relevant to pediatric populations (and cannot be answered without inclusion of pediatric populations); processes and procedures were included to establish pediatric-focused scientific infrastructure; children were recruited and seen as integral to the study from the beginning; and the initiatives received adequate and sustainable funding.

CHALLENGES TO CONDUCTING PEDIATRIC HEALTH RESEARCH

All types of health research routinely encounter challenges, including a finite pool of research funds and lengthy timelines between grant submission and final funding decisions. However, pediatric health researchers also face some unique legal and ethical, methodological, and structural challenges which often require more funding, time, or both to conduct pediatric research.

Legal and Ethical Challenges

Unique regulatory and legal requirements and ethical considerations impact research involving children. Children are considered a vulnerable

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population, which means that additional ethical and legal safeguards must be in place when conducting pediatric research. One challenge is ambiguity in the interpretation of regulatory requirements associated with different levels of risk to children who participate in a research study. To protect pediatric research participants, federal regulations impose varying requirements based on the research risks and whether those risks are justified by the prospect of direct benefits. However, institutional review boards (IRBs) often use restrictive interpretations of risk and benefit, resulting in study delays, redesign, or cancellation altogether. This occurs despite the existence of NIH's Inclusion of Children policy and Inclusion Across the Lifespan policy, both of which require children be included in human subjects research unless there are scientific and ethical reasons that would prohibit their participation.

Another challenge is deciding when ethical concerns about pediatric participants are best addressed by incorporating additional safeguards, such as extended safety monitoring, to protect current and future participants and patients, as well as the balance of increased cost and complexity.

To mitigate these challenges, NIH can foster a culture of responsible inclusion of children in research, focused on protecting them *through* appropriate research rather than *from* research. NIH can provide the scientific community with clear guidance on ethical protections for children and require explicit articulation of scientific, legal, or ethical reasons for the exclusion of children in its funded research.

Recommendation 4-3: Initiatives within and across the National Institutes of Health (NIH) institutes and centers should coordinate with the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to include children and/or the downstream life course impact of childhood chronic disease, exposures, and experiences in studies in a strategic and deliberate way. NPeRC (or its equivalent) should support the enforcement of existing policies requiring that studies not exclude children or childhood downstream impacts on the life course without clear and adequate justification.

Recommendation 5-1: The National Institutes of Health (NIH) should work to create a culture of ethical inclusion of children in research, focused on protecting children *through* research rather than *from* research. To this end, NIH should develop measurable strategies and mechanisms to better implement and enforce existing policies to ensure inclusion of children at the project and proposal level. Strategies and mechanisms should include:

a. Updating the simplified review framework by explicitly considering the inclusion of children and its implications for health

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during the life course as a component of evaluating a proposal (i.e., its significance) during review.

b. Continuing to support the NIH Center for Scientific Review in implementing and monitoring structural changes to support pediatric research:
   - Requiring and monitoring that pediatric representation is consistent with the number of applications on all relevant standing study sections.
   - Ensuring provision of adequate justification for exclusion of children during grant review: If there is a scientific, legal, or ethical reason for the exclusion of children, this justification should be clearly articulated in the grant application and assessed and documented during the grant review process.
   - Elevating prioritization of implications of research on the life course during scientific review: NIH should identify the impact of pediatric research on the life course as an important component of a proposal's significance section and should do so directly within reviewer guidance. In addition, training for study section reviewers should encourage them to consider whether a proposal adequately addresses the importance of the research for the life course when evaluating the significance or impact of extramural grant applications.

c. Improving transparency on inclusion: The Office of the Director of NIH should annually track and report justifications provided for the exclusion of children. This information should be included in the NIH annual report and published on the NIH Pediatric Research Consortium (NPeRC) website, or an NPeRC equivalent website, to track progress on the improved inclusion of children across NIH activities over time.

d. Providing clearer institutional guidance about the ethical inclusion of children in research: NPeRC (or NPeRC equivalent), in consultation with the NIH Clinical Center's Department of Bioethics, should develop a widely accessible toolkit and guidance on ethical issues in pediatric research that have the potential to unduly impede research if misunderstood, such as when to include children in research, provisions for assent, adolescent independent consent, age de-escalation, and payment for research participation.

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Scientific Challenges

The physiology and behavior profiles of children, the complexity of diseases that affect children, and the limitations in current research models and scientific approaches for fully unraveling the intricacies of pediatric diseases are also challenges.

Developmental growth and maturation differ between children and adults. Disease and injury risk, progression, and treatment responses may differ due to these developmental stages, making it difficult and sometimes inappropriate to translate adult disease models to pediatric populations. In addition, pediatric models often need to account for age-specific factors such as hormonal changes, brain development, and skeletal maturation, which may not be well represented in animal models or adult human models.

Health outcomes in children are shaped by a complex interplay between environmental exposures and genetics and vary across populations. Pediatric models need to incorporate a wide variety of genetic mutations, which can make the models complex and difficult to study. Environmental factors can influence disease development and response to treatment in children, but these factors are difficult to incorporate into animal or laboratory models. These factors are embedded within broader social and structural contexts that influence children's development and disease risk.

Disease can also have a significant impact on normative developmental processes. Chronic illness in childhood affects cognitive, emotional, and social development, and these impacts can complicate the study of disease progression and treatment. The psychological burden of disease may affect children's ability to participate in clinical trials or models. Furthermore, pediatric disease models often need to consider long-term quality of life outcomes, which can be harder to measure and predict in young patients and require longitudinal study.

Methodological Challenges

Research studies that include human participants include several methodologic considerations which take on an additional complexity when children are a part of the study, including in participant recruitment, the number and diversity of participants, and data collection and measurement. These challenges are more than just considerations for good study design. Left unaddressed, they generate structural barriers to pediatric representation in research.

Recruiting and retaining children in clinical trials is often more difficult than recruiting and retaining adults. Parents or guardians are essential participants in recruitment and study processes, meaning that successful

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pediatric recruitment requires both building rapport with and educating participants as well as parents or guardians about the study. There is often a lengthier decision-making process among participants in pediatric research than in adult research, and additional time may be needed to obtain a child participant's assent. Pediatric clinical research is often conducted at urban medical centers, making it difficult for families living in rural and underserved areas to participate. Language barriers may dissuade families from participating in research if adequate interpretation or translation services are not offered. Finally, rare disease studies are a cornerstone of pediatric research, but low prevalence precludes recruitment and enrollment targets on par with adult studies.

Ensuring that the results of pediatric research are generalizable across diverse populations is critical. Many children live in families in which members may have limited English proficiency. Children from rural areas and those living in poverty face distinct health risks and barriers to accessing health care. These children are often underrepresented in clinical trials, so their specific needs and treatment responses are less understood. To increase the participation of rural, underserved, and non-English speaking children and families, flexibility, multilingual resources, and increased collaboration with communities are needed.

Pediatric researchers face several data-collection challenges. Longitudinal data collection may be required to be able to observe relevant outcomes across the life course, and data collection mechanisms often need to evolve as participants transition from childhood to adulthood. Many research tools have not been validated across all age groups. Additionally, outcomes in childhood are best understood within the broader context of family and household factors, where parental health, caregiving practices, and family stressors strongly shape child development and well-being. Capturing these interdependencies requires approaches that extend beyond the individual child to include the larger family unit, but established standards for dyad- or family-unit–based data collection are lacking. Studies relevant to child health may have a longer trajectory in reporting groundbreaking findings, something that must be acceptable to many, including Congress. Pediatric research projects and funding should not be shortchanged due to the more expensive nature (infrastructure needs) and a longer timeframe needed to attain fruitful results.

Trust is critical in pediatric research, given that parents or guardians choose to enroll their children, and not themselves, in a study that may pose risk. Entire communities may be deterred from participating in pediatric research if researchers do not practice cultural humility and language justice. Others may mistrust the larger research enterprise because of historical harms done to their communities in the name of science. These concerns and issues of mistrust may be mitigated through meaningful engagement

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of communities in the research process. The development of lasting trust is increasingly recognized as a component of research that is ethically important and a facilitator of increased participation. Community-based participatory research, the use of patient and family advisory councils, and other robust community-engagement methods could help increase trust, ensure that researchers address actual community concerns, and ultimately ensure the success of pediatric research for all communities.

There are also unique challenges for pediatric health services research. Pediatric health care delivery systems and financing are unique in workforce, geographic distribution, policy, and financing. Financing and policy for children are determined much more at the state level than at the federal level due to the large proportion funded by Medicaid rather than Medicare. Databases commonly used in adult research (Medicare, Veterans Affairs) are not relevant to children, requiring instead that other databases, such as state Medicaid records, be used. These factors require pediatric expertise, data, and funding to answer key questions in child health services research.

Structural, Procedural, and Financial Challenges

Pediatric researchers face an array of unique challenges during the grant application and review processes. While all investigators who seek NIH grant awards grapple with protracted timelines, studies that include children often face an extended period of review due to concerns about additional safeguards for children as a vulnerable population. Pediatric expertise can vary widely among reviewers in a study section, potentially leading to inadequate reviews and lower prioritization of pediatric research.

Funding for pediatric grants may not meet the actual costs required for pediatric trials, which can be more expensive and logistically complex than adult trials. The pediatric population available for study for many diseases is significantly smaller than the available population for adult studies, since most children are healthy. Thus, pediatric research studies, especially for rarer conditions or diseases, often have multiple study and recruitment sites, requiring greater infrastructure costs and time, for example, for multiple IRB reviews or to develop a consortium agreement among the institutions.

Due to the accelerated pace of development in childhood and critical periods which creates the potential for delayed outcomes from interventions, it may require multiple sequential studies to ensure generalizability across pediatric populations.

Researchers should also plan to compensate parents or guardians for missed work time and travel to enrollment sites. Additional research time may be needed if data collection includes parents, siblings, and the children enrolled in the study, which is often the case for pediatric studies.

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Recommendation 5-2: National Institutes of Health (NIH) institutes and centers should award pediatric research grants and budgets that support the specific needs of pediatric studies. In addition, to address the mismatch between study timelines and funding cycles, NIH should:

- **a. Allow for extended or extendable award timelines and/or renewable grant mechanisms specifically designed for pediatric research, allowing investigators to sustain long-term studies that align with children's developmental trajectories;**
- **b. Provide higher budget maximums to account for the need for representative recruitment and multi-center studies, when scientifically justified;**
- **c. Create supplemental funding opportunities specifically designed for longitudinal or follow-up data collection, which would enhance learning across the life course while capitalizing on previously funded and ongoing studies;**
- **d. Provide funding supplements to support multilingual research and community engagement to facilitate representation for generalizable results; and**
- **e. Increase access and usage of the Clinical Translational Science Awards as a mechanism for promoting pediatric research.**

A VISION FOR THE FUTURE OF PEDIATRIC HEALTH RESEARCH AND CHILD HEALTH

The committee envisions the United States as a global leader in promoting child health, preventing childhood diseases, reversing recent declines in child health, and applying scientific discoveries through a robust, dynamic, and integrated pediatric research program at the NIH.

To actualize that vision, the committee created a set of four goals (see Figure S-4) that served as guideposts for the formulation of its conclusions and recommendations. These goals will ensure that all children are supported, resilient, and thriving across the life course.

The committee sees an opportunity for NIH to lead the United States out of the current crisis in child health to a brighter future. The NIH has an opportunity to reaffirm the unique value of pediatric research to the health and well-being of children and, because children are the future, to demonstrate the significant return on investment that the nation gains in health, prosperity, opportunity, innovation, and growth by prioritizing pediatric health research that is comprehensive, equitable, and groundbreaking.

The future of pediatric research will be shaped not only by scientific innovation, but by the willingness of leaders to steward public resources wisely, build cross-sector coalitions, and keep children at the center of national health priorities. NIH is uniquely positioned to lead this effort,

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aligning research to public concerns and demonstrating and communicating impact from the laboratory to the community. A renewed commitment to child health research is not an expansion or a transformation per se—it is a reorientation: toward value, impact, and a future in which every child can thrive. America's children are worth the investment.

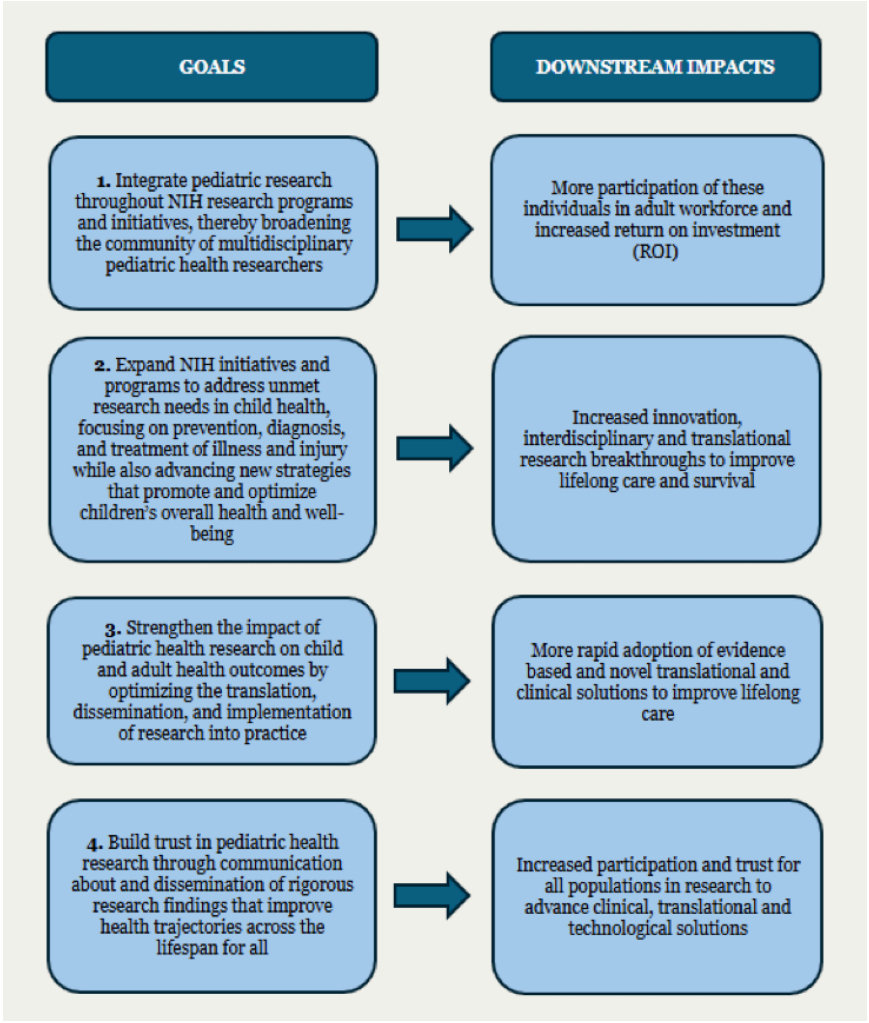

FIGURE S-4 Committee goals and downstream impacts.

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1

Introduction

The goal of pediatric health research is to understand, prevent, and treat diseases and conditions primarily affecting children, adolescents, and young adults. This research is vital to ensuring healthy development, advancing medical knowledge, and informing evidence-based public health policy. It also illuminates our understanding of the underlying causes of disease and guides the development of treatments and cures. Because children have unique and rapidly evolving physical and socioemotional needs, pediatric health research helps tailor treatments and prevention strategies to be safe and effective for them. It also generates data and evidence that support public policies and programs such as care for children with medical complexity, nutrition guidelines, and injury prevention. Pediatric research addresses health gaps and environmental influences of health such as family environment, socioeconomic status, and chemical exposures by identifying risks and effective interventions. Since many adult health conditions originate in childhood or prenatally, early identification and prevention measures based on pediatric research can improve lifelong health outcomes.

STUDY BACKGROUND AND CONTEXT

The National Institutes of Health (NIH) is the world's largest medical research agency and public funder of biomedical research (NIH, 2025b). NIH supports scientific discovery through intramural research and extramural grants and contracts to institutions across the United States. NIH's mission is "to seek fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health,

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lengthen life, and reduce illness and disability" (NIH, 2025d). NIH supports pediatric research across basic, translational, and clinical science, with more than $6.19 billion invested in fiscal year (FY) 2024. Nearly all NIH institutes and centers (ICs) fund pediatric research, led by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), which accounts for about 17 percent of all pediatric research funded by NIH (see Chapter 3). Other key funders of pediatric health research at NIH are the National Cancer Institute; National Heart, Lung, and Blood Institute; National Institute of Allergy and Infectious Diseases; and National Institute of Mental Health. NIH pediatrics research funding increased steadily from FY 2015 to FY 2024, with a cumulative increase of 70.52 percent in real dollars and by 28.72 percent in inflation-adjusted dollars, though the spending power has flattened since 2020 (see Chapter 3 for more detail).

Along with its extensive support for extramural research, NIH also conducts pediatric studies within its Intramural Research Program (IRP), which accounts for 11 percent of the NIH budget (NIH, 2025a). The NIH Clinical Center is the world's largest research hospital, and, as a major component of the IRP, it hosts around 560 current research studies involving children (NIH Clinical Center, 2023). Approximately half of these are natural history studies of disease; the rest test new therapies, including first-in-human or pediatric-first trials.

To facilitate and improve coordination of pediatric research across NIH, the NIH Pediatric Research Consortium (NPeRC) was launched in 2018 (NICHD, 2025b). Although NPeRC has no direct funding or research authority, it facilitates cross-IC collaboration on the research and development of pediatric devices, clinical trials, pediatric-to-adult health care transitions, and COVID-19, among many other important pediatric research subjects. The NPeRC membership includes representatives from 25 institutes and centers, five offices, and two research programs, All of Us and Environmental influences on Child Health Outcomes (NICHD, 2025b). NPeRC is currently chaired by the director of NICHD's Division of Extramural Research. NICHD's progress has been highlighted in several of NIH's recent Congressional justifications (NICHD, 2023), including NPeRC's efforts to identify research gaps and opportunities that arise when adolescents with chronic conditions transition to adult health care (2023), efforts to address COVID and long COVID issues among pediatric populations (2024), and efforts to improve the development of pediatric medical devices (2025) (NICHD, 2023, 2024, 2025a).

The mechanism used to track NIH spending, the Research, Condition, and Disease Categorization (RCDC) system, was initiated in 2009 after the National Institutes of Health Reform Act of 2006. The RCDC system categorizes NIH-funded projects into non mutually exclusive scientific

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"spending categories" for public reporting (see Chapter 3 for further detail). The NIH Research Portfolio Online Reporting Tools (RePORT) website provides access to a variety of reporting tools, datasets, and analyses on NIH-funded research as well as research funded by other federal agencies, such as the Administration for Children and Families, the Agency for Heathcare Research and Quality, the Centers for Disease Control and Prevention, the Health Resources and Services Administration, the Food and Drug Administration, and the Department of Veterans Affairs (RePORT, 2025). The RePORT Expenditures and Results module (RePORTER) provides searchable access to NIH-funded projects, publications, and patents (see Chapter 3 for further detail).

PROJECT ORIGIN AND STATEMENT OF TASK

In 2024, NIH requested that the National Academies of Sciences, Engineering, and Medicine (the National Academies) examine NIH's pediatric research portfolio and structure, as was mandated by Congress in an amendment to the Appropriations Act of 2024.[1] In response, the National Academies formed the Committee on Strategies to Enhance Pediatric Health Research Funded by NIH. The sponsor's charge to the committee is presented in Box 1-1.

SIGNIFICANCE OF PEDIATRIC HEALTH RESEARCH

> *Children are not just small adults, and they are our future. Children grow, develop, and respond to the world in ways that are completely different than adults, and they are entirely dependent on the adults in their lives to help keep them safe and healthy. We need research that's specifically focused on them to truly understand what makes them sick, but also what helps them thrive.*
>
> *– Participant in the committee's call for perspectives*

Pediatric health research is vital for advancing our understanding of childhood diseases and developmental processes, leading to more effective, age-appropriate treatments and preventive strategies. Because children represent the future of our country's economy, its cultural fabric, and its success on the global stage, pediatric health research is also essential for the future vitality of the United States. Child health directly impacts physical and mental health across the entire life course. It lays the foundation for lifelong health by identifying interventions that can improve outcomes from

[1] *Further Consolidated Appropriations Act, 2024*, Public Law 118-47, 118th Cong., 2nd sess. (March 23, 2024). See also https://www.nationalacademies.org/en/ocga/public-laws/further-consolidated-appropriations-act-2024 (accessed July 10, 2025).

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BOX 1-1
Statement of Task

The National Academies of Sciences, Engineering, and Medicine (NASEM) will convene an ad hoc committee to examine pediatric research supported by NIH, as described below. The committee will consider the perspectives of multiple NIH institutes and centers (ICs) in its deliberations, as pediatric research is conducted throughout NIH.

The committee is tasked with examining the current NIH pediatric research portfolio and structure including:

- The methods and rationale involved in categorizing projects as "Pediatrics" research using the Research, Condition, and Disease Categorization Process;
- How pediatric components have been included or excluded from larger NIH initiatives;
- Structural or process impediments to pediatric research applicants;
- How pediatric research priorities are established within and across ICs; and,
- How pediatric research activity is coordinated across NIH ICs

Based on this review, the committee will make recommendations focused on improving NIH's overall support of child health research. Any recommendations for structural or process changes should include consideration of burden (including financial) on both researchers and NIH staff.

The committee may also consider ways in which the NIH Clinical Center (CC) could be used to advance innovative pediatric research (e.g., whether it would be advantageous to expand the ages eligible for research studies, areas of science uniquely suited to being conducted at the NIH CC, and potential partnerships to expand the research portfolio at the CC).

infancy through adolescence and beyond. Therefore, it is critically important that children receive adequate and appropriate care in childhood that optimizes their health, enabling them to thrive as children and setting them up for optimal health as adults. This kind of care would not be possible without advances in pediatric health research.

In the United States, there is broad consensus among scientists, clinicians, politicians, and public health officials that there is a current crisis in child health (Forrest et al., 2025; HHS, 2025; NASEM, 2024). Children are experiencing high incidences of chronic diseases (obesity, asthma, diabetes,

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etc.) as well as poor mental, emotional, and behavioral health (Heffernan and Macy, 2025; NASEM, 2019, 2024). A series of studies conducted in the second half of the 20th century established the importance of in utero and early child health on the trajectory of health over the life course (van den Broek and Fleischmann, 2019). Malnutrition during pregnancy increases the risk of adult coronary artery disease and premature brain aging, as shown by studies of adults born during the Dutch Famine of World War II (Franke et al., 2018). Studies of adverse childhood experiences (ACEs) demonstrate the cumulative effects of these events on the risk of adult depression, substance abuse, obesity, hypertension, and diabetes (RePORT, 2025; Roseboom et al., 2006; van den Broek and Fleischmann, 2019; Webster, 2022). The seminal report *From Neurons to Neighborhoods* summarized the biological effects on the brain of early life events that affected later cognitive ability and mental health (IOM and NRC, 2000). Most importantly, research has not only uncovered these causal effects but also has shown that interventions in utero, infancy, and early childhood will have effects throughout the life course; the impacts of negative or positive exposures in early life are cumulative, and adults who experienced poor health or adversity in childhood have substantially higher rates of a variety of costly chronic health conditions (NASEM, 2019, 2024). However, these outcomes across the life course are not fixed, as research has also demonstrated that individual exposures, choices, and resilience can modulate risk over time (NASEM, 2019).

The health and well-being of too many American children are being imperiled by social influences of health such as poverty, food insecurity, and poor access to health care. According to the American Community Survey, based on family income in 2022, the rate of child poverty in the United States was 16.3 percent, 3.7 percentage points higher than the overall rate of poverty (U.S. Census Bureau, 2023). These rates vary by demographic characteristics, such as geographic location and race/ethnicity. For example, in 2022, "9 of 17 states in the South region had child poverty rates of 18.0 percent or more," compared with only one state each in the Midwest, Northeast, and West regions (U.S. Census Bureau, 2023). According to a 2023 analysis of data that estimated the overall child poverty rate at 16, child poverty was lower than the overall average among non-Hispanic White and Asian and Pacific Islander children (10 percent among both groups) and higher among American Indian or Alaska Native, Black, and Hispanic or Latino children (27, 29, and 22 percent, respectively) (Annie E. Casey Foundation, 2024). Often associated with factors such as poor living conditions, lack of insurance, and lack of access to healthy food, poverty can give rise to a variety of health problems.

These barriers to health and well-being, as well as gaps in our knowledge of pediatric health, have important consequences. Forrest and colleagues

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found that children's health steadily worsened in the United States between 2007 and 2023 across multiple domains including mortality; chronic physical, mental, and neurodevelopmental conditions; functional status; and physical and emotional symptom domains (Forrest et al., 2025). During the same time period, the study found that infants were 78 percent and 1- to 19-year-olds were 80 percent more likely to die compared with children in comparable high-income countries (Figure 1-1). Pediatric research is critical to a life course approach to health, offering insights into early-life origins of disease, prevention strategies, and interventions that can reduce chronic illness and improve outcomes well into adulthood (Speer et al., 2023). Poor health and disability affect one's ability to participate in the workforce, military, and society overall, affecting health care expenditures, the economy, national security, business viability, and public finances (NASEM, 2019). These observations underscore the need to sustain investment in pediatric science as a foundation for improving population health. With declining child health in the United States, the need for pediatric research and innovation has never been more critical (Forrest et al., 2025).

> *If we don't take steps now to build a strong, healthy foundation for our young people, then it will not just be our military that pays the price—our nation as a whole will suffer also.*
>
> *– Richard R. Jeffries, Rear Admiral, U.S. Navy (Retired) and former medical officer of the U.S. Marine Corps*

For nearly 100 years, NIH has led the way in biomedical research. The federal government's interest in improving the health of its citizens, its consistent appropriations, and its recognition that basic and clinical research are essential to life-changing medical advances have made the NIH and the U.S. biomedical community global leaders in advancing scientific discovery and medical breakthroughs.

Challenges to Conducting Pediatric Health Research

Pediatric health researchers encounter unique medical, ethical, logistical, and scientific considerations due to the vulnerability and rapid development of children. As a result, in comparison with health research for adult populations, pediatric health research requires the inclusion of age-appropriate methods, additional protection in the informed consent process, and specialized designs of clinical trials (NIH, 2025c; Shah, 2021). These factors shape the pediatric research landscape and contribute to ongoing challenges in conducting pediatric research, such as funding levels that are not commensurate with either disease burden or the timelines needed to conduct pediatric health research. These issues and challenges are explored further throughout the report, especially in Chapter 5.

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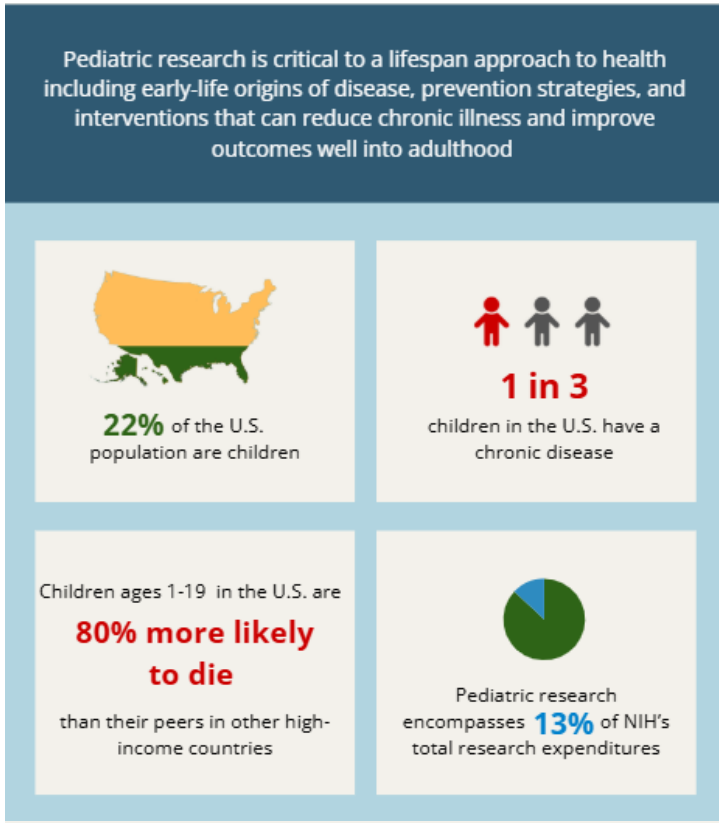

FIGURE 1-1 Pediatric health in the United States.
SOURCE: Data compiled from Forrest et al. (2025); NASEM (2024); and Wisk and Sharma (2025).

Several historical cases of ethically problematic research led to the creation of the National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research in 1977 and the passage of regulations intended to ensure the ethical conduct of research for all participants, including children (Shah, 2021). An unintended consequence of this history is that a protectionist mentality has taken hold (Levine, 1988). Described by Carol Levine as "born in scandal and reared in protectionism," the research oversight system responded to a troubling history by focusing on protecting children *from* research, rather than *through* research (Levine, 1988). Clinical care guidelines for children are often based on data from adults, resulting in care that is not optimized for children, and which may be ineffective or even harmful (Petkova et al., 2023). Furthermore, without research to

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support the evidence and approval for the use of many interventions, clinicians caring for children too often are forced to rely on guesswork or to extrapolate from data in adults in ways that can fail to benefit children or even harm them in some cases (Joseph et al., 2015; Klassen et al., 2008; Nardini, 2014; Sinha et al., 2008).

Pediatric health research has led to significant benefits for children and society (see Table 2-1). However, as Chapter 5 of this report discusses in more detail, inadequate communication about these achievements, lingering mistrust due to the historical cases of medical abuse, and a lack of thoughtful community engagement in research have created a gulf between the public and the pediatric health research community. To foster trust and carry out the research needed to fill the gaps in our knowledge of pediatric health, the pediatric health research enterprise needs to bridge this divide. Community engagement, including the engagement of youth, improved dissemination of research, and communication about the benefits of pediatric research to children and the nation at large, is a critical component of successful pediatric research (see Chapter 5 for more on engaging communities in the research process).

STUDY APPROACH

The Committee on Strategies to Enhance Pediatric Research Funded by NIH included 17 members with a range of expertise in basic, clinical, and translational pediatric research; pediatric health services research; biostatistics; pediatric health science policy; bioethics; pediatric health disparities; patient advocacy; and NIH research programs. Biographical information for each member can be found in Appendix A.

The committee held five 2-day hybrid meetings and multiple virtual meetings between December 2024 and September 2025 to gather information, consider findings, and develop recommendations. Several of the committee's information-gathering sessions featured invited speakers from NIH, who shared valuable insights into the current state of the pediatric health research funding landscape, how NIH prioritizes and impacts pediatric health research, how the various NIH ICs contribute to pediatric health research in their respective health fields, and how the Research, Condition, and Disease Categorization (RCDC) and RePORTER systems catalog and track pediatric research (see the open session agenda's in Appendix B). NIH speakers also discussed some of the challenges they encounter in reviewing, funding, and tracking pediatric research. The committee issued two call for perspectives (CFPs) through the project webpage: one for input and perspectives from research applicants on the challenges that they face in obtaining funding for pediatric research, how they have approached incorporating pediatric populations into their work, and any real or perceived

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barriers to conducting such research, and a second for research participants and families that have participated in NIH research studies. The full list of CFP questions is provided in Appendix C.

The committee reviewed existing literature to inform its analysis. This included peer-reviewed scientific articles as well as publications from public agencies, private organizations, and governmental bodies, with a focus on materials published from 2010 to the present. The review centered on U.S.-based research and covered a wide range of topics relevant to pediatric science, including federal mandates related to pediatric research, trends in funding, notable successes in the field, and barriers that researchers face in entering or remaining in pediatric research, such as difficulties securing funding, limited job opportunities, and attrition from the research workforce. Both extramural and intramural research activities were considered, including studies conducted at the NIH Clinical Center. The committee also reviewed literature referencing the RCDC system, particularly regarding challenges in accurately categorizing pediatric research within this taxonomy. Search terms covered age groups from birth through adolescence, including "child," "lifespan," "adolescent," and "young adult."

Vision and Goals

This report examines how pediatric research is defined and tracked, its inclusion in NIH-wide initiatives, barriers faced by pediatric investigators, how priorities are set, and coordination mechanisms, culminating in recommendations for strengthening NIH support for child health research. The committee's vision for the future of NIH pediatric research, and the goals to achieve this vision, are in Box 1-2.

Life Course Developmental Perspective

The committee used a life course developmental perspective as a lens through which child health and pediatric research are understood. The term "life course" is preferred over "lifespan" (duration of life) or "life cycle" (biological maturation from child to adult form) because it emphasizes the dynamic, developmental, and contextual processes that shape health over time (Alwin, 2012). Health is not static but changes across the life course, molded by biological, psychological, and social transitions; life's turning points; physical and social exposures; and lived experiences. NIH funds research on individuals at various points in the life span. Pediatric research is unique because of the effects of child health over the life course.

Health is acquired, developed, optimized, sustained, and ultimately lost across the human life course. It arises through developmental and relational processes produced by continuous interactions between an individual (I)

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BOX 1-2
The Committee's Vision and Goals

The United States will be the global leader in promoting child health, preventing childhood diseases and morbidity, reversing recent declines in child health, and using scientific discoveries, through a robust, dynamic, and integrated pediatric research program at the National Institutes of Health (NIH). These goals will ensure that all children are supported, resilient, and thrive across the lifespan. To accomplish this vision NIH should:

GOAL 1: Integrate pediatric research throughout NIH research programs and initiatives, thereby broadening the community of multidisciplinary pediatric health researchers .

GOAL 2: Expand NIH initiatives and programs to address unmet research needs in child health, focusing on prevention, diagnosis, and treatment of illness and injury while also advancing new strategies that promote and optimize children's overall health and well-being.

GOAL 3: Strengthen the impact of pediatric health research on child and adult health outcomes by optimizing the translation, dissemination, and implementation of research into practice.

GOAL 4: Build trust in pediatric health research through communication about and dissemination of rigorous research findings that improve health trajectories across the lifespan for all.

and the ecosystems (E) in which they live, grow, learn, work, and age. These I⇔E interactions, which span multiple levels—from the inner world of mind–body functioning to families, communities, and the global environment—constitute the dynamic foundation of health development. This perspective positions health as emergent, relational, and ever-changing, rather than as a fixed state, and this perspective is at the core of the life course developmental approach (Halfon, 2018).

Life course development recognizes that experiences may have disproportionate effects when they occur during sensitive or critical periods of life, may accumulate incrementally over time, and may occur sequentially in ways that amplify or mitigate earlier exposures. In each case, the timing, duration, and sequencing of exposures interact with individual biobehavioral systems and context to shape outcomes. The health effects may manifest within minutes (e.g., a toxin exposure), years (e.g., obesity trajectories), or decades (e.g., adverse childhood experiences) (Felitti et al., 1998; NASEM, 2019). Second, health emerges from interactions within relational networks that include people, technologies, objects,

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non-human living beings, and physical and social settings. These networks create flows of resources, stressors, and supports that channel developmental outcomes. Third, life course development emphasizes the biobehavioral mechanisms and regulatory systems, including epigenetic, neural, physiological, and behavioral processes, through which exposures and experiences are transduced into patterns of mind–body functioning (Aristizabal et al., 2020; NASEM, 2019).

Although development occurs over the full life course, it occurs most rapidly during childhood. These rapid changes set the foundation for adult health. Childhood is a time of both resilience and vulnerability. It is when sensitive periods are most common, a period of life when health can be shaped in both positive and negative ways with short-term and long-term implications. In fact, childhood itself can be considered a sensitive period, which is one of the chief reasons that the United States needs a concentrated focus on research on child health. Therefore, beyond understanding childhood health related issues, it is critical to understand the downstream life course impacts of childhood chronic diseases, exposures and experiences on adult populations (see Figure 1-2).

Life course development views health not as a static state but as a set of interrelated trajectories, each reflecting the patterns and trends of specific health attributes (e.g., longevity, diseases, mind–body functioning, and distress). These trajectories are not linear; they are shaped by an individual's interactions with their physical and social environments, and they can accelerate, plateau, or decline, shaped by resilience and disruption. From this perspective, health development is a continuous, cumulative, and adaptive process, in which childhood provides the foundational structures and capacities that enable—or constrain—human health across the remainder of life.

Over the past decade, the National Academies has published a number of reports that examine factors influencing pediatric research and child health and recommend ways these can be improved. Because this report focuses specifically on enhancing NIH-funded pediatric research, readers may find these other reports, which focus more on recommendations for broader policies that influence pediatric health, of interest as well. Many previous studies and reports have relevance to this study, and several are referenced in this report. Some recent National Academies' reports focused on pediatric health and research and NIH's research portfolio include:

- *A New Vision for Women's Health Research: Transformative Change at the National Institutes of Health* (2025)
- *The Future Pediatric Subspecialty Physician Workforce: Meeting the Needs of Infants, Children, and Adolescents* (2023)

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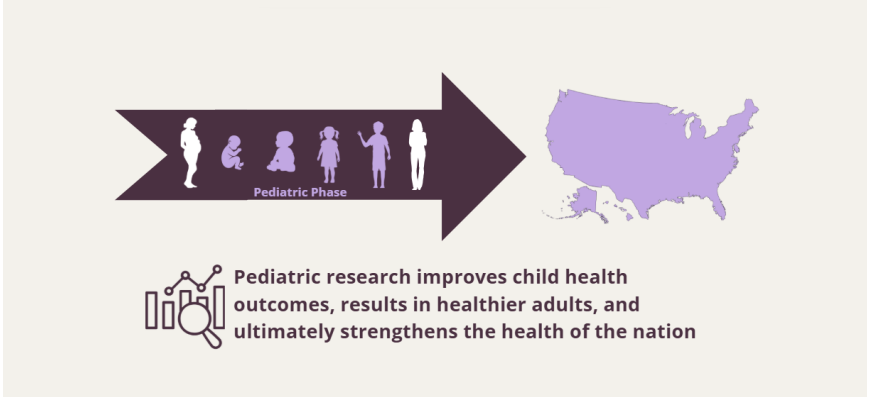

FIGURE 1-2 Pediatric research across the life course.
Previous Work of the National Academies

- *Launching Lifelong Health: Supporting Children, Youth, and Families for a Thriving America* (2024)
- *Childhood Cancer and Functional Impacts Across the Care Continuum* (2021)
- *Vibrant and Healthy Kids: Aligning Science, Practice, and Policy to Advance Health Equity* (2019)
- *The Promise of Adolescence: Realizing Opportunity for Youth* (2019)

Data Limitations and the Changing Research Environment

The committee encountered several challenges in assessing the scope of pediatric research supported by NIH. One involved limitations on available data. Age tagging of grants is not consistent among ICs, and differences in how ICs track and report their work in general make it hard to get a clear picture of the details of pediatric research supported by NIH. A second challenge was tied to new technologies, emerging health issues, and evolving research priorities. These important developments mean that "pediatric research" is a constantly evolving category of work. The committee has attempted in this report to provide a snapshot of current pediatric research in the United States through the lens of NIH. The report also builds on the committee's vision and goals so that as pediatric research evolves, its North Star remains, ensuring that all children are supported, resilient, and thriving across the life course.

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TERMS USED IN THIS REPORT

Pediatric Research

Pediatric research addresses "fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health, lengthen life, and reduce illness and disability" among individuals who have not yet reached an adult level of biological or socio-emotional maturity (NIH, 2025d). There are myriad opportunities at the National Institutes of Health to define or categorize "pediatric research," many of which are used to suit the needs of different institutes or centers, consortia, or reporting groups. While NIH uses a strict definition of "child" as far as participation in research—i.e., a child is any individual younger than 18 years old (NIH Office of Extramural Research, 2015)—it does not strictly define the concept of pediatric research (Glavin, 2024).[2] Instead, it offers three categories of research that may be categorized as "pediatric" by the RCDC tool. These include

- A study in which the research participants do not specifically exclude children,
- A study in which the research participants specifically include children, and
- A study in which the research aims, and clinical or public health relevance focus on conditions or developmental processes that typically originate and manifest in childhood.

Notably, the third category relates to the use of a life course approach when addressing, evaluating, or performing pediatric health research (see the section Life Course Developmental Perspective, above). A life course approach requires understanding the distinctions between developmental stages and how they may impact health conditions or diseases. It includes a broad spectrum of ages because parental health preconception can affect a child's health (NASEM, 2019) and diseases that originate in childhood do not typically disappear once a person turns 18 years old.

Researchers

The committee's statement of task includes a charge to address impediments faced by pediatric research applicants. These applicants include individuals with a range of degrees including, but not limited to, M.P.H., M.D.,

[2] Presentation by Sarah Glavin to the Committee on Strategies to Enhance Pediatric Health Research Funded by NIH, meeting 1, December 5, 2024.

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D.O., Ph.D., Dr.P.H., Sc.D., D.N.P., and J.D. In many cases, individuals have more than one of these degrees.

This report primarily uses "investigators" to describe this diverse range of individuals and less frequently uses "researcher" or "scientist" in place of "investigator." Terms such as "clinician–investigator" or "physician–scientist" are used to specifically refer to individuals with both clinical and research duties. Many investigators often possess a clinical degree such as an M.D., D.O., or D.N.P. in addition to a research degree such as a Ph.D., Dr.P.H., Sc.D., or M.S. "Physician" refers specifically to individuals with an M.D. or D.O. while "clinician" encompasses a broader range of individuals with clinical duties, such as nurses, midwives, psychologists, social workers, physical and occupational therapists, and community health workers in addition to physicians (HHS, n.d.; NCI, n.d.-a, n.d.-b).

ORGANIZATION OF THE REPORT

Chapter 2 provides background information on the pediatric research landscape, including NIH's history within it and the wider funding landscape. Chapter 3 examines the current NIH pediatric research portfolio, including both intramural and extramural research across the ICs. Chapter 4 reviews how pediatric research is prioritized, coordinated, and integrated across and within ICs. Chapter 5 discusses structural and process impediments that pediatric researchers may experience while applying for funding and conducting the research. Finally, Chapter 6 offers a vision for a future robust pediatric health research enterprise.

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2

Background: History, Funding Landscape, and NIH Structure

HISTORY OF NIH-SUPPORTED PEDIATRIC RESEARCH

The National Institutes of Health (NIH) began in 1887 as the Hygienic Laboratory for the study of bacteria (NIH Almanac, 2025). After several decades of growth, it was officially designated by Congress in 1930 as the National Institute of Health. Eighteen years later, it became the National Institutes of Health. NIH gained prominence and grew rapidly during the middle of the 20th century. Between 1947 and 1957, NIH grant awards grew 25-fold, from about $4 million annually to over $100 million. Similarly, the overall NIH budget grew from $8 million in 1947 to over $1 billion in 1966. Government funding allowed NIH to rapidly become a global leader in supporting research on human health, including the health of children.

Throughout the 20th century—and thanks in large part to NIH support for scientific inquiry—medical science advanced quickly. Penicillin, discovered as an antibacterial agent in 1928 by Alexander Fleming, became widely used and available during and after World War II. Jonas Salk developed the polio vaccine in 1954. The 1955 discovery by Joe Hin Tijo that humans had a total of 46 chromosomes led to recognition of genetic disorders, including substantial research on children's intellectual and developmental disability created by chromosomal abnormalities and genetic mutations. In 1961 Robert Guthrie developed a simple blood test to screen for phenylketonuria in newborns, the first newborn test for a genetic disorder.

Because of these advances, the nation's attention became focused on understanding and improving the health of children. The National Institute

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for Child Health and Human Development (NICHD) was created in 1962, representing the first institute at NIH not focused on a specific disease or organ system. An early focus at NICHD was on intellectual and developmental disabilities, inspired by the Kennedy family and especially Eunice Kennedy Shriver, who had a personal dedication, given her sister Rosemary's disability. Another early focus at NICHD was the consideration of biobehavioral and social science aspects of children's health. Researchers recognized the multiple systems that impact a child's development and thus their physical and mental health, and so pediatric health research began to consider family and community systems that might impact the healthy development of children across the country. See Appendix D for a more detailed history of pediatric health and research.

Child health in the United States experienced another concentrated flurry of activity and successful initiatives between the 1980s and 2000s. Some of the exemplary NIH-enabled research advances that improved the health of children are found in Table 2-1. For each advance summarized in Table 2-1, the NIH-supported translational process began decades before widespread clinical uptake. For example, the discovery of cell-free fetal DNA in maternal plasma in the late 1990s eventually enabled today's noninvasive prenatal testing, and the identification of the *SMN1* gene in 1995 launched a 20-year trajectory culminating in gene therapy for spinal muscular atrophy. Similar multi-decade arcs link early NIH investments to today's front-line therapies and public health recommendations.

Initiatives like the National Children's Study, a prospective longitudinal study to collect a broad range of environmental exposures on child health, provided important lessons for the success of future studies (IOM, 2014). Reflections on the study led to a recognition of the need for leadership buy-in, a clearer study design, more expertise in relevant scientific and survey disciplines, and stronger oversight and review structures (IOM, 2014).

FEDERAL LAWS AND POLICIES SUPPORTING AND PROTECTING CHILDREN IN HEALTH RESEARCH: A BRIEF HISTORY

Though children have been included in research for decades, they are considered a vulnerable group because they lack the capacity to provide their own informed consent and protect their own interests, putting them at increased risk of being harmed or wronged (CIOMS, 2016; Hurst, 2008). In addition, biological and behavioral differences between children and adults may make children more likely to suffer harm from the use of experimental interventions, unless care is taken to protect their safety and well-being.

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TABLE 2-1 Pediatric Health Achievements Funded by NIH

Innovations enabled by NIH funding	Approximate start of NIH-supported research*	Consequent improvements to child health
Cell-free prenatal screening (NHGRI, 2024)	**Late 1990s** - discovery of cell-free fetal DNA in maternal plasma and early NIH-supported non-invasive prenatal testing development	Opportunities to diagnose and treat congenital anomalies in children before birth; invasive amniocentesis rarely necessary (Turriff et al., 2024).
Advances in prevention and treatment of neonatal conditions (NICHD, 2025d)	**1960s** - early NIH investments in neonatal intensive care, surfactant, and antenatal steroids that laid the foundation for current survival at 22–23 weeks	Babies born as early as 22 weeks survive (Edwards et al., 2024).
Newborn screening for genetic diseases (NIH Office of Strategic Coordination, 2025b)	**1960s** - NICHD-supported development and validation of newborn screening for PKU and other inherited conditions	Interventions for genetic diseases can be started within hours of birth (CDC, 2024).
New approaches to treat and cure childhood cancer (NCI, 2023, n.d.-a; NIH Office of Strategic Coordination, 2025a)	**1950s** - creation of NCI pediatric cooperative groups and early multi-agent chemotherapy trials that launched modern childhood cancer treatment	Increased 5-year survival rates for children to over 85%, with more than 80% of children expected to survive into adulthood (Hunger et al., 2012; Moke et al., 2018; NASEM, 2021).
Safe Sleep campaigns (NICHD, n.d.-b)	**1990s** - NIH-supported sudden infant death syndrome (SIDS) epidemiology and sleep-position studies	50% reduction in infant deaths from SIDS (Moon et al., 2022a, 2022b).

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TABLE 2-1 Continued

Innovations enabled by NIH funding	Approximate start of NIH-supported research*	Consequent improvements to child health
Reduction in maternal transmission of HIV to their infants (Connor et al., 1994)	**Late 1980s** - NIH AIDS Clinical Trials Group studies of antiretrovirals in pregnancy and delivery culminating in ACTG 076 (published 1994)	Infants born to mothers with HIV are routinely born without the virus (Irshad U, 2023; Nesheim et al., 2018).
New therapies for chronic diseases of childhood (e.g. cystic fibrosis) (NHLBI, 2024)	**1980s** - CFTR gene discovery and NIH-supported studies of CF pathophysiology that enabled CFTR-modulator and other targeted therapies	Life expectancy for children born with cystic fibrosis was once in the single digits and is now over 60 years (Cystic Fibrosis Foundation, n.d.).
New vaccines (e.g. malaria, measles, Haemophilus influenzae type B [HIB], rotavirus) (NIH, 2024a; Sanaria, 2025)	**1960s** - early NIH-supported development and trials of childhood vaccines such as measles, followed by HIB conjugate, rotavirus, and malaria vaccines over subsequent decades	>99% decrease in measles, HIB, rotavirus in vaccinated children (Dempsey and Zimet, 2015; Esposito et al., 2014; Kao et al., 2014; Khabbaz et al., 2014; Temoka, 2013).
Development of coalitions to study diseases impacting children (e.g., childhood cancer, autism) (Children's Oncology Group, n.d.; NICHD, 2022)	**1950s–2000s** - from early NCI cooperative pediatric oncology groups to later NIH-supported rare disease research networks such as CARRA and similar consortia	Children across the United States benefit from specialty centers through early diagnosis, optimal interventions/treatments, and education. More children across the United States are appropriately diagnosed and receiving timely care.
Gene therapy for spinal muscular atrophy type 1 (NINDS, 2024)	**Mid-1990s** - discovery of the SMN1 gene and start of NIH-supported SMA biology and gene-targeted therapy research, leading to AAV gene therapy and antisense drugs	Life-saving treatment that improves survival, preserves motor function, and reduces lifelong disability (Mendell et al., 2017).

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TABLE 2-1 Continued

Innovations enabled by NIH funding	Approximate start of NIH-supported research*	Consequent improvements to child health
Cryotherapy and laser surgery for retinopathy of prematurity (Early Treatment For Retinopathy Of Prematurity Cooperative, 2003)	**Mid-1980s** - NEI-funded CRYO-ROP trial launched in 1986, followed by NIH-supported laser photocoagulation trials in the 1990s	Provided an intervention for a disease that previously had no treatment. Reduced rates of childhood blindness and visual impairment in premature infants (Chow et al., 2022; Cryotherapy for Retinopathy of Prematurity Cooperative Group and National Eye Institute, 1988; Lumsden and Urv, 2023).
Whole body cooling for hypoxic ischemic–ischemic encephalopathy (Shankaran et al., 2005)	**Late 1990s** - planning and initiation of NICHD Neonatal Research Network whole-body hypothermia trials, with first major results published in 2005	Reduced risk of death and severe neurodevelopmental disability. Improves survival and cogitative outcomes in affected infants (Shankaran et al., 2012).
The development and clinical implementation of clinical whole-genome sequencing (NCATS, 2024)	**Early 2010s** - NIH-funded pilots of rapid clinical whole-genome sequencing (e.g., NSIGHT and NICU rWGS studies) in critically ill infants and newborns	Dramatically improved clinicians' ability to diagnose children with rare diseases in both intensive care and outpatient settings (Brlek et al., 2024).
Prenatal surgery for myelomeningocele	**Early 2000s** - NICHD-funded MOMS trial of prenatal vs postnatal repair, launched in 2003 with NEJM results in 2011	Reduced the need for shunting and improved motor outcomes in young children (Adzick et al., 2011; Houtrow et al., 2020).
Antenatal corticosteroid therapy (NICHD, 2025g)	**Early 1970s** - first randomized trials of antenatal corticosteroids for fetal lung maturation, followed by NICHD Neonatal Research Network trials and implementation studies in the 1990s	Decreases neonatal morbidity and mortality, with substantial savings in health care costs. The use of antenatal corticosteroids for fetal maturation is a rare example of a technology that yields substantial cost savings in addition to improving health (Gilstrap et al., 1995; McGoldrick et al., 2020).

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TABLE 2-1 Continued

Innovations enabled by NIH funding	Approximate start of NIH-supported research*	Consequent improvements to child health
Very early antiretroviral therapy for infants with HIV	**Early 2010s** - NIH-supported studies of combination ART initiated within hours–days of birth (e.g., "Mississippi baby" case and subsequent early-ART cohorts)	Advances have made it possible to achieve viral remission in infants (Persaud et al., 2024).

*Decades are approximate and refer to the period when NIH-supported work that was clearly on the translational path to the listed advance began (e.g., gene discovery, first randomized control trials, or launch of NIH cooperative trials), not necessarily the very first experiment performed in each area.

NOTE: AAV = adeno-associated virus; ART = antiretroviral therapy; CARRA = Childhood Arthritis and Rheumatology Research Alliance; CF = cystic fibrosis; CFTR = cystic fibrosis transmembrane regulator; HIB = Haemophilus influenzae type B; NEI = National Eye Institute; NEJM = *New England Journal of Medicine*; NICU = neonatal intensive care unit; NIH = National Institutes of Health; PKU = phenylketonuria; rWGS =rapid whole-genome sequencing; SIDS = sudden infant death syndrome; SMA = spinal muscular atrophy.

Without established systems of oversight and ethical frameworks for research involving human subjects, questionable and unethical conduct often occurred in early research studies with children. Historical examples of ethically problematic research include cases in which children were exposed to considerable risk, often without informed permission from their parents or caregivers or sufficient scientific justification for doing so. In 1796, Edward Jenner proved the concept of vaccination by exposing individuals to cowpox and subsequently inoculating them with smallpox. Among these individuals were his own 1-year-old son and James Phipps, the son of his gardener. While the importance of the study was clear and ultimately provided Phipps with protection against smallpox, it is unclear whether his parents understood the risks or gave their permission for James to participate in the experiment (Zildzic and Zunic, 2023). In the 1930s, a researcher conducted a study with children which has been referred to as the "monster" study, in which the goal was to induce children to stutter or exacerbate existing stuttering (Silverman, 1988). Horrific and sometimes fatal studies involving children, including studies with young twins, were conducted by the Nazis during World War II. In response to these atrocities, the first international ethical guidance for research was developed, referred to as the Nuremberg code, which required voluntary informed consent from all research participants, thereby ruling out

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the participation of children (Diekema, 2006; Holocaust Memorial Museum, n.d.). In the 1950s and 1960s, controversial research was conducted at the Willowbrook State School in which healthy children with cognitive disabilities were deliberately infected with hepatitis. The principal investigator said that the study was done to address an existing widespread hepatitis infection in the institution; and they noted that inoculation led to mild disease (Krugman, 1986). On the other hand, others questioned the ethics of these studies, particularly related to the level of risk, whether it was necessary to do the study with institutionalized children with disabilities, and whether parents were freely able to decide to enroll or exclude their children (Beecher, 1966; Diamond, 1973).

In response to significant public concern about the Willowbrook hepatitis study and similar research involving children, Congress passed the National Research Act in 1974. This act created the National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research. The National Commission wrote the highly influential *Belmont Report*, which laid out principles for the ethical conduct of research for all participants, including a special focus on whether research with children could be ethically acceptable. The National Commission ultimately found that pediatric research is important and necessary to promote child health and well-being and that specific safeguards could help ensure that research with children is conducted ethically. The National Commission's work led ultimately to new U.S. federal regulations, which created institutional review boards to oversee research and provided a framework for the protection of children based on risk/benefit considerations and parental permission requirements to protect children and respect parental authority (Shah, 2021). The National Commission's work and subsequent regulations and guidelines led to the modern system of research ethics oversight that serves as a bulwark against many types of unethical research with children.

Following the National Commission's determination that pediatric health research is important and necessary to improve child health, the U.S. Congress took steps to encourage and incentivize pediatric research. In 2002 the Best Pharmaceuticals for Children Act directed the NIH to prioritize pediatric trials on medications and devices being used off-label in children. It also gave pharmaceutical companies that added pediatric labeling to their products an extension of patent exclusivity (NASEM, 2012). In 2003 the Pediatric Research Equity Act required pediatric assessments of new drugs and biologics with new active ingredients, indications, dosages, and routes of administration (Bourgeois et al., 2012). The Gabriella Miller Kids First Research Act, passed in 2013, required the director of the NIH to allocate $12.6 million annually from the 10-year Pediatric Research for FY 2014–2024 Initiative Fund to ICs to fund grants for pediatric research

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on childhood cancer and structural birth defects.[1] The Gabriella Miller Kids First Research Act extended funding until FY 2028.[2]

NIH implemented its Inclusion Across the Lifespan policy in 2019 to ensure that individuals of all ages, including children and older adults, are appropriately represented in NIH-funded research that includes human participants. This policy mandates that researchers include participants across the lifespan unless there are scientific or ethical reasons for exclusion. Applicants must provide a rationale for any age-related exclusions, and progress reports are required to include data on participant age at enrollment (NIH, 2025b). By requiring the inclusion of children in research, the policy is intended to generate data that can inform better health care decisions for pediatric populations. This approach acknowledges that many adult health conditions have roots in early life and that early interventions can have long-term health benefits.

Despite these changes in federal law and NIH policy, however, many barriers remain for pediatric research that make it difficult to address the national pediatric health crisis (Forrest et al., 2025). A deeper discussion of the intersection of ethical considerations and pediatric health research along with the committee's conclusions and recommendations can be found in Chapter 5.

THE ORGANIZATION OF NIH AND ITS CAPACITY AND SUPPORT FOR PEDIATRIC HEALTH RESEARCH

The organizational structure of NIH includes central leadership from the Office of the Director (OD) and 27 semi-autonomous institutes and centers (ICs) (Figure 2-1). Twenty-two ICs fund intramural research (NIH Intramural Research Program, 2022a),[3] and 24 ICs[4] support extramural research. In addition, NIH supports a variety of strategic initiatives that cut across diseases, systems, and populations (NIH, n.d.).

Each IC has its own research agenda, often focusing on specific diseases or body systems (NIH, 2024b). Table 2-2 lists the ICs, the year each was established, and their primary focus areas.

All of the ICs support pediatric research, although the absolute dollar amount as well as the proportion of its budget varies widely among

[1] H.R.2019 – 113th Congress (2013–2014); Gabriella Miller Kids First Research Act, 2014.

[2] H.R.3391 – 118th Congress (2023–2024); Gabriella Miller Kids First Research Act 2.0, 2025.

[3] 22 ICs provide funding for intramural research while the Clinical Center serves to house intramural research (see Figure 2-2).

[4] 24 of the 27 ICs provide research funding, and the National Institute of General Medical Sciences, one of the funding ICs, exclusively funds basic research not conducted at the Clinical Center.

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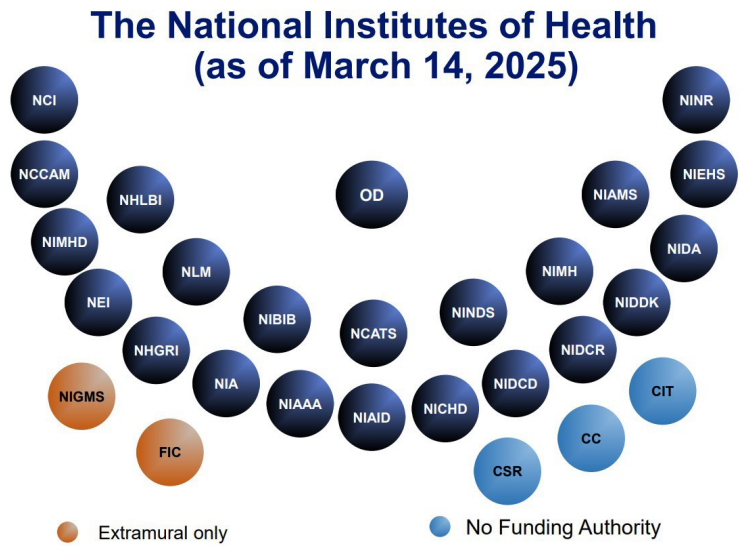

FIGURE 2-1 The National Institutes of Health, as of March 14, 2025.
SOURCE: Presentation by Diana Bianchi, M.D., director of the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), at the Open Session: Committee on Strategies to Enhance Pediatric Health Research Funded by NIH, hosted by the National Academies on March 14, 2025.

them. The Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) allocates the largest portion of its budget to pediatric research (historically around 60 percent) and is the largest contributor to NIH's pediatric portfolio (17 percent for FY 2024 and approximately 18 percent in FY 2021). Other ICs, including several with budgets substantially larger than NICHD, such as the National Institute of Allergy and Infectious Diseases (NIAID), the National Cancer Institute (NCI), the National Institute of Mental Health (NIMH), and the National Heart, Lung, and Blood Institute (NHLBI), also allocate significant funds to pediatric health research (see Figure 3-6) (NICHD, 2025a,b,c,e,f). For example, the NCI funds extensive research on pediatric cancers, including initiatives to mitigate shortages of pediatric cancer drugs as part of the Cancer Moonshot initiative (NCI); the NHLBI supports studies on congenital heart defects and blood disorders, such as sickle cell disease, aiming to enhance diagnosis and treatment in pediatric populations(NHLBI); and the NIAID funds research on pediatric infectious diseases, including studies on vaccines and treatments for conditions such as pediatric HIV/AIDS (NIAID). There is robust support of pediatric research across most ICs, with all the ICs funding some research categorized as "pediatric," according to

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TABLE 2-2 The 27 NIH ICs, Year of Establishment, and Primary Focus Area, as of March 21, 2025

Institute/Center	Year Established	Primary Focus Area
National Cancer Institute (NCI)	1937	Cancer research and training
Center for Scientific Review (CSR)[a, b]	1946	Peer review of NIH grant applications
National Heart, Lung, and Blood Institute (NHLBI)	1948	Heart, lung, and blood diseases
National Institute of Allergy and Infectious Diseases (NIAID)	1948	Infectious, immunologic, and allergic diseases
National Institute of Dental and Craniofacial Research (NIDCR)	1948	Dental, oral, and craniofacial health
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)	1948	Diabetes, metabolic, digestive, and kidney diseases
National Institute of Mental Health (NIMH)	1949	Mental illnesses
National Institute of Neurological Disorders and Stroke (NINDS)	1950	Neurological disorders
NIH Clinical Center (CC)[b]	1953	Clinical research facility and patient care
National Library of Medicine (NLM)	1956	Biomedical information resources
Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)	1962	Child health, development, and reproductive health
National Institute of General Medical Sciences (NIGMS)[a]	1962	Basic biomedical research
Center for Information Technology (CIT)[a, b]	1964	Biomedical computing and IT
John E. Fogarty International Center (FIC)[a]	1968	Global health research and training
National Eye Institute (NEI)	1968	Eye diseases and vision research
National Institute of Environmental Health Sciences (NIEHS)	1969	Environmental health sciences
National Institute on Alcohol Abuse and Alcoholism (NIAAA)	1970	Alcohol-related research
National Institute on Aging (NIA)	1974	Aging and age-related diseases
National Institute on Drug Abuse (NIDA)	1974	Drug abuse and addiction

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TABLE 2-2 Continued

Institute/Center	Year Established	Primary Focus Area
National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)	1986	Arthritis, musculoskeletal and skin diseases
National Institute of Nursing Research (NINR)	1986	Scientific basis of nursing practice
National Institute on Deafness and Other Communication Disorders (NIDCD)	1988	Communication disorders including hearing and speech
National Human Genome Research Institute (NHGRI)	1989	Human genome structure and function
National Center for Complementary and Integrative Health (NCCIH)	1999	Complementary and integrative health practices
National Institute of Biomedical Imaging and Bioengineering (NIBIB)	2000	Biomedical imaging and bioengineering
National Institute on Minority Health and Health Disparities (NIMHD)	2010	Minority health and health disparities
National Center for Advancing Translational Sciences (NCATS)	2011	Translational science and therapeutic development

NOTES: [a] = ICs that do not fund intramural research; [b] = ICs that do not fund extramural research; IC = institutes and centers, NIH = National Institutes of Health.
SOURCE: NIH (2024b).

the NIH Research, Condition, and Disease Categorization (RCDC) system. Chapter 3 provides a more in-depth analysis of pediatric health research funding across the ICs.

The NIH Clinical Center: A Critical Resource for Advancing Health Across the Life Course

The NIH Clinical Center (CC) is a vibrant hospital system hosting more than 2,000 health care professionals and investigators, who engage in scientific research to advance human health (NIH, 2021; Sekar, 2025). Opened in 1953, the CC's primary innovation was the co-location of patient care and research laboratories, thus facilitating research intended to advance diagnosis, disease characterization, and treatment effectiveness (NIH Clinical Center, n.d.-a). Originally conceived as an inpatient facility, the CC added an outpatient infrastructure in the 1980s (NIH Clinical Center, n.d.-b). For more information about the CC and its evolution, please see Appendix D.

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The Clinical Center's Vision for Pediatric Research

The Clinical Center is uniquely positioned to conduct research in several key areas of pediatric science due to its specialized infrastructure, collaborative networks, and focus on underserved populations, making the Clinical Center a key component of any NIH strategy to advance innovative pediatric research, which is discussed in detail in Chapter 3.

The NIH Pediatric Research Consortium

The NIH Pediatric Research Consortium (NPeRC) was established in 2018 to enhance collaboration and coordination in pediatric research across the NIH's ICs (NICHD, 2025e). The NPeRC membership includes representatives from 25 institutes and centers, five offices, and two research programs (All of Us and ECHO; both discussed in depth in Chapter 4). NPeRC focuses on harmonizing pediatric research activities, identifying gaps and opportunities in the pediatric research portfolio, and taking advantage of existing resources and expertise. It currently operates without dedicated funding and does not directly support research projects or initiatives. The consortium convenes several times a year to discuss scientific opportunities and potential new areas of collaboration, including efforts to enhance research training for the next generation of pediatricians. Among its accomplishments, NPeRC has facilitated discussions on common areas of interest and needs in pediatric research, which have contributed to the development of a child health agenda within NIH. NPeRC has also collaborated with the Helping to End Addiction Long-term Initiative to address pediatric pain research and has engaged in efforts to bolster research on improving the transition from pediatric to adult health care. Additional findings, conclusions, and recommendations about the NPeRC program can be found in Chapter 4.

Intramural Pediatric Research at the NIH

NIH's Intramural Research Program (IRP) funds biomedical research conducted by NIH-employed investigators within its own laboratories and the Clinical Center (Cryotherapy for Retinopathy of Prematurity Cooperative Group and National Eye Institute, 1988). With around 1,200 principal investigators and over 4,000 postdoctoral fellows, the IRP is unique: rather than relying on competitive grants like the extramural program, it receives direct appropriations from Congress, making up approximately 11 percent of the NIH budget, which in recent years has translated to around $4 billion annually (NIH, 2025a; NIH Intramural Research Program, 2022d). This internal funding mechanism makes it possible to carry out long-term,

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high-risk/high-reward projects, which are often resource-intensive (such as longitudinal or population-based studies) and which would be challenging to support through standard extramural grant mechanisms.

To ensure accountability and scientific excellence, IRP investigators undergo rigorous review cycles approximately every 4 years. These reviews are conducted by external boards of scientific counselors (HELP, 2024; NIH IRP, 2022). Reviews include in-person site visits and focus on both past accomplishments and future research plans. Evaluation criteria include three factors: importance of the research, rigor and feasibility, and expertise and resources. Final recommendations are presented to the NIH deputy director for intramural research, who discusses it with the relevant scientific director. When decisions on funding levels, staffing, and promotions are finalized, they are provided to the relevant IC council.

Ten percent of intramurally funded projects focus on pediatrics, based on available data (see the data analyses in Chapter 3). Some recent intramural research includes identifying new therapies for pediatric cancers, the relationship between maternal folic acid levels and childhood asthma risk, and the creation of robotic exoskeletons to improve walking posture among children with cerebral palsy (NIH Intramural Research Program, 2016, 2020, 2022c).

Extramural NIH-Supported Pediatric Research

Extramural research applications go through a rigorous process to receive funding from NIH. Once a research application has been submitted, it first undergoes an administrative review to confirm that the application complies with submission requirements and eligibility criteria. Next, the application is assigned to a study section, a panel of experts in relevant scientific fields who evaluate the scientific merit of the proposal. During the peer review, reviewers provide scores and written critiques focusing on several criteria, including significance, innovation, and approach. The application is assigned a percentile score that ranks its overall scientific quality relative to other applications in the same review cycle. In parallel, the application is assigned to the relevant NIH IC which in turn assigns a program officer who manages a relevant portfolio of research. That program officer does not plan any direct role in the peer review process to maximize integrity and independence of review but is available to an applicant following completion of peer review to help interpret the review results. Program staff considers program relevance, strategic priorities, and budget considerations when making funding recommendations. An application considered for funding advances to the IC's advisory council or board, where it undergoes a second level of review (NIH Grants & Funding, 2025). The advisory board/council considers the IC's goals and needs and advises the IC director

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concerning funding decisions. Based on committee discussions with IC leaders, most ICs prioritize those grants with the most meritorious scores, irrespective of pre-conceived "targets" for types of research (e.g., ICs do not currently have pre-determined target proportions of "pediatric"-focused grants to fund) (NIH Office of Extramural Research, 2024).

Investigator-initiated awards are a core component of the NIH funding portfolio. These grants are designed to support researchers who propose their own innovative ideas and hypotheses without predefined project constraints (NIGMS, 2025; NIH Grants & Funding, n.d.). These awards provide flexibility for researchers to explore novel scientific questions, develop new methodologies, or test emerging theories aligned with their expertise and interests. Typically awarded through R programs (research project grants such as R01 or R21), these grants are highly competitive and require detailed proposals demonstrating scientific rigor, significance, and innovation. When specific funds have been set aside for particular research topics (e.g., pediatric asthma), specific request for applications (RFAs) are developed, with submitted grants going through a process similar to that of investigator-initiated awards. Similarly, notices of special interests (NOSIs) allow ICs to solicit research applications that focus on particular topics (NIAID, 2023).[5] For NOSIs, no specific funds are prospectively set aside for funded applications. In addition to NIH ICs, the OD has numerous funding opportunities known as NIH Director's awards (e.g., Early Independence Award, Transformative Research Award, New Innovator Award, and Pioneer Award) that can be used to fund investigator-initiated pediatric research (NIH Office of Strategic Coordination, 2024a, 2024b, 2024c, 2024d).

Research Center Grants Supporting Pediatrics

NIH-supported research center grants have played an important role in advancing pediatric research by promoting multidisciplinary collaboration, resourcing shared infrastructure, and supporting long-term scientific programs focused on child health. These center grants—such as the P30 (Center Core), P50 (Specialized Center), and U54 (Specialized Collaborative Center)—have enabled institutions to bring together basic, clinical, and translational scientists around shared thematic priorities in pediatrics, including rare diseases, developmental disorders, pediatric cancers, and environmental influences on child health. By supporting centralized cores, career development opportunities, and pilot project funding, NIH center

[5] Going forward, NIH will no longer use NOSIs. Instead, NIH will use Highlighted Topics, which are not replacing NOSIs but serve a similar purpose. More information is available: https://grants.nih.gov https://grants.nih.gov/funding/find-a-fit-for-your-research/highlighted-topics.

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grants nurture innovation and sustain research ecosystems that might otherwise be difficult to maintain through individual investigator-initiated grants alone. Many of these centers have also emphasized community engagement and training of the next generation of pediatric investigators, thereby ensuring that the infrastructure not only generates high-impact science but also builds capacity for the future of child health research. Some exemplary centers and their contributions to child health are listed in Table 2-3. Additional information about how ICs and the OD prioritize and fund research centers to promote child health can be found in Chapter 4.

TABLE 2-3 NIH Research Center Awards

Exemplary Center	Focus	Highlighted Contribution to Child Health
Intellectual and Developmental Disabilities Research Centers (Kennedy Krieger, 2025)	Intellectual and developmental disabilities research infrastructure	Improved diagnosis, support, and clinical care of children with neurodevelopmental disorders
Child Health Research Career Development Awards (CHRCDAs) (NICHD, 2023)	Career development for pediatric researchers	Trained pediatric physician-scientists who drive innovations in child health research
Pediatric Clinical and Translational Research Centers (UPMC CHP, 2025)	Translation of basic research into clinical applications in children	Enabled early-phase trials and translational studies tailored to pediatric populations
Pediatric Environmental Health Specialty Units (PEHSU, 2025)	Focus on addressing environmental health issues affecting children	Provided expertise to prevent and respond to environmental exposures affecting children
Pediatric HIV/AIDS Cohort Study (PHACS, 2025)	Research on long-term effects of HIV and its treatment in children	Improved long-term care and outcomes for children affected by perinatal HIV and its treatments
Pediatric Heart Network (Pediatric Heart Network, n.d.)	Multi-center studies on congenital and acquired heart disease in children	Developed standards of care for diagnosis, treatment, and outcomes in children with congenital and acquired heart disease

NOTE: NIH = National Institutes of Health.

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THE BROADER LANDSCAPE: OTHER PEDIATRIC RESEARCH FUNDERS

The U.S. pediatric research funding landscape is complex. Beyond the NIH, there is an array of other federal, state, and private funders. Some pediatric research involves collaborations between NIH ICs and other federal agencies, such as the Centers for Disease Control and Prevention, Agency for Healthcare Research and Quality, Health Resources and Services Administration, Substance Abuse and Mental Health Services Administration, Food and Drug Administration, Environmental Protection Agency, National Institute of Justice, National Science Foundation, Department of Defense, Department of Transportation, Department of Education, the Indian Health Service, and other agencies within the Department of Health and Human Services. State and local governments as well as private funders such as health systems, foundations, industry, and voluntary health associations provide critical supplementary support for pediatric health research. NIH, however, remains the primary funder of pediatric research through its extramural funding, collaborations, and extensive intramural research programs.

Changes in the federal budget and policies may influence the future funding environment for biomedical and public health research, including pediatric health research. Congressionally approved cuts to Medicaid will likely have profound effects on pediatric research. More than half of the patients treated in clinical programs in academic pediatric departments and children's hospitals are covered by Medicaid or the Children's Health Insurance Program (CHIP), and the approved reductions in reimbursement will represent the loss of tens to hundreds of millions of dollars for these institutions. Thus, the ability of these institutions to support pediatric research at the level they have done in the past will be severely compromised. The contributions of these other funders cannot replace the sustained, targeted NIH funding for pediatric health that has led to many significant breakthroughs in the past and is essential for ensuring that the United States remains a leader in pediatric health research into the future.

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3

Current NIH Pediatric Research Portfolio

"We will look to the National Institute of Child Health and Human Development for a concentrated attack on the unsolved health problems of children and of mother–infant relationships. This legislation will encourage imaginative research into the complex processes of human development from conception to old age. . . For the first time, we will have an institute to promote studies directed at the entire life process rather than toward specific diseases or illnesses."

—John F. Kennedy, October 17, 1962[1]

DELINEATING PEDIATRIC RESEARCH AT NIH

At the National Institutes of Health (NIH) "pediatric research" is defined in different ways, depending on the needs of different institutes or centers (ICs), consortia, or reporting groups. As noted in Chapter 1, NIH defines a child for the purposes of research participation as any individual younger than 18 years. An internal memo from NIH characterizes pediatric *research* as "studies in all categories of biomedical research (basic, clinical, epidemiologic, behavioral, prevention, treatment, diagnosis, as well as outcomes and health services) that relate to diseases, conditions, or the health/development of neonates, infants, children, and adolescents up to age 21" (Gitterman et al., 2023, p. 797). The contrast between the two reflects the distinction between necessary legal protections for individual human participants in research and a developmental approach to defining

[1] NICHD (2019).

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the scope of "pediatric research." Also discussed in Chapter 1, the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development reported to the committee that "pediatric research" may encompass several distinct areas of study: research in which participants do not specifically *exclude* children; research in which participants specifically *include* children; or research involving children with a clinical or public health relevance focus on conditions or developmental processes that typically originate and manifest in childhood. (It is also important to note that this research nearly always involves the participation of caregivers as guardians of the pediatric research participants—whether they are reporting information to investigators or ensuring safe transportation to clinical trial sites, successful pediatric research could not occur without them.) While each of the various definitions in use has merit, it is essential to have a singular definition across NIH to better describe and evaluate pediatric research.

Recommendation 3-1: The National Institutes of Health should adopt and apply a consistent agency-wide definition of pediatric research. The definition should recognize that pediatric research may include studies that: (1) involve data or specimens from or about infants, children, adolescents, or young adults; (2) inform the prevention, diagnosis, or treatment of conditions affecting these populations; (3) advance the health, well-being, or resilience of children, adolescents, or young adults; or (4) investigate childhood origins of adult health conditions.

NIH DATA SYSTEMS FOR MONITORING PEDIATRIC RESEARCH SUPPORT

The National Institutes of Health maintains several systems for public reporting on its funded research projects; these systems can be found on the Research Portfolio Online Reporting Tools (RePORT) website.[2] One of these systems is the RePORT Expenditures and Results (RePORTER) which allows users to search NIH-funded research projects using a wide range of fields, such as principal investigator, project location or congressional district, funding IC, funding mechanism, award type, project number, spending category (of RCDC; see below), and much more; users can also view publications and patents resulting from NIH funding as well as research funded by other federal agencies, such as the Administration for Children and Families, the Agency for Healthcare Research and Quality, the Centers

[2] Both systems are found at https://report.nih.gov, but the "Categorical Spending webpage," as it is frequently identified in this report, can be found at https://report.nih.gov/funding/categorical-spending#/. It is sometimes referred to as the RCDC webpage. Advanced searches in RePORTER can be performed at https://reporter.nih.gov/advanced-search.

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for Disease Control and Prevention, the Health Resources and Services Administration, the Food and Drug Administration, and the Department of Veterans Affairs (NIH RePORT, n.d.-a, 2025). Another system, which is the focus of this chapter, is the Research, Condition, and Disease Categorization (RCDC) system, developed specifically to comply with legislative mandates to report the spending of appropriated funds on specific research topics, known as categories. These categories do not constitute a taxonomy, and many of them overlap. Both of these systems feature bulk export options for the public to download the data (RePORTER has a separate webpage for ExPORTER, which "provides bulk administrative data found in RePORTER to the public for detailed analyses or to load into their own data systems . . . generally in CSV formats"), although the number of search fields and amount of information included differs between the systems. The Categorical Spending webpage exports offers 11 fields when exporting bulk data by spending category, including spending category, funding IC, project number and title, principal investigator name, and funding amount. By contrast, RePORTER has 46 fields, which include the 11 fields in Categorical Spending webpage exports along with other fields providing much more extensive detail about each project.[3] As part of its charge, the committee was asked to examine "the methods and rationale involved in categorizing projects as 'Pediatrics' research using the [RCDC] process." This chapter presents the committee's analysis of the RCDC reporting system as it appears to the public.

How the Research, Condition, and Disease Categorization System Works

Initiated in 2008 in response to the NIH Reform Act,[4] RCDC ensures uniform coding of research grants and activities across NIH ICs so that the public can analyze NIH appropriations and identify trends and gaps in research funding (NASEM, 2024). RCDC categories can be created at the request of Congress, the White House, advocacy groups, and NIH leadership (NIH, n.d.).

Assigning a grant to one or more of the RCDC spending categories involves several steps. First, the NIH Automated Indexing Service scans each research project's title, abstract, specific aims section, and public health relevance section for "concepts" that are included in the RCDC Thesaurus.

[3] See https://report.nih.gov/funding/categorical-spending#/ to export data by RCDC term within the Categorical Spending webpage and https://reporter.nih.gov/exporter to export and RePORTER project data by fiscal year.

[4] P.L. 109-482, *The National Institutes of Health Reform Act*, 109th Cong., January 15, 2007.

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The thesaurus is based on several established biomedical vocabularies and can be "anything ranging from specific diseases, gene names, types of diagnostic tools, therapeutic techniques, to patient populations" (NIH RePORT, n.d.-c). A project typically has many concepts assigned to it. Once this text analysis is done, a project index is produced with the full list of concepts and the weighted number of times each appears in the text.

The project index is then evaluated against the RCDC "category fingerprints," which are a set of concepts identified by scientific experts as being important in determining whether a given research project should be publicly reported as relating to a category. Each "category fingerprint" includes a set of concepts (see Figure 3-1) and may also incorporate administrative business rules. The set of concepts in the fingerprint is identified by a panel of scientific experts drawn from the NIH ICs. These scientific experts set the parameters for each category, and review example projects to determine whether that research should be publicly reported as belonging to a category. This process results in a weighted set of concepts known as a fingerprint. When there is sufficient overlap between the concepts indexed by the project and the category fingerprint, the project will generally be assigned to the category. The threshold for determining sufficient overlap is developed empirically to maximize the validity and reliability of the category—that is, to minimize the occurrence of false positive and false negative results. After this process is completed, projects in the resulting categories are validated by IC experts to ensure that the fingerprint is identifying projects correctly according to the parameters determined by the scientific reviewers. The validation process also includes an AI component as a secondary quality check and to minimize burden on the scientific experts while ensuring that the experts' review contributes to the category validity.

RCDC categories are neither exclusive nor exhaustive. A single project can have multiple RCDC categories assigned to it. For example, "pediatrics," "childhood injury," and "unintentional childhood injury" are all distinct categories (Gitterman et al., 2022), and a project studying unintentional poisonings among children would likely be categorized under all three. This is in part because the categories can reflect conditions or diseases of significant public and or Congressional interest. By nature, the categories overlap, and the categories do not encompass all types of biomedical research (NIH Grants and Funding, 2025a; NIH RePORT, n.d.-c). As of fiscal year (FY) 2024, RCDC had 327 total categories of research, condition, and disease areas. For FY 2024, the committee found there were 310 spending categories assigned to projects that were also assigned to the "Pediatric" category.

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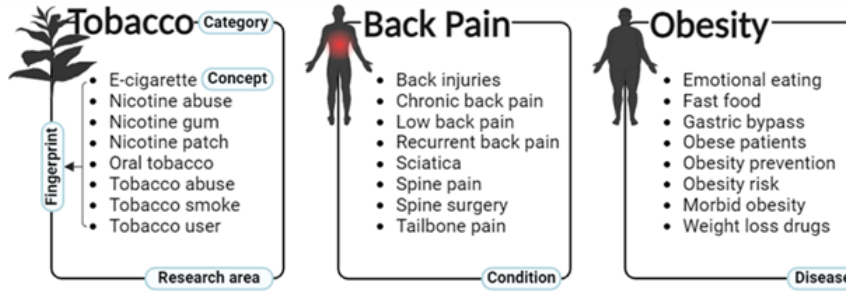

FIGURE 3-1 Examples of category fingerprints for the tobacco, back pain, and obesity RCDC categories.
NOTE: RCDC = Research, Condition, and Disease Categorization.
SOURCE: NIH (n.d.).

The RCDC system describes the Pediatric category as including "research on health conditions that originate during the embryonic period to age 21, [along with] congenital and developmental diseases" (NIH Grants and Funding, 2025b). Some categories use business rules beyond text analysis to include or exclude projects—e.g., using metadata-based rules such as grant codes, funding announcements, or NIH institute (NIH, n.d.). Category relationships are employed to include projects from "narrower" categories into broader ones (referred to as a child–parent relationship) without changing category fingerprints. For example, the following child categories are automatically assigned to the parent Pediatric category: Adolescent Sexual Activity, Child Abuse and Neglect Research, Childhood Obesity, Conditions Affecting the Embryonic and Fetal Periods, Congenital Heart Disease, Congenital Structural Anomalies, Duchenne/ Becker Muscular Dystrophy, Pediatric—AIDS, Infant Mortality, Pediatric Cancer, Pediatric Cardiomyopathy, Perinatal Period—Conditions Originating in Perinatal Period, Rett Syndrome, Spinal Muscular Atrophy, Youth Violence, Childhood Injury, and Underage Drinking (NIH Grants and Funding, 2025b).

GAPS AND OPPORTUNITIES FOR THE RCDC PEDIATRIC SPENDING CATEGORY

Per the committee's statement of task, the committee evaluated the RCDC pediatric spending category in order to assess its utility for public reporting of pediatric research funded by NIH. RCDC is a consistent, NIH-wide classification framework which standardizes how research areas are defined and reported across ICs. The Categorical Spending website is user-friendly, allowing for the selection of individual RCDC categories to be examined. In addition, the website supports data downloads of these

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category-specific analyses by year. The categorization process is scalable and adaptable. Because the RCDC categorization process uses automated text mining rather than relying on manual review, as NIH's portfolio expands the categorization process can handle an increase to volume without loss of performance. In addition, RCDC categories can be added as needed based on Congressional and IC priorities and public input. Finally, the system allows projects to be tagged under multiple categories, effectively capturing the interdisciplinary nature of biomedical research.

However, a few gaps limit the utility of the categorical spending webpage and interface. Regarding the technical aspects of the system, the Categorical Spending webpage supports single-spending-category downloads only, even though the majority of projects are tagged with multiple categories. Thus, it lacks functionality to query RCDC category intersections (e.g., *Pediatric* × *ADHD*).

The content of the Categorical Spending webpage could also be enhanced. The committee found that important analytical fields are missing. It is important to note that many of these fields exist in some form when using the RePORTER webpage advanced search function. Nevertheless, it would be useful to be able to access these fields in the Categorical Spending webpage directly. Clear designation of whether a project is intramural or extramural research is lacking, and a designation for clinical, basic, and translational research is missing. There is an RCDC spending category for clinical research, but none for basic or translational sciences. (There is a term for basic behavioral and social science research, but this does not cover the full range of basic science.) (NIH Grants and Funding, 2025b). Therefore, the fields available in the Categorical Spending webpage were insufficient to identify projects that are classified as basic, clinical, or translational research. The committee was attentive particularly to the inability to place research into the basic, clinical, and translational categories as these categories were also used in several NIH presentations on the subject, although other important research categories were also absent. In RePORTER, various research categories, including basic, clinical, translational, dissemination and implementation sciences, and health services research, can be found under the Project Terms field, though there is no equivalent search field within the interface or data export in the Categorical Spending webpage. Some of these terms may have overlap (i.e., the spending category Clinical Research includes health services research), and, at a minimum, it would be useful to be able to distinguish basic science research from clinical research in the context of the Categorical Spending webpage.

It is not currently possible to determine funding by category with the sort of specificity that might be useful for policy making, prioritization of resource distribution, or other impactful decisions made by industry, academia, government, and nonprofit leaders. As an example, one might

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hope that funding is reasonably commensurate with the burden of disease, injury, and mortality in each stage of child and adolescent development. Knowledge about NIH funding during major stages of development would provide data to confirm that this is the case or to adjust funding patterns otherwise. Similarly, scholars recognize the value of conducting a range of research that falls into the basic to clinical to translational science paradigm, working toward interventions that improve human health. The current categorization system does not classify research into those categories, preventing an analysis of spending along the basic to translational research spectrum.

It is also not feasible to disaggregate projects by sub-categories such as child development stages (e.g., infant, child, adolescent). When describing pediatric developmental stages, the American Academy of Pediatrics uses the following categories: prenatal; birth–12 months; 1–3 years; 3–5 years; 5–12 years; 12–18 years; 18–21 years (AAP, n.d.). For research purposes, it is the committee's view that, in addition to the categories listed above, adding a category for neonates (0–28 days) is critical as more research for this specific age group is greatly needed. For example, very little is known about the efficacy, safety, and dosage for medications for newborns, and, as a result, over 90 percent of medication for them is prescribed off-label or off-license (Modi, 2023). Additionally, for research purposes, the committee feels it important to extend the 18–21 category to 18–24 years as significant biological and neuropsychological maturation and social transitions occur during the early adulthood period (Hochberg and Konner, 2019; Sawyer et al., 2018), with some aspects of executive function developing well into the 20s (Ferguson et al., 2021; Shulman et al., 2015).

The committee also observed the use of eponyms in the classification system. Because eponyms are often local or historical, they may be familiar in one region but confusing or unknown to clinicians and patients elsewhere. They can also complicate reporting, since coders have to memorize arbitrary names instead of terms that encode pathophysiology. Using descriptive terminology for conditions improves clarity, supports communication, and better aligns with modern, mechanism-based medicine. The committee found variations and inconsistencies in spelling or phrasing of RCDC terms (despite a standard RCDC term list), creating redundant or mismatched category names, which requires manual cleaning and makes automated processing by external researchers difficult. Data for RePORTER come from the Electronic Research Administration's (eRA's) IRDB (IMPAC II Reporting Database—NIH grant data for reporting) as well as from iEdison (inventions), Clinicaltrials.gov, NIH intramural database, and PubMed, while data from RCDC appear to come from eRA alone; this discrepancy likely accounts for some differences or lack of updates between older category names assigned to projects (NIH RePORT, n.d.-b).

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When the committee merged its data between its RCDC and RePORTER search, it found several discrepancies:

- Year-to-year variation: Minor grammatical or word order changes that occurred over time in the RCDC were not reflected retroactively (e.g., ADD updated to ADHD).
- Category updates and renaming: Some RCDC categories have changed names (e.g., Pneumonia & Influenza became Pneumonia and Influenza), but these updates are not consistently reflected in older RePORTER data.

It would, of course, be useful if the RCDC system were a complete and exhaustive taxonomy of research, condition, and disease categories of projects funded by NIH, but this effort would prove to be immensely time-consuming for NIH staff, and, absent that, the committee is issuing the recommendation below to specifically enhance pediatric research development.

Conclusion 3-1: The National Institutes of Health (NIH) Research, Condition, and Disease Categorization (RCDC) system is a valuable resource for public reporting of National Institutes of Health–sponsored research projects. The system's value would be improved by enhancing its specificity and validity for pediatric research as well as its transparency and technical application.

Recommendation 3-2: The National Institutes of Health (NIH) should improve the Research, Condition, and Disease Categorization (RCDC) system to better assess funding for pediatric research by (a) adding the new spending categories and (b) improving the Categorical Spending webpage in RePORT.

The following new spending categories should be updated:

a. **Add pediatric developmental stages: during pregnancy, neonate (≤28 days), infant (<1 year old), early childhood: age 1–2 years (1- to 2-year-olds), age 3–5 years (3- to 5-year-olds), middle childhood (6- to 11-year-olds), adolescence (12- to 17-year-olds), and young adults (18- to 24-year-olds).**
b. **Add a Basic Science spending category and ensure its validity.**
c. **Add new terms for pediatric health conditions to better reflect the breadth of pediatric health conditions. The process to determine these new terms should include child health expertise from each institute and center (IC).**
d. **To improve specificity of diagnostic labeling, consider switching eponyms to indicate diseases—e.g., change Cooley's Anemia to "Beta Thalassemia Major."**

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To improve the Categorical Spending (RCDC) webpage in RePORT, NIH should:

a. **Publish a reference paper that clearly and comprehensively describes the RCDC classification methodology with sufficient detail that it can be replicated or a similar assessment can be done by the scientific community. Update the methodology report whenever the methodology changes.**
b. **Evaluate the validity of key RCDC terms, such as "pediatric" and update the classification to improve accuracy and remove possible biases.**
c. **Allow more than one RCDC term to be exported in a specific project. (This functionality currently exists in RePORTER, but not RCDC.)**
d. **Enable download of all projects funded by the NIH for a given year.**
e. **Add a flag to clearly identify extramural, intramural, and, specifically, Clinical Center research and ensure that this information is available for all research projects.**
f. **Add a search field that links projects to their respective notice of funding opportunity, program announcement, program announcement with special review, request for application, notice of special interest, or request for proposal.**

Pediatric Research Project Audit

To evaluate the classification accuracy of the RCDC category Pediatric and examine a sample of pediatric research projects funded during the 10-year study period, the committee randomly sampled 20 projects per year assigned the Pediatric category for each of the 10 years (total number of projects was 200; the total number of projects in a given year ranged from about 9,000 to 12,000). A single committee member reviewed the title, abstract, and public health relevance statement for the 200 projects and summarized the findings. Projects for loan repayment and training grants were excluded. Each project was assessed for its pediatric relevance (whether or not it was a study that focused on pediatric health conditions that affect children and youth <21 years old), whether or not it could be considered pediatric basic science (i.e., research not involving data collected from human subjects), and the specific health conditions addressed by the project. Data were entered into a RedCap form and analyzed.

This audit found that 12 of 200 (6 percent) projects categorized as pediatric research by the RCDC system had no public-facing evidence of being pediatric research as determined by the pediatric scientific expert conducting the review of abstracts. Some of the incorrectly classified projects

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focused exclusively on maternal health during pregnancy and appeared to exclude pediatric outcomes or child health assessment. Examples include a study on interpersonal psychotherapy for major depression following a perinatal loss, promoting maternal mental health and well-being for mothers of infants in neonatal intensive care, and a study that examined mindfulness-based interventions for poor maternal sleep quality during pregnancy. While it can be argued that any of these subjects may affect the health of a fetus or infant, such studies were excluded when they did not appear to assess those outcomes.

Of the 188 projects classified as pediatric research, 83 (44 percent) had a component of or were fully characterized as pediatric basic science. These projects included animal studies, work with human cell lines, stem cell research, and other methods that did not directly collect or analyze data from human subjects but did address health conditions relevant to children and adolescents. Half (50 percent) of the 188 projects indicated in publicly accessible materials that they would enroll children and youth <21 years old.

Data from the NIH Office of Budget

The NIH Office of Budget oversees financial planning and reporting across all NIH ICs. As part of its external reporting, the office provides publicly accessible data that detail NIH's historical and current funding trends (NIH Office of Budget, n.d.-b). Available data include annual budget request summaries; annual spending by IC, mechanism, and research category; and archived appropriations data (NIH Office of Budget, n.d.-a). These data were used by the committee to obtain the expenditures for the NIH overall and by IC, excluding the line item for administrative services.

It may pose a challenge for NIH to implement these changes to the RCDC system, as they will each require financial and staffing resources. However, the committee maintains that updates to RCDC are paramount to enhancing the value and impact of pediatric research supported by NIH and to improving access and transparency to policy makers and the public. Improved spending categories would allow NIH and external stakeholders to see the true scale of investment in pediatric research. It would support data-driven policy and funding decisions as well as prioritization of allocated resources by industry, nonprofit entities, and academia. In addition, with enhanced transparency, data, and accountability, NIH could track, monitor, and demonstrate through targeted and broad communications the positive outcomes of its investments in pediatric research. Finally, if these proposed changes to the RCDC system were implemented, they would highlight underfunded or overlooked areas in pediatric research and enable strategic prioritization of research initiatives. In short, accurate, consistent

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data would make it easier to assess and understand the impact of NIH investments on pediatric health outcomes over time.

DATA ON PEDIATRIC RESEARCH FUNDING BY NIH

Overview

Part of the committee's charge in its statement of task was to use its review of the RCDC process to inform recommendations on improving NIH's overall support of pediatric research. A dataset of NIH-funded pediatric research projects spanning a 10-year study period, from FY 2015 through FY 2024, was constructed by exporting data from the Categorical Spending webpage. Projects with the RCDC category of Pediatric were considered to be pediatric research. RCDC files were linked to records in RePORTER using the Application ID, which uniquely identifies a project. This linkage was done to obtain all RCDC categories assigned to each project. The Categorical Spending webpage allows for search and export of research projects only one category at a time, and virtually all projects are assigned multiple RCDC categories. The funding IC variable in RePORTER was used selectively when it provided more specific information than RCDC. For example, some projects in RCDC do not list a funding IC because they are tagged under NIH-wide initiatives. Those same projects in RePORTER are tied to specific ICs (e.g., the National Institute of Diabetes and Digestive and Kidney Diseases, the National Institute of Allergy and Infectious Diseases, and the Eunice Kennedy Shriver National Institute of Child Health and Human Development). In those cases, the committee pulled the funding IC from RePORTER. Data were accessed between May 2 and July 15, 2025.

To adjust research project expenditures for inflation, actual dollars were converted to constant 2020 dollars using the Biomedical Research and Development Price Index (BRDPI) published by the Bureau of Economic Analysis of the Department of Commerce (BLS, n.d.; NIH Office of Budget, 2025). Specifically, nominal outlays for each fiscal year (FY 2015 to FY 2024) were multiplied by the ratio of the annual average BRDPI for 2020 to the BRDPI for the corresponding fiscal year, thereby removing the effects of general price inflation and facilitating comparisons over time. Certain multi-project grant mechanisms—such as P01, P50, P30, P60, U19, and U54—include multiple, independently funded subprojects. In the NIH reporting systems, each subproject and supplement is assigned its own identifier and reported as a unique project (NIH, n.d.). For the committee's analysis, each subproject was considered as a separate project.

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Overall Spending Trends

NIH expenditures for research, excluding administrative costs, rose from $28.5 billion in FY 2015 to $46.1 billion in FY 2024, a 62 percent increase (see Figure 3-2). However, inflation-adjusted growth in expenditures flattened beginning in FY 2020, and real growth over the 10-year period was just 26 percent.

Similar trends occurred with pediatric research (see Figure 3-3). Actual pediatric research expenditures increased from $3.5 billion in FY 2015 to $6.2 billion in FY 2024, a 70 percent increase. In parallel with overall NIH research funding, a plateau in inflation-adjusted funding was observed beginning in FY 2020, and real growth during the 10-year period was just 32 percent.

The total pediatric research expenditures, based on the pediatric RCDC data, in FY 2024 were $87 per child under 18 years old in the United States. As a proportion of NIH, non-administrative expenditures, pediatric research accounted for a constant level of about 13 percent from FY 2015 to FY 2024 (see Figure 3-4). This contrasts with the fact that children and

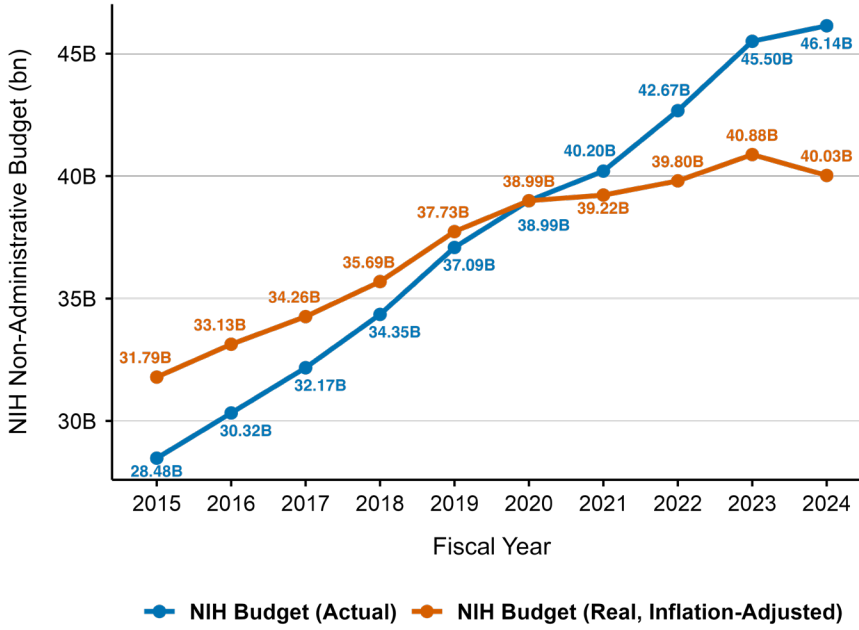

FIGURE 3-2 Total NIH non-administrative spending for research, FY 2015–FY 2024.
SOURCE: Committee-created figure based on analysis of the data from the NIH Office of Budget.

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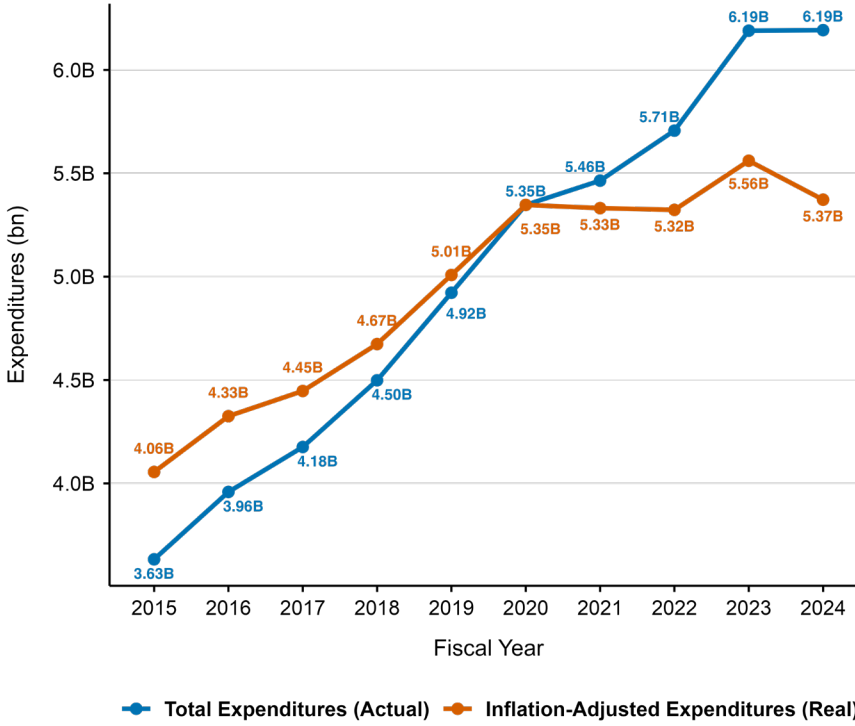

FIGURE 3-3 Pediatric research expenditures, FY 2015–FY 2024.
SOURCE: Committee's analysis of data from the RCDC system.

adolescents under 18 years of age in 2024 accounted for about 22 percent of the U.S. population (Forum on Child and Family Statistics, 2024a,b).

During the 10-year period, the number of pediatric research projects rose from 9,079 to 12,527, a 38 percent increase. The flattening of pediatric research inflation-adjusted funding since FY 2020 led to a decline in real dollars per project from $455,000 in FY 2020 to $429,000 in FY 2024, resulting in a decline in purchasing power for NIH-funded pediatric research projects.

On an annual basis, intramural pediatric research conducted by federal employees and federal researchers within the NIH's laboratories and facilities accounted for between 9 percent and 11 percent annually of pediatric research expenditures across the 10-year period.

Conclusion 3-2: Over the decade analyzed in this report, pediatric research consistently accounted for about 13 percent of non-administrative NIH spending. Growth in actual dollars largely reflected

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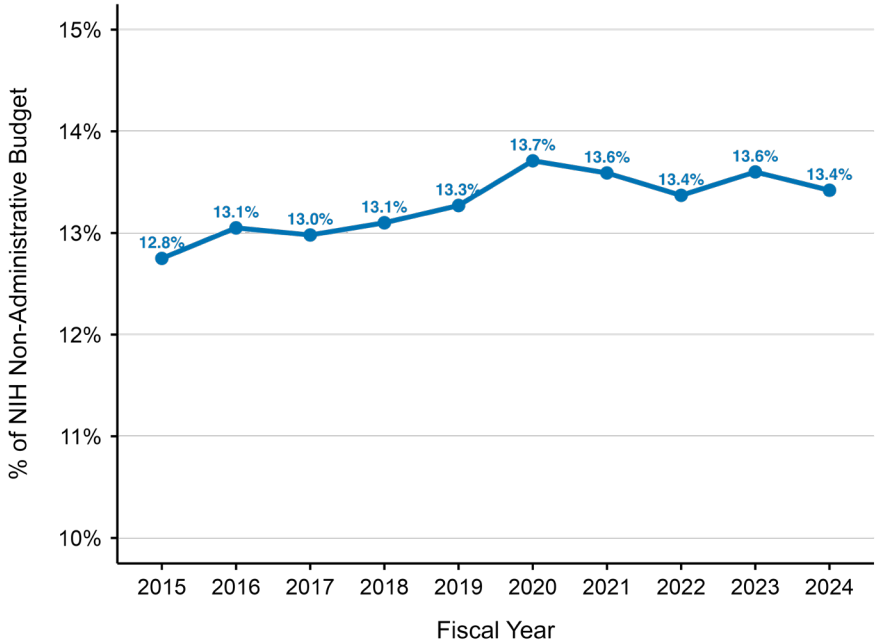

FIGURE 3-4 Pediatric research expenditures as a proportion of NIH, non-administrative expenditures, FY 2015–FY 2024.
SOURCE: Committee's analysis of data from the RCDC system and data from NIH Office of Budget.

agency-wide expansion rather than a strategic re-balancing toward pediatric health. Although National Institutes of Health (NIH) pediatric-research dollars rose 70 percent in nominal terms between FY 2015 and FY 2024 and outpaced overall NIH growth (62 percent), the inflation-adjusted trend flattened after FY 2020. Real dollars per project fell from $455,000 in FY 2020 to $429,000 in FY 2024, signaling a loss of purchasing power that may erode pediatric research project scope.

Grantee Institution and IC

Each grantee institution was assigned to one of four categories: academic, which included universities, medical schools, and research centers affiliated with universities; children's hospitals, both freestanding (e.g., Seattle Children's Hospital) and children's hospitals within hospitals (e.g., Benioff Children's Hospital at the University of California, San Francisco); general or specialty hospitals not primarily pediatric, such as Veterans

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Affairs hospitals and Kaiser Permanente; and, all other organizations (e.g., nonprofit organizations, transfers between federal agencies). Additionally, joint appointments are also common.

The committee recognized the limitations of this assignment. Some investigators are employed at children's hospitals, but their grants are managed by partner institutions, such as degree-granting institutions. For example, the Lucile Packard Children's Hospital is a freestanding children's hospital, but its grants are managed by Stanford University.

The number of institutions that received funding for pediatric research increased from 754 in FY 2015 to 887 in FY 2024. In FY 2024, the distribution of institution type was heavily weighted toward academic institutions (see Figure 3-5). Children's hospitals as the grantee institution accounted for 10 percent of the pediatric projects, and children's hospitals and other organizations each accounted for 10 percent of pediatric research expenditures.

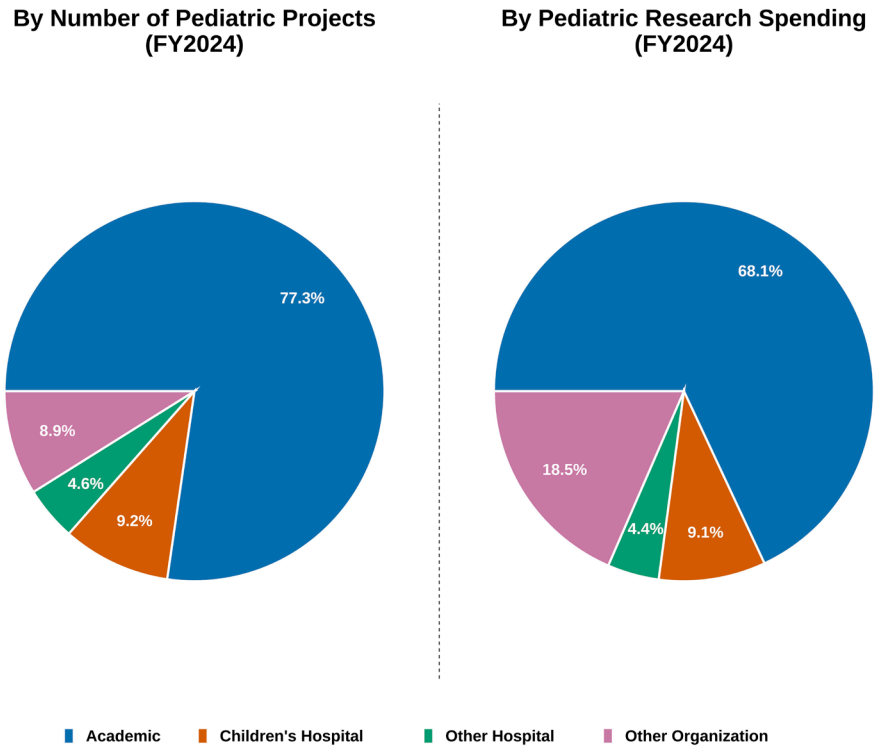

FIGURE 3-5 Proportions of pediatric research projects and expenditures by type of grantee institution, FY 2024.
SOURCE: Committee's analysis of data from the RCDC and RePORTER systems.

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Principal investigator academic departments were categorized into basic science, non-pediatric clinical, and pediatric (see Table E-2 for full list). It is noteworthy that 40 percent of projects had no departmental information. This high rate of missing data is likely because hospitals, including children's hospitals, do not have department information that is reported to NIH, and not all institutions report their departmental affiliations for their faculty and staff. This table refers to contact principal investigators (PIs) and does not include the multiple appointments that some researchers may have. Of the 60 percent of projects with available data, non-pediatric clinical departments accounted for the majority (52–54 percent) of pediatric research (Figure 3-6). Pediatric departments accounted for 12–14 percent of pediatric research. In FY 2024, 19 percent of pediatric research expenditures were from either pediatric departments or children's hospitals. Pediatric research led by principal investigators in basic science departments was 13–15 percent of the total. International PIs were responsible for less than 2 percent of project expenditures.

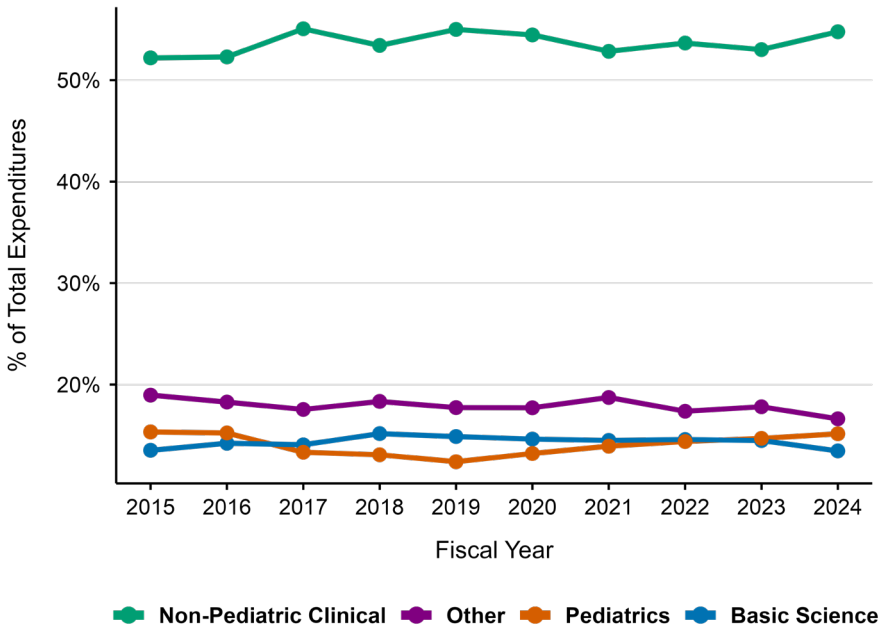

FIGURE 3-6 Academic departmental location of the principal investigator, FY 2015–FY 2024.
SOURCE: Committee's analysis of data from the RCDC and RePORTER systems.

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NICHD Pediatric Research Funding

The *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD) provides leadership within the NIH on children's health research. Although it funds just 17 percent of NIH-funded pediatric research, it provides the largest amount of funding in absolute terms of any IC ($998 million in FY 2024), both for research and for pediatric research training. From FY 2015 to FY 2024, NICHD funding as a proportion of the overall NIH research budget was approximately 4 percent. It is worth noting that this contrasts with what several NIH-funded investigators reported in their responses to the committee's call for perspectives (see Box 3-1).

Conclusion 3-3: The Eunice Kennedy Shriver National Institute for Child Health and Human Development (NICHD) is an essential leader for pediatric research within the National Institutes of Health (NIH) and funds the plurality of all pediatric research at the NIH, although all funding institutes and centers (ICs) support some level of pediatric research. The diversity of ICs that fund pediatric research and training suggests a continued need for NICHD leadership, which houses a substantial amount of pediatric expertise that other ICs can take advantage of.

The proportion of each IC's total research budget spent on pediatric research varied widely, as shown by the black circles corresponding with the secondary y-axis in Figure 3-7. Although every IC funded some pediatric research, there was wide variation in the level of funding. Aggregating the total pediatric research funding across FY 2015–FY 2024, ICs were categorized into five tiers (see Figure 3-7, bars). Tier 1 included a single institute, NICHD, which funded the plurality of research, accounting for 17 percent of total pediatric research expenditures across NIH. Tier 2 ICs included the National Cancer Institute (11 percent), National Heart, Lung, and Blood Institute (10 percent), National Institute of Mental Health (NIMH) (10 percent), and National Institute of Allergy and Infectious Diseases (9 percent). Combined, Tier 1 and Tier 2 ICs accounted for the majority (57 percent) of pediatric research funding. Using each IC's pediatric research expenditures divided by its total research budget (summed across the 10-year period FY 2015–FY 2024), the five ICs with the highest pediatric shares were NICHD (60 percent), NIMH (26 percent), the National Institute of Dental and Craniofacial Research (26 percent), the National Institute on Alcohol Abuse and Alcoholism (25 percent), and the National Institute on Minority Health and Health Disparities (21 percent).

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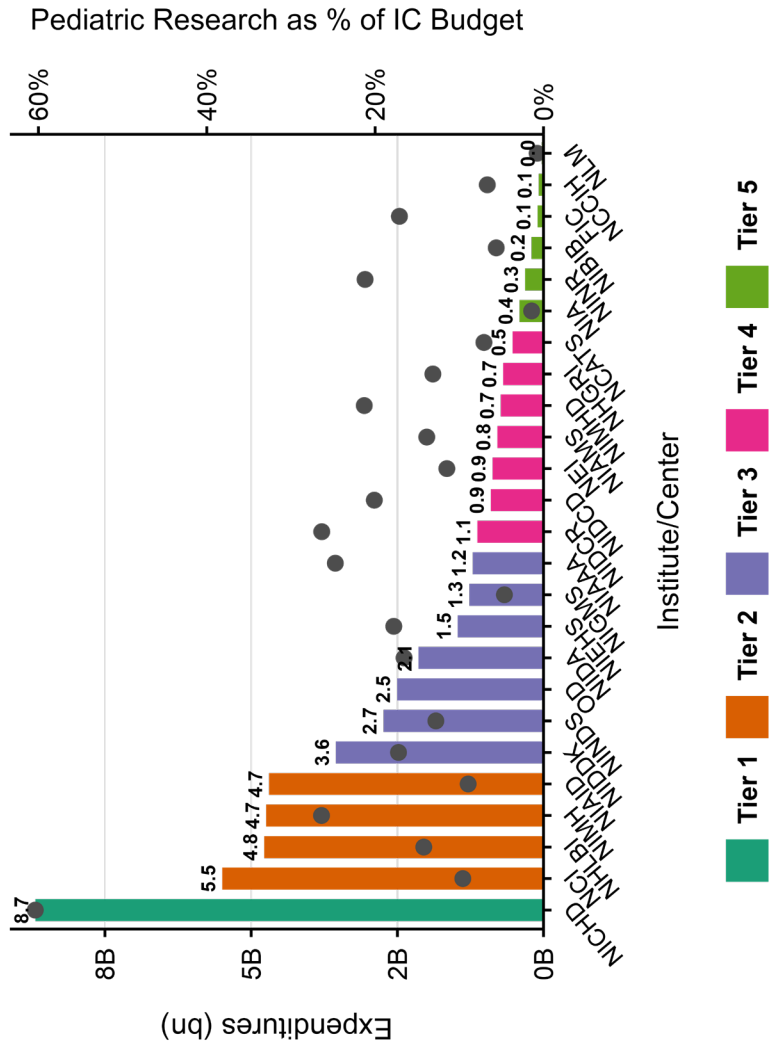

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FIGURE 3-7 Pediatric research expenditures by IC and Office of the Director, FY 2015–FY 2024.
NOTE: The bars represent expenditures by the billion corresponding with the left y-axis. The dots represent the proportion of each IC total research budget that went to pediatric research corresponding with the right y-axis. IC = institutes and centers; NICHD = *Eunice Kennedy Shriver* National Institute of Child Health and Human Development; NCI = National Cancer Institute; NHLBI = National Heart, Lung, and Blood Institute; NIMH = National Institute of Mental Health; NIAID = National Institute of Allergy and Infectious Diseases; NIDDK = National Institute of Diabetes and Digestive and Kidney Diseases; NINDS = National Institute of Neurological Disorders and Stroke; OD = Office of the Director; NIDA = National Institute on Drug Abuse; NIEHS = National Institute of Environmental Health Sciences; NIGMS = National Institute of General Medical Sciences; NIAAA = National Institute on Alcohol Abuse and Alcoholism; NIDCR = National Institute of Dental and Craniofacial Research; NIDCD = National Institute on Deafness and Other Communication Disorders; NEI = National Eye Institute; NIAMS = National Institute of Arthritis and Musculoskeletal and Skin Diseases; NIMHD = National Institute on Minority Health and Health Disparities; NHGRI = National Human Genome Research Institute; NCATS = National Center for Advancing Translational Sciences; NIA = National Institute on Aging; NINR = National Institute of Nursing Research; NIBIB = National Institute of Biomedical Imaging and Bioengineering; FIC = Fogarty International Center; NCCIH = National Center for Complementary and Integrative Health; NLM = National Library of Medicine.
SOURCE: Committee's analysis of data from the RCDC system.

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Spending categories assigned by RCDC are critically important as they enable analysis of patterns of funding for disorders, injuries, and illnesses that affect children and youth. In a study conducted by Rees and colleagues, pediatric research project expenditures obtained from the RCDC were correlated with a variety of population health statistics (Rees et al., 2021). The authors found moderately strong correlations of 0.63 for disability-adjusted life-years, 0.56 for years of potential life lost, and 0.68 for hospital days.

To estimate the level of funding for research that includes a focus on the social influences on health, projects were identified that included at least one of the following RCDC categories of research: social determinants of health (published in 2020); health disparities and racial or ethnic minority health (published in 2023); health disparities research (published in 2023); or, racial and ethnic minority health (published 2023). In FY 2024, 29 percent of all pediatric research included a focus on social influences on health (and were accordingly assigned to one of those spending categories).

Conclusion 3-4: National Institute of Health (NIH) pediatric research funding is broad but concentrated. Every NIH institute and center (IC) supports pediatric projects, yet five of them (the National Institute of Child Health and Human Development; National Cancer Institute; National Heart, Lung, and Blood Institute; National Institute on Mental Health; National Institute of Allergy and Infectious Diseases) account for the majority of expenditures. No single IC exclusively funds any major health condition category, underscoring both the cross-cutting nature of pediatric research funding and the need for strong inter-IC coordination.

CURRENT PEDIATRIC RESEARCH AT THE CLINICAL CENTER

The NIH Clinical Center plays a critical role in enabling pediatric research, particularly for children with rare, complex, or currently untreatable diseases. The Clinical Center hosts clinical trials and natural history studies for rare and complex pediatric diseases and offers highly specialized wraparound care tailored for children involved in research protocols. Pediatric research at the Clinical Center includes gene, stem cell, and cellular therapies with an emphasis on deep phenotyping, genotyping, and novel therapeutic strategies. The Clinical Center hosts an 18-bed inpatient pediatric unit, 14-bed pediatric day hospital, 4-bed pediatric behavioral health unit with 2-bed day hospital, and dedicated outpatient clinics; it also provides access to pediatric subspecialists (e.g., endocrinology, GI, infectious disease) via NIH institutes or partner hospitals and a growing pediatric ICU capability, with 24/7 anesthesia, child life specialists, and pediatric

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BOX 3-1
Insights from Grantees: Perceived Funding Discrepancies between ICs

"Pediatric research is not prioritized across all ICs. NICHD has a limited budget and cannot be the sole or even primary source of funding for pediatric research. Child health at all stages influences adult health. Pediatric populations should be included across all ICs and life course research prioritized."
— Researcher funded by NICHD, NIAID, NCI, NIGMS, and NCATS

"Investigator-initiated R01s for children's health-related research often gets assigned to NICHD, but then the low paylines of the NICHD means even if it was well-scored, it will not get funded."
— Recipient of NIH funding for 30 years from NIDA, NIAAA, and NICHD

"Cross-agency mechanisms are a great way to address needs. Pediatric research by its nature is human focused and would benefit from set aside mechanisms that recognize the need to recruit from established clinical networks, such as groups of children's hospitals working together."
— Pediatric researcher with more than 30 years of NIH funding

"To better support pediatric researchers, NIH could prioritize mechanisms that explicitly include adolescents and emerging adults, encourage cross-institute initiatives focused on life course transitions, and expand funding for long-term cohort studies that can track early exposures through adult outcomes."
– Recipient of NIH funding from NINDS

"There are significant opportunities to strengthen pediatric research by embracing a life course approach. Facilitating cross-Institute collaborations, harmonizing age-related eligibility criteria across RFAs, and supporting cohort extensions into young adulthood would enable a more complete understanding of how early-life exposures shape long-term health."
– Neonatal pediatric researcher and recipient of NIH funding

NOTES: NIAAA = National Institute on Alcohol Abuse and Alcoholism; NIAID = National Institute of Allergy and Infectious Diseases; NIDA = National Institute on Drug Abuse; NCATS = National Center for Advancing Translational Sciences; NIGMS = National Institute of General Medical Sciences; NINDS = National Institute of Neurological Disorders and Stroke; NCI = National Cancer Institute.
SOURCE: These quotes were collected from the committee's call for perspectives from NIH grant applicants and should not necessarily be considered representative. Responses were collected by staff and synthesized by theme and anonymized to the committee (see Appendix C for the questions posted in the call for perspectives).

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hospitalists on staff.[5] As of the first quarter of 2025, there were over 1,500 active research protocols at the Clinical Center; approximately one-third included children under the age of 18, and 80 protocols included children under 2 years old (Hadigan, 2025). From 2018 to 2024, 7.7 percent to 13.5 percent of clinical center patients were pediatric (see Table 3-2). The percentage of children was at an all-time high in FY 2018 and dropped significantly in FY 2020 and FY 2021, due to the pandemic. While the number of pediatric patients has increased since the pandemic, it has not returned to pre-pandemic levels. It is also worth noting that there was a greater decline in pediatric patients than in adult patients during this time.

In 2019 the CC issued a strategic plan, People, Places, Capabilities: The NIH Clinical Center at 65, which identified pediatric health research as an area for expansion (NIH Clinical Center, n.d.). Following discussions with the CC governing board and the NIH Pediatric Research Consortium, a working group was established and charged with identifying pediatric scientific priorities particularly suited to the CC (NIH Clinical Center, 2023). A 2023 report by eight pediatric department chairs which constituted the Children's Working Group assessed the possibility of increasing volumes of children cared for at the CC and of lowering the age of patients from 3 years to 6 months, while maintaining patient safety (CWG, 2023). It supported the expansion in the numbers and ages of children studies at the CC, while recognizing the challenges in providing adequate staffing and maintaining competence for the care of critically ill children.

Conclusion 3-5: The current portfolio of studies at the National Institutes of Health Clinical Center has been limited by the clinical acuity of participants and the availability of pediatric critical and emergency care service lines.

The findings of the working group formed the basis of the Clinical Center Pediatric Research Strategic Plan (PRSP), which was released in 2023 (NIH Clinical Center, 2023). These priorities included natural history studies, gene therapy and precision medicine, pharmacokinetics and pharmacogenomics, and metabolic and nutrition studies (see Box 3-2 for the full list of priorities) (NIH Clinical Center, 2023). In addition to these scientific priorities, the PRSP also emphasized the need for infrastructure enhancements, including a pediatric protocol development core, child-friendly imaging equipment, institutional review board (IRB) and ethical review

[5] In total, the hospital has 200 inpatient beds, 11 operating rooms, 93 day hospital stations, critical care services and research labs, an ambulatory care research facility for outpatient visits, two onsite pharmacies, a blood bank, and a complex array of imaging and diagnostic services (NIH Clinical Center, 2024).

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TABLE 3-2 Clinical Center's Pediatric Capacity from FY 2018 to FY 2024

Summary	FY 2018	FY 2019	FY 2020	FY 2021	FY 2022	FY 2023	FY 2024
#peds patients	3,377	3,209	1,764	1,684	1,821	2,120	2,269
#total CC patients	25,072	24,851	20,937	21,904	18,335	20,086	21,757
%CC patients <18 yr	13.47%	12.86%	8.42%	7.69%	9.93%	10.55%	10.43%

SOURCE: Hadigan (2025).

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enhancements, and workforce development (NIH Clinical Center, 2023). Improvements across these domains would serve to streamline and standardize protocols (facilitating data comparisons), enhance access to novel technologies (facilitating inclusion of pediatric populations), and deepen pediatric expertise within the CC IRB (reducing unnecessary regulatory barriers where appropriate) (NIH Clinical Center, 2023). The PRSP recommended expanding training opportunities for pediatricians at the CC and developing a directory of all pediatric research being conducted at the CC (NIH Clinical Center, 2023). While the number of pediatricians at the CC has increased (about 250 pediatricians in 2022), the strategic plan reiterated the potential for further training opportunities for pediatricians to "increase collaboration and competency" (NIH Clinical Center, 2023, p. 10).

BOX 3-2
Pediatric Scientific Priorities Identified in the 2023 Pediatric Research Strategic Plan

- Expanding the scope of natural history studies to support research on the continuum from diagnosis to treatment throughout the disease trajectory within the same disease
- Building a unified clinical and scientific infrastructure to support studies of gene therapy, chimeric antigen receptor T-cell therapy, and other cell therapies
- Exploring the efficacy of precision medicine interventions in rare, nonmalignant diseases
- Increasing the number of pharmacokinetic and pharmacodynamic studies to improve rational medication use and proper dosing in children
- Performing metabolic phenotyping across a variety of pediatric conditions as well as linking metabolic phenotyping in nutrition studies and assessing the impact of diet on immune phenotypes and metabolism
- Developing a cohort of all pediatric patients at the CC to measure physical and mental health and disease across disorders, along with a deeply phenotyped pediatric cohort of healthy volunteer children to establish a standard set of control samples that can be used across studies
- Increasing support for research studies in pregnant and lactating people

SOURCE: NIH Clinical Center (2023).

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Key Clinical Center Partnerships

The Clinical Center is an essential partner to the NIH Intramural Research Program (IRP). Historically, the IRP has been the NIH's internal research program and has included researchers conducting basic, translational, and clinical research, as well as training programs, in federal laboratories across six research campuses and 23 ICs, including the CC (Aitken, 2021; Schor, 2023). More than 10 percent of NIH's budget supports intramural research at NIH laboratories, and approximately 41 percent (492/1,200) of all NIH intramural researchers are principal investigators at the CC (NIH Clinical Center, 2024; NIH Intramural Research Program, 2022; NIH Office of Budget, 2023).

The CC has beneficial partnerships with institutions outside of the NIH as well. It currently collaborates with the Children's National Hospital and Walter Reed National Military Medical Center and is exploring further partnerships with regional pediatric centers for "safety, transfer, protocol integrity, and joint protocol development to enable pediatric trials" (NIH Clinical Center, 2023, p. 12). Additionally, the Opportunities for Collaborative Research at the Clinical Center program fosters "collaborations between extramural and intramural investigators using the U01 Collaborative Agreement mechanism to take advantage of the unique resources and patient populations available at the Clinical Center" (NIH Office of Clinical Research Education and Collaboration Outreach, n.d.).

The NIH Undiagnosed Diseases Program (UDP) is an exemplar of successful pediatric research initiatives, led by the CC, the National Human Genome Research Institute, and the NIH Office of Rare Disease Research (NHGRI, 2025). The NIH UDP was launched in May 2008 to meet the needs of individuals and families with undiagnosed rare or multisystem diseases. These patients remained undiagnosed despite exhaustive evaluations, which for most pediatric patients in the program lasted more than 5 years (Tifft and Adams, 2014). It is part of the Undiagnosed Disease Network, and 40 percent of the patients seen in the UDP are in the pediatric age group.

Conclusion 3-6: The National Institutes of Health Clinical Center, as the site of most intramural clinical research activities, has facilitated the performance of studies that have not been easily performed in the extramural research community, for example, long-term longitudinal studies and first-in-human gene therapy in rare diseases.

Recommendation 3-3: The National Institutes of Health (NIH) Clinical Center (CC) should continue to prioritize the current level of pediatric research, particularly on topics and using methods that are difficult to

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pursue in extramural environments (e.g., longitudinal studies of rare diseases and early-stage interventional trials) as well as on the long-term outcomes of interventions. Potential mechanisms include:

- **a. Increasing capacity while ensuring safety by overcoming operational constraints at the CC. Younger and more acute patient populations should be supported in early-phase interventional and observational protocols.**
- **b. Continued, expanded, or new partnerships with children's hospitals, academic medical centers, schools, public health departments, and other large health centers across the country to best support pediatric populations.**
- **c. Offering pediatric education and training opportunities at the CC to continue to develop the specialized workforce needed to support this research.**
- **d. Collecting and presenting data in the annual report to the NIH Pediatric Research Consortium (or its equivalent) on the proportion of pediatric research studies, support, and usage by each institute and center at the CC.**

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Prioritization, Coordination, and Integration of Pediatric Research Within and Across NIH Institutes and Centers

National Institutes of Health (NIH) studies are established, prioritized, and funded through a variety of mechanisms (see Chapter 2). Once a research priority has been articulated, the coordination of the research endeavor can occur from within a single institute, center, or office, or multiple institutes and centers (ICs) can collaborate on a research priority. As Chapter 3 makes clear, nearly every IC supports some amount of pediatric health research. For the past 7 years, pediatric health research has gained a higher profile at NIH thanks to the work of the NIH Pediatric Research Consortium, or NPeRC, which coordinates and facilitates pediatric research across NIH.

In this chapter, the committee evaluates efforts by NIH to incorporate pediatric research priorities within and across ICs, along with the challenges to doing so and how these challenges may be mitigated or eliminated. Several examples of successful NIH initiatives are summarized, including ones focused fully on pediatrics and others that included both adult and pediatric participants. Factors are identified that influenced the success of these studies in advancing our understanding of pediatric health and disease.

HOW INSTITUTES AND CENTERS PRIORITIZE PEDIATRIC RESEARCH

Every IC at NIH prepares and regularly updates its strategic plan, which serves as that IC's roadmap for establishing goals and research priorities as well as for identifying challenges and opportunities associated with implementing the plan to achieve the IC's research objectives (RePORT,

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n.d.). The committee reviewed all available IC strategic plans, focusing on how pediatric research was highlighted, articulated, and evaluated in each plan. Recognizing that pediatric research initiatives are funded in each IC, the committee was most interested in whether and how these pediatric research initiatives were featured within the current strategic plans. To further inform this chapter, the committee interviewed representatives from several ICs about their prioritization and planning around pediatric research (see Appendix B for the open session agendas).

Each IC engages in its own strategic planning process, typically producing a new plan every 5 years (FABBS, 2025). These plans are public facing and are meant to share the goals and priorities of the specific IC with other ICs at NIH, the extramural scientific community, Congress, advocacy groups, and the general public. The strategic plans are generally similar to one another in structure, and each relates to the NIH-wide strategic plan. However, there is considerable heterogeneity in the level of detail provided on the process of developing the plan, identifying priority research areas, assessing success, and modifying goals, priorities, and methodologies based on research outcomes.

Though strategic planning across NIH ICs is not standardized, most ICs report using a process that includes an internal analysis of major areas, a coordinated effort to collect input from stakeholders, an interim call for feedback on strategic focus areas, and the finalization of strategic plans by IC leadership. Engaged stakeholders typically include subject experts, patient advocacy groups, and the broader public. Feedback is generally collected through workshops, requests for written feedback, and institute councils. The committee found no information about the specific identities or profiles of these subject experts or stakeholders, so there was no opportunity for the committee to learn more from them about the IC strategic planning process generally and about the incorporation of pediatric research priorities specifically. Community engagement with patient advocacy groups and individuals impacted by specific health issues is highlighted in many strategic plans as a crucial input into the strategic planning process. Finally, some opportunities are developed in response to time-sensitive needs (e.g. HIV, Zika, COVID-19).

The Inclusion of Pediatric Research in IC Strategic Plans

The committee reviewed all the available strategic plans (or the equivalent documents found among the strategic plan reports) of the 25 ICs that fund research and found that 19 of them included the term "pediatrics" or "children" (CSR, 2025; FIC, 2025; NCATS, 2025; NCCIH, 2021; NCI, 2025; NEI, 2021; NHGRI, 2020; NHLBI, 2025; NIA, 2020; NIAAA, 2024; NIAID, 2025; NIAMS, 2025; NIBIB, 2012; NICHD, 2025b; NIDA, 2022;

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NIDCD, 2023; NIDCR, 2025; NIDDK, 2021; NIEHS, 2025; NIGMS, 2021; NIH Clinical Center, n.d.-b; NIMH, 2025; NIMHD, 2021; NINDS, 2021; NINR, n.d.; NLM, 2017).[1] Despite that, every IC supports intramural and/or extramural projects categorized as "pediatric," based on the Research, Condition, and Disease Categorization (RCDC) system (see Figure 3-7). Of note, and as discussed in Chapter 3, the Clinical Center produced its Pediatric Research Strategic Plan in 2023. Within the 19 strategic plans that address children or pediatrics, the details around strategic direction and plans for pediatric health are generally not directly articulated and often are embedded within larger strategic priorities. For instance, numerous ICs highlight research on specific topics "across a lifespan," which includes childhood origins of chronic diseases. Nearly all strategic plans also emphasize efforts that broadly impact child health, such as advancing medical diagnostics and technologies for high-consequence viral pathogens (e.g., the National Institute of Allergy and Infectious Diseases [NIAID]), interventions as early in the lifespan as possible (e.g., the National Institute of Mental Health [NIMH] and the National Institute of Diabetes and Digestive and Kidney Diseases [NIDDK]), and disease prevention (e.g., NIMH and the National Institute on Drug Abuse [NIDA]). In fact, most ICs prioritize diseases that originate in or are specific to children but don't necessarily support research related to lifelong care of those conditions. For example, strategic plans underscore the importance of pediatric diseases, including monogenic traits (the National Human Genome Research Institute [NHGRI]), childhood cancers (the National Cancer Institute [NCI]), epilepsy (the National Institute of Neurological Disorders and Stroke [NINDS]), pediatric liver diseases (NIDDK), and type I diabetes (NIDDK).

The NICHD strategic plan includes focuses on "Improving Child and Adolescent Health and the Transition to Adulthood" and "Advancing Safe and Effective Therapeutics and Devices for Pregnant and Lactating Women, Children, and People with Disabilities" (NICHD, 2025b). Similarly, the National Institute on Alcohol Abuse and Alcoholism (NIAAA) has a specific initiative focused on developing mechanisms to identify children affected by prenatal alcohol exposure (NIAAA, 2024). The National Institute on Deafness and Other Communication Disorders (NIDCD) presents a strategic initiative specifically focused on "early detection, diagnosis, and treatment for newborns, infants, and young children who are deaf or hard-of hearing" (NIDCD, 2023). NCI articulates the importance of its strategic focus

[1] The following ICs' strategic plans or equivalent documentation mentioned "children" or "pediatrics": CC; FIC; NCI (using the Annual Plan & Professional Judgment Budget, which NIH links under strategic plans and visions); NIA; NIAAA; NIAID (six disease-specific strategic plans, four of which mention children); NICHD; NCCIH; NIDCD; NIDCR; NIDDK; NIDA; NIEHS; NEI; NHLBI; NIAMS; NIMH; NIMHD; and NINDS.

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on cancers of childhood, identifies challenges to research in this area, and prioritizes focus areas within childhood cancer research (NCI, 2025). The Clinical Center's strategic plan highlights the importance of advances in gene therapy, cellular engineering, and diagnosis of heritable rare diseases to the ability to care for pediatric patients. It also identifies a short-term goal of performing a "gap analysis between current capabilities and those required to care for younger patients safely" (NIH Clinical Center, n.d.-a).

Measuring the Success of Strategic Plan Goals

Few plans articulate exactly how success in achieving their goals will be measured or monitored. Some plans point out broadly that benchmarks are developed and measured, and in several plans, there is a mention of identifying new ways to track research progress and identify opportunities. For example, the 2025 NICHD strategic plan states that the IC has "defined metrics to measure success and implementation efforts and will track and report on those" (NICHD, 2025b). Most ICs provide yearly updates of their strategic plans, with revisions that result from an assessment of the prior year's activities. For example, NIMH provides a section called "Progress" for each of its four goals, with high-level description of grants funded and discoveries made as a result of funding relevant to the programmatic goal (NIMH, 2025). Specific notices of special interest (NOSIs) and requests for applications (RFAs) within each goal are described as examples of progress. However, there is no public facing avenue to determine what grants were funded under a specific NOSI.[2] For this reason, the committee was unable to determine what NPeRC-driven NOSIs culminated in funded grants. Compared with most other ICs, NIAID provides considerable detail about outcome measurement through a section in its strategic plan called "program evaluation." (NIAID, 2025). The narrative in this section describes NIAID's process of assessment, which includes a needs assessment, feasibility studies, and process and outcome evaluations. The institute's evaluation metrics include bibliometrics (publications, patents) changes to clinical guidelines that lead to changes in clinical practice, evolution of collaborative networks, and program influence and impact.

In addition to measuring success through goal monitoring, NIH has also successfully engaged with the pediatric research community to establish ongoing research priorities, as indicated by a number of examples provided by NIH grant recipients in response to the committee's call for perspectives (see Box 4-1).

[2] Going forward, NIH will no longer use NOSIs. Instead, NIH will use "highlighted topics," which are not replacing NOSIs but serve a similar purpose. More information is available at: https://grants.nih.gov/funding/find-a-fit-for-your-research/highlighted-topics.

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BOX 4-1
Successful Engagement with the Pediatric Research Community

"Technical advisory panels, especially when they have been focused on pediatrics, have been very successful and beneficial. I am often the only panelist talking about pediatric populations in meetings organized by non-pediatric-focused institutes, and the pediatric perspective gets lost when the majority of experts are focused on adults. Study sections with pediatric foci are also important, so that the pediatric work is evaluated by people with expertise in pediatrics."

— Recipient of NIH grant funding from NIMH

"NIH has had some successful engagements with the pediatric research community in recent years, particularly when it has convened multi-disciplinary stakeholders to identify pressing scientific gaps and align research priorities. Notable examples include the collaborative effort across institutes to support research on probiotics and their role in neonatal health as well as the cross-institute group brought together on necrotizing enterocolitis and nutrition. These discussions brought together neonatologists, microbiologists, immunologists, and clinical trialists to inform future directions, recognizing both the promise and risks of therapeutics in this vulnerable population."

— Neonatal pediatric researcher and recipient of NIH grant funding

SOURCE: These quotes were collected from the committee's call for perspectives from NIH grant applicants; responses were collected by staff and synthesized by theme and anonymized to the committee (see Appendix C for the questions posted in the call for perspectives).

A combination of both transparent, measurable benchmarks and increased opportunities to collaboratively establish research priorities is critical for improving the success of NIH's pediatric research portfolio. There remain opportunities in this process for more granularity around outcome measurement regarding pediatric research and, ultimately, the impact of that research on pediatric health.

Conclusion 4-1: Although each National Institutes of Health institute and center (IC) allocates a portion of its budget to pediatric research, a substantial proportion of ICs do not mention children, child health, a life course approach, or pediatrics in their strategic plans. Among ICs that do address child health in their strategic plans, there is limited

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standardization of how ICs prioritize and measure success around pediatric research in the strategic planning process, both within and across ICs. As an example of the life course benefits of pediatric research, investments in research to study and address the childhood antecedents of chronic disease could improve the health of older adults in the long term.

Recommendation 4-1: All directors of National Institutes of Health institutes and centers that fund research should explicitly incorporate pediatric health and potential downstream life course impacts into their strategic plans, as childhood chronic conditions, exposures, and experiences may have disproportionate health impacts into adulthood. Include metrics such as those focused on investigators (e.g., funding rates and career advancement), scientific discovery (i.e., publications and innovations), impact on pediatric health (e.g., morbidity, mortality), and downstream life course impacts (e.g., premature cardiovascular disease) to measure success in meeting their goals.

The Impact of Executive Branch and Congressional Mandates

Executive branch and congressional mandates impact the strategic focus and funding of specific research areas by NIH ICs. Sometimes a mandate is the result of Congress passing a law (e.g., the 21st Century Cures Act) or inserting specific language in an annual appropriations bill (e.g., a directive for NIH to fund certain types of research, establish programs, or meet specific goals such as advancing childhood cancer or autism research). It is the responsibility of the NIH director, through the Office of the Director (OD), to interpret the mandate and assign responsibility to specific ICs. When the language is broad, it can be coordinated across multiple ICs by developing inter-NIH initiatives. When language is topic-specific (e.g., pediatric cancer), the responsibility is assigned to one or a few relevant ICs. Congress authorizes and can appropriate funding directly for an initiative to an IC. For example, congressional support for the Undiagnosed Disease Network was given to NINDS.[3] Ultimately, other funds were needed, including from the OD, so this, de facto, is a direction for an IC to implement a program.

The IC impacted by executive and congressional mandates operationalizes the request through a variety of mechanisms. Sometimes, the mandate is formally added to the strategic planning process. The IC then sets aside funding specifically designated for that mandate and may create new funding lines. Often, new requests for RFAs and NOSIs are developed to

[3] H.R. 2617, Public Law 117-328 – 117th Congress (2021–2022); Consolidated Appropriations Act, 2013.

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engage extramural researchers, and new research programs, clinical trial networks, or training grants are developed around the mandated topic. Some congressional mandates also result in the development of new data repositories or biobanks to support the research topic (e.g., the All of Us research program).

ICs often convene workshops, advisory councils, and stakeholder meetings to solicit input from the scientific community and the public to ensure that the proposed implementation is responsive to the needs of the public. ICs must track their mandate-related activities and report to Congress—through annual NIH reporting and/or ad hoc updates—the relevant accomplishments, as requested by congressional committees (e.g. Children's Health Act [2000][4], Best Pharmaceuticals for Children Act [2002] for safer therapies in children, 42 US Code 282 to support pediatric device development) (NICHD, n.d.). In June 2024, for example, NIH submitted a report to Congress detailing its pediatric research activities in fiscal years 2021 and 2022 (NIH, 2024d). Another example is Environmental Influences on Child Health Outcomes (ECHO), which as of June 2024 consolidated data from over 107,000 participants, including over 64,000 children from many studies cohort studies that were harmonized. It also released these data in de-identified fashion and expanded access for use in research studies. It has generated over 1,700 publications and led to the development of interventional trial networks including support of Institutional Development Award (IDeA) states. It has a robust outreach and policy arm publicizing its achievements to the media and public. This was driven by support and funding that drove a strategic planning and assessment operationalized by an program office in the OD that led an infrastructure that combined all ICs activities studying childhood environmental exposures (ECHO, 2021, 2024, n.d.-b; NIH, 2025a). Congressional mandates are thus one way that pediatric research and downstream impacts of chronic disease at NIH might be specifically enhanced.

THE NIH PEDIATRIC RESEARCH CONSORTIUM

In 2018, representatives from each of the NIH ICs created a collaboration known as the NIH Pediatric Research Consortium (NPeRC), with the goal "to improve child health research collaboration and coordination" across its 27 ICs and offices." (NICHD, 2025a). NICHD is the lead IC for the consortium (NICHD, 2025a). NPeRC was intended to harmonize pediatric research activities across ICs, identify gaps within the NIH pediatric research portfolio, and take advantage of existing resources, infrastructure,

[4] H.R. 4365, Public Law 106-310 – 106th Congress (1999-2000); Children's Health Act, 2000.

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and expertise (NICHD, 2025a). NPeRC does not receive any funding or resources, with engagement from representatives from each of the ICs on a volunteer basis. It does not directly support research projects or initiatives (NICHD, 2025a), nor does it have funding for any administrative support or other infrastructure supports. Although there are discrete examples where NPeRC was successful, it has been limited in its reach and effectiveness due to a lack of dedicated resources, formalized leadership structure, and the agency to enforce requirements for inclusion of children. As of October 2025, the NPeRC website listed 37 individuals representing 25 ICs, two research programs (i.e., All of Us and ECHO) and five offices (i.e., Office of Strategic Coordination, Office of Disease Prevention, Office of AIDS Prevention, Office of Behavioral and Social Sciences Research, Office of Research on Women's Health). NPeRC members meet several times a year to evaluate individual ICs' pediatric research portfolios, meet with American Academy of Pediatrics committee liaisons, develop workshops, evaluate research programs (including the NIH Clinical Center Pediatric Research Strategic Plan), discuss scientific opportunities and potential new areas of collaboration, and promote research training for the next generation of pediatrician–scientists and surgeon–scientists (see meeting agenda items in Appendix F). Since its inception, NPeRC has strived to (1) identify and address research gaps, (2) enable a nimble response to emerging threats to pediatric health, (3) promote greater efficiency and reach of ongoing initiatives through data harmonization, (4) spearhead novel partnerships, and (5) provide consultation to ambitious inter-IC initiatives. The progress made has the potential for much larger scale success with adequate funding and infrastructure, as there is a need for dedicated staff, a communications platform, and an infrastructure to support collaborative pediatric-focused projects.

Addressing Research Gaps

NPeRC designated the transition from pediatric to adult health care, especially for youth with special health care needs, as a scientific priority area containing substantial knowledge gaps (Calabrese et al., 2022). The NPeRC Health Care Transition Subgroup conducted an analysis of NIH's portfolio of research grants and scientific publications from 2007 to 2020 in order to "identify topics, research gaps, and opportunities in various chronic health conditions that affect child health and well-being for which transition research could synergize and expand scholarship (Calabrese et al., 2022)." NPeRC developed a definition of health care transition that served as the framework for the coding of NIH-supported research (Calabrese et al., 2022). NIH ICs were then solicited to catalog NIH-funded grants related to transition. The ICs nominated 88 NIH-funded grants, and

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60 were deemed to meet the inclusion criteria. Of these 60 grants, 20 were identified as gold-standard NIH-supported projects (Calabrese et al., 2022). Further searches, with adjudication by 11 experts, resulted in a final health care transition analysis database of 166 NIH-funded grants across 14 ICs. This included five ICs not previously identified as supporting transition research: NINDS, NIDCD, the National Institute on Minority Health and Health Disparities, NIAID, and the National Center for Complementary and Integrative Health (Calabrese et al., 2022).

The portfolio analysis culminated in a 2-day workshop in 2020 entitled Lost in Transition: Navigating Pediatric to Adult Healthcare, organized by NPeRC, NICHD, the Office of Behavioral and Social Sciences Research, and other NIH ICs (including NCI, the National Heart, Lung, and Blood Institute (NHLBI), NIDDK, the National Institute of Nursing Research, and NIMH). The workshop engaged experts from various disciplines along with patients, families, and caregivers, to examine high-priority research topics related to services and supports that facilitate the transition from pediatric to adult care. The workshop helped identify themes, gaps, and opportunities to supplement ongoing NIH disease-specific work and promote collaboration with public and private organizations. After the workshop, NPeRC efforts to support research gaps in this area continued with a subsequent NOSI—initially supported by eight NIH ICs—entitled Navigating Pediatric to Adult Health Care: Lost in Transition (NOT-HD-21-027). The NOSI encouraged applications in "high-priority research areas related to pediatric health care transition for youth with chronic physical/medical conditions or intellectual or developmental disabilities with the goal of improving care quality and patient and family outcomes" during and after health care transition (NIH, 2021).

Responding to Emerging Threats in Pediatric Health

In March 2020, immediately after the start of the COVID-19 pandemic, NPeRC quickly established a working group with representation from 18 ICs, led by NICHD and NIDA, to facilitate an immediate response to the looming global health crisis (NICHD, n.d.-a). The Trans-NIH COVID Workgroup on Pregnant and Lactating Women and Children focus included examining the risk of infection and severity of disease (particularly in the presence of comorbid conditions) as well as investigating factors underlying mild illness, community transmission, and protective factors. Additional areas of emphasis included severe disease (e.g., multisystem inflammatory syndrome in children), return to school or daycare, and the social, behavioral, economic, and environmental impacts of exposure (NICHD, n.d.-a). The workgroup coordinated funding strategies and other activities across

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NIH related to COVID-19 in pregnant and lactating people and in children (Bianchi, 2025).

In 2022, to facilitate additional and long-term research on the physical, mental, and behavioral health impacts of COVID–19 on children, NIH released two NOSIs: Emerging and Existing Issues of Coronavirus Disease 2019 (COVID-19) Research Related to the Health and Well-Being of Women, Children and Individuals with Physical and/or Intellectual Disabilities (NOT-HD-22-002); and Administrative Supplements for Research of Emerging and Existing Issues of COVID-19 Related to the Health and Well-Being of Women, Children and Individuals with Physical and/or Intellectual Disabilities (NOT-HD-22-003) (NIH, 2022a, 2022b).

The working group facilitated supplemental funding for projects in fiscal year (FY) 2020 and FY 2021 amounting to more than $5 million total. These projects, funded by various ICs and the Office of the Director, included:

1. Augmenting a large cohort study including more than 10,000 children to examine pandemic-related perturbations in developmental trajectories of brain functioning, cognition, substance use, academics, social functioning, and physical and mental health (Jernigan, 2021; NIMH, 2022).
2. "A study assessing the impact of a universal intervention on the mental health of adolescent Black children during COVID-19 and identifying longitudinal multi-level risk and protective factors that predicted adolescent mental health during the pandemic" (Mendelson, 2021).
3. "Testing a coping intervention in preadolescent urban youth designed to prevent the onset of anxiety, depression, and posttraumatic stress symptoms in children facing chronic stress" (Wadsworth, 2020).
4. "A study to examine COVID-related changes in substance use and the relationships between substance use and psychosocial and other variables that may be affected by the pandemic among indigenous adolescents who live on or near reservations" (Stanley, 2021).

The NPeRC COVID-19 working group contributed to broader NIH initiatives facilitating the inclusion of children in the Rapid Acceleration of Diagnostics (RADx) programs and the Researching COVID to Enhance Recovery (RECOVER) effort (Horwitz et al., 2023; NIH, 2025c; RECOVER Initiative, n.d.). The RADx–RadicalSM program includes the Predicting Viral-Associated Inflammatory Disease Severity in Children with Laboratory Diagnostics and Artificial Intelligence (PreVAIL kIds) study, which seeks to identify innovative diagnostic and analytic methods for examining

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factors that shape the diverse clinical manifestations of SARS-CoV-2 infection in children (Goff and Pearson, 2025).

Participation in the COVID-19 research efforts can be mutually beneficial for both the child volunteers and the NIH researchers. From the participant perspective:

> *My children continue to participate in a long COVID research study. The study coordinators have been caring and share information on what they know so far. Participating in research has also improved my children's understanding of health care and increased their interest in science. They like the idea that they can help people by volunteering for research.*
>
> *— California parent*

Data Harmonization

When integrated across datasets, biomedical and psychosocial common data elements, measures, and terminology can enhance collective understanding of a specific research topic and improve the efficiency of ongoing NIH initiatives. Led by NPeRC, the NIH COVID workgroup coordinated "data and terminology harmonization efforts related to its populations of interest, including pregnant and lactating people, infants, and children." NICHD convened representatives from the NIH Office of the Director, NIDA, NIAID, and its intramural and extramural programs, to develop recommendations for a standardized set of biomedical and psychosocial common data elements and measures. Three working groups (Biomedical, Psychosocial, and Biospecimens) were formed with representation from more than eight intramural and extramural NIH-funded pregnancy cohort studies, including: ECHO, NIH Helping to End Addiction Long Term (HEAL) and the Early Intervention to Promote Cardiovascular Health of Mothers and Children (ENRICH) program. Participating NIH ICs included 13 institutes, three centers (Fogarty International Center, National Center for Advancing Translational Science, and National Center for Complementary and Integrative Health), and the Office of Research on Women's Health.

Pediatric Medical Device Development through a Public–Private Partnership

Despite significant legislative, regulatory, and scientific efforts in recent years, innovative pediatric medical devices continue to reach the market at slower pace than adult medical devices (Reamer, 2022). Often this is due to the lack of incentives for the industry to research and develop pediatric medical devices. Between 2011 and 2023, the FDA Center for Devices and

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Radiological Health premarket approvals and humanitarian device exemptions that were labeled "only for adults" increased at about twice the rate of devices labeled for "both adult and pediatric use" and about 21 times the rate of devices labeled solely for pediatric use (FNIH, n.d.). This is a longstanding and significant health problem.

In response, NPeRC formed a Pediatric Medical Devices subgroup and sponsored a System of Hospitals for Innovation in Pediatrics Medical Devices webinar in partnership with the American Academy of Pediatrics, the Food and Drug Administration (FDA), the Critical Path Institute, and others (NICHD, 2021). In 2022, a request for information was published (NOT-EB-22-008) "to seek public input on research gaps, needs, best practices, innovative study designs and measurement, resources and data resources, and opportunities to inform a public–private partnership to enhance pediatric medical device development (NIBIB, 2022)."

The nonprofit Foundation for the NIH (FNIH) launched the design phase of the public-private partnership in September 2023 with key federal partners including the FDA and the Biomedical Advanced Research and Development Authority (FNIH, 2023). Both agencies have provided scientific and regulatory insight and expertise as well as funding. Additional funding and expertise have been provided by the private sector, including industry and nonprofit organizations working through FNIH. Within NIH, NICHD and the National Institute of Biomedical Imaging and Bioengineering (NIBIB) are leading this effort, with a number of other ICs engaged through NPeRC. Project goals include optimizing the translation of "technological advancements in medical device design, evaluation, and approval for pediatric populations"; minimizing risk; and streamlining processes to translate research on pediatric medical devices from the bench to the bedside (FNIH, 2023). The public–private partnership is designed to facilitate the successful development and commercialization of pediatric medical devices.

These projects highlight NPeRC's potential to facilitate and coordinate pediatric research across NIH and beyond. As this report demonstrates, NIH can do even more to advance pediatric health in the United States, and the committee believes that NPeRC is an excellent model for getting more value from NIH-funded pediatric research. But to do that, NPeRC will need to be provided the financial resources and human expertise to enable it to mature and grow.

Conclusion 4-2: The National Institutes of Health Pediatric Research Consortium (NPeRC) has insufficient infrastructure, financial support, and representation of designated expertise in bioethics, patient advocacy, and communications. There is considerable potential for expansion and formalization of NPeRC's influence with adequate committed

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resources and leverage to support successful research across Institutes and Centers.

Recommendation 4-2: The National Institutes of Health (NIH) director should:

- **a. Elevate the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to the Office of the Director, with membership including senior leaders within each institute or center (IC) with pediatric health and life course expertise, who systematically review all relevant components of strategic plans, including progress and impact made in the past year. Each senior leader should then report back to their IC and to the NIH director.**
- **b. Provide appropriate infrastructure funding and adequate resources (including personnel) to enable NPeRC (or its equivalent) to accomplish its goals and for its members to participate in its work, which includes (1) identifying and addressing research gaps, (2) enabling a nimble response to emerging threats to pediatric health, (3) promoting greater efficiency and reach of ongoing initiatives through data harmonization, (4) spearheading novel partnerships, (5) providing consultation to ambitious inter-IC initiatives, and (6) ensuring representation and visibility at national scientific meetings and societies.**
- **c. Have NPeRC's membership (or its equivalent) include expertise in pediatric bioethics. NPeRC should also partner with patient advocacy or community representatives and develop a communications plan to disseminate pediatric research findings to both the scientific community and the general public. NPeRC (or its equivalent) should be prepared to coordinate the NIH-wide response to any emerging pediatric research needs.**

INTER-IC INITIATIVES: SUCCESSES AND CHALLENGES

Inter-IC pediatric health research initiatives are those supported by two or more ICs and/or the Common Fund within the Office of the Director (Common Fund, 2024). They are not necessarily supported or coordinated by NPeRC. These initiatives may be congressionally mandated or initiated by an IC that engages other ICs for support. Typically, one IC will manage the inter-IC project programmatically, and, in the case of Common Fund projects, one or more IC are chosen to take the lead. While inter-IC initiatives begun by an IC are driven by the strategic plans of participating ICs, inter-IC studies initiated from the Common Fund reflect priorities across NIH and emphasize transformative, catalytic, goal-driven, synergistic, and

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novel areas established by the NIH director with programmatic input. Another category of NIH-wide programs includes new initiatives from the NIH director. Historically, these have often fallen under the Common Fund, but not always.

There is no NIH mandate or requirement per se to address or consider pediatric health through the vehicle of inter-IC initiatives. In the context of preclinical science, targeting pediatric-relevant health issues has been driven ad hoc by the topic studied. In these cases, scientific advancement has been largely driven by the solicited and/or submitted projects, especially because the relevant goals can be highly technical. For clinical and translational studies, a limited number of inter-IC initiatives have been successful in addressing pediatric health problems. Those that have succeeded tend to have several things in common (Table 4-1).[5] First, they ask scientific questions that are best answered by cross-cutting, collaborative efforts among the appropriate ICs. Second, those scientific questions raise issues critically important to pediatric populations; moreover, they cannot be answered without inclusion of pediatric populations. Third, they include processes and procedures to establish pediatric-focused scientific infrastructure. Fourth, children are recruited and seen as integral to the study from the beginning. And, fifth, successful inter-IC initiatives receive sustainable funding. In contrast, other projects were limited by the inhibitors delineated in Table 4-1. NPeRC did not have sufficient resources or mandate to overcome these barriers.

Conclusion 4-3: Facilitators of pediatric research initiatives include collaborative efforts from the start, a scientific premise and question that warrant pediatric representation, infrastructures for the conduct of pediatric research and pediatric institutions, inclusion of pediatric patients from the beginning of enrollment, and stable funding.

Recommendation 4-3: Initiatives within and across the National Institutes of Health (NIH) institutes and centers should coordinate with the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to include children and/or the downstream life course impact of childhood chronic disease, exposures, and experiences in studies in a strategic and deliberate way. NPeRC (or its equivalent) should support the enforcement of existing policies requiring that studies not exclude children or

[5] The characteristics that either facilitate or inhibit successful pediatric research within inter-IC initiatives have been synthesized from presentations and discussions with several NIH leaders held at a public webinar on March 13, 2025. See https://www.nationalacademies.org/event/44604_03-2025_open-session-part-1-strategies-to-enhance-pediatric-health-research-funded-by-nih for more information.

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TABLE 4-1 Facilitators and Inhibitors of Successful Inter-IC Pediatric Research Initiatives

Facilitators	Inhibitors
Concept design and implementation that begins collaboratively across ICs	Concept design and implementation that begins within a single IC (including with intentions to bring pediatrics stakeholders onto the team at later timepoints)
The scientific question requires pediatric populations to answer	The scientific questions are not conceived in a way that includes children
Early implementation includes infrastructure for pediatric research practices, including protection of vulnerable subjects and procedures for surrogates such as parents/guardians	The infrastructure for pediatric research practices, including protection of vulnerable subjects and procedures for surrogates like parents/guardians, is delayed until after program launch
Funding is stable and sustainable, including via congressional appropriations and/or dedicated support from NIH and/or IC directors	Funding is variable and uncertain

SOURCE: Synthesized from comments made by inter-IC initiative leadership at the committee's March 13 webinar; see https://www.nationalacademies.org/event/44604_03-2025_open-session-part-1-strategies-to-enhance-pediatric-health-research-funded-by-nih.

childhood downstream impacts on the life course without clear and adequate justification.

To close this chapter, the committee presents four inter-IC projects that highlight how these facilitators and inhibitors impact the successful advancement of pediatric health research.

Adolescent Brain Cognitive Development

One of the most successful cross-IC initiatives is the Adolescent Brain Cognitive Development (ABCD) Study. Established in 2015, ABCD was founded with an initial goal of fostering research on poly-substance use. Collaborators within the NIAAA, NIDA, and NCI came together with a shared understanding that to improve the risk factors and mitigate the consequences of substance use, it is necessary to first examine how childhood experiences influence childhood development (Dowling, 2025). ABCD uses multiple methods and data sources (e.g., neuro-imaging, neurocognitive testing, biospecimens, geocoded data), evaluates multiple exposures (e.g., environment, culture, prior physical and family health) and examines multiple outcomes (e.g., physical health, mental health, substance use) to achieve its goals (Hawes et al., 2025; NIMH, 2022).

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ABCD has expanded beyond its original focus on substance use to meet the scientific interests of several additional institutes. NICHD participates to advance understanding of healthy brain development and traumatic brain injury. NIMH focuses on identifying factors that influence the course and severity of mental disorders that commonly emerge during adolescence and are associated with substance use. NINDS contributes by measuring how frequently sports injuries (e.g., concussions) take place during adolescence and to determine their impact on brain development. More recently, the NIH Office of Research on Women's Health joined to examine sex and gender influences on adolescent brain development, and NHLBI was added to address issues including adolescent cardiovascular and hematologic health.

Today, ABCD is the largest long-term study of cognitive development and child health in the United States. The consortium infrastructure consists of a coordinating center; a data analysis, informatics, and resource center; and 21 research sites across the country. The project has included nearly 12,000 children who joined the study at the ages of 9–10 years (NIMH, 2022). Researchers track participants' biological and behavioral development through adolescence and into young adulthood. Specifically, ABCD uses its multiple methods and technologies to determine how childhood experiences (e.g., use of videogames, sleep habits, tobacco use, substance use, and sexual activity) interact with each other to affect a child's social, behavioral, academic, health, and other outcomes.

During a public information-gathering session, the director of the ABCD project, Dr. Gaya Dowling, reported that NPeRC played an important role in its success:

> *During the pandemic, we created a subgroup of NPeRC that was focused on [COVID], and that gave us the opportunity to pool resources to be able to fund supplements and grants that the institutes may not have been able to do on their own. And it also gave us the ability to leverage money we were getting from the institutes to get additional money from the Office of the Director to support some of these. So, I think . . . fewer of those supplements would have gotten funded without [NPeRC].*[6]

The impacts of ABCD have been substantial. The program has published approximately 1,300 manuscripts and has been cited in 83 policy papers and clinical guidelines worldwide (ABCD, n.d.). Its continued success is in large part because it has always used an open-data sharing model, acted on the numerous requests for applications to use the data, and received sustainable funding from NIDA (RePORT, 2025). ABCD

[6] The webinar recordings can be accessed at https://www.nationalacademies.org/event/44616_03-2025_open-sessions-part-2-strategies-to-enhance-pediatric-health-research-funded-by-nih.

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has inspired and laid the groundwork for other inter-IC projects, including the Healthy Brain and Child Development (HBCD) study (focused on early brain and child development from the prenatal period through age 9) (HBCD, n.d.). Together, ABCD and HBCD are elucidating numerous facets of cognitive, social, emotional, and physical development during childhood that would be difficult to detect from single-IC sponsored projects (Dowling et al., 2024).

Environmental Influences on Child Health Outcomes

Whereas ABCD started with a scientific question, other successful cross-IC endeavors started with a policy mandate. The Environmental Influences on Child Health Outcomes program, for example, was mandated by the CURES Act of 2016[7] with a mission to better understand the impacts of early environmental exposures on later child health and development (DPCPSI, 2024). Organizationally, ECHO is located within the Office of the Director. Like ABCD, ECHO addresses scientific questions of interest to multiple ICs, including pre-, peri-, and postnatal outcomes. Current priorities include lung diseases, obesity, neurodevelopment, and "positive health" behaviors (Gillman, 2025). Unlike ABCD, ECHO goes beyond an observational cohort study to also support a clinical trial network for child intervention research (ECHO, n.d.-a). Requests for applications to use ECHO's cohort or trial network are sponsored by multiple ICs (NIH, 2024c).

To date, the ECHO cohort has included more than 107,000 participants, including people traditionally underrepresented in research such as members of rural and low-income populations (NIH, 2025a). The project's impacts include approximately 2,000 publications, endorsements and adoptions of practice recommendations from state governments, and the development of influential public health guidelines (ECHO, n.d.-c). Despite these achievements, ECHO leaders highlighted several current challenges that the program faces: difficulties with research infrastructure, including capacity building and data-harmonization across sites; changes in funding appropriations; and shifting federal priorities (Gillman, 2025). These challenges and uncertainties may impact the program's continued success.

All of Us

The All of Us research program is an example of an NIH initiative, that, to date, has fallen short of its potential to address child health. Funded in 2016, the program proposed a platform of precision medicine to "accelerate health research and medical breakthroughs, enabling individualized

[7] 21st Century Cures Act of 2016, Public Law 114-255, 114th Congress, 1st Sess.

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prevention, treatment, and care for all of us" (NIH, 2025b). Specifically, the program argued that there is no one-size-fits-all approach to health care, and a large dataset representing diverse populations would better facilitate tailored, successful treatment plans.

At first glance, All of Us seems to have had the necessary elements of success. It was funded through federal appropriations from Congress as part of the 21st Century Cures Act[8] as well as through an additional base appropriation to the NIH Office of the Director, which is intended to offset fluctuations in 21st Century Cures funding levels. Despite historical bipartisan support, however, funding for both Cures and All of Us base appropriations have diminished. Notably, the budget was cut by 34 percent in FY 2024 and by 71 percent in FY 2025. The result has been a substantial decrease in enrollment and data collection and, as noted on the All of Us home page, delays in the "ability to build a robust pediatric cohort" (NIH, 2024a), making it difficult for researchers to recruit enough children to validly answer the study questions (NIH, 2024b).

These delays compounded existing challenges to including children in the All of Us study. Despite early recognition that precision medicine should include people across the life course, program leaders did not create pediatric-focused procedures when they launched the program's infrastructure. It was not until the fifth year of funding (2021) that All of Us called for a pediatric director and not until the seventh year (2023) that the first pediatric participant was enrolled (Van Driest, 2025). Even after these delays in including children, some researchers suggested starting with older children and then gradually enrolling younger children over time. This strategy, known as age de-escalation, is a form of protection that is widely and sometimes reflexively used, even when it may not be warranted (Harbin et al., 2023). The All of Us study posed relatively low risk for children of all ages, so there was no valid reason to wait to enroll younger children in the study.

When the committee asked All of Us leaders why the program struggled to include pediatric patients, they answered by referring to the commonly used approach to clinical research: that adult-aged participants go first (Van Driest, 2025). This practice can be important when considering vulnerable subjects such as minors for research that entails some risk. In the All of Us study, however, this protectionist approach did not make sense because the study did not pose significant biomedical risks to any of the participants. All of Us compiled data from people in a range of ways, including through collecting biological samples and using wearable devices to collect data about local environments. Community consultation could have provided avenues

[8] 21st Century Cures Act of 2016, Public Law 114-255, 114th Congress, 1st Sess.

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for assessing whether data privacy was a significant concern for members of the public and, if so, how to mitigate these concerns.

Indeed, community-based and other stakeholder-type partnerships, be they between researchers and families or clinicians and advocates, play key roles in pediatric health care delivery and policy (Chung et al., 2022). Had the All of Us investigators worked with community representatives, they may have found that most parents support their family's or child's research participation (Kim et al., 2020; McCarthy et al., 2025). Moreover, they feel disappointed when clinicians and researchers make assumptions about their willingness or "vulnerability"; rather, they suggest that research participation is a critical way to amplify their voice and experience (McCarthy et al., 2025). While this community engagement is neither unique to pediatrics or to All of Us, it is key when considering how best to support research that will inform pediatric patient- and family-outcomes.

Had there been a permanent structure within the NIH focused on the benefits of research for children and adolescents and with the authority and resources to support pediatric enrollment, the repeated delays that resulted in near exclusion of children from the All of Us study might not have occurred. A strengthened NPeRC with such a mandate might serve as that structure.

The Patient-Reported Outcomes Measurement Information System Initiative

The Patient-Reported Outcomes Measurement Information System (PROMIS) was designed to create and validate self-report symptoms and quality of life measures that could be used to document the health status of people experiencing a variety of health conditions (Garcia et al., 2007). In 2001, Congress requested that the Institute of Medicine prepare a report on how to optimize collaborative science in the 21st century (Rettig, 2004). The 2003 report included recommendations for planning across NIH to identify ideas for multi-year research initiatives that could address cross-cutting issues (IOM, 2003). The ensuing, initial request for applications did not specifically name pediatrics research as being eligible, but a strong application submitted by a pediatric-focused team was selected for funding along with several adult-focused submissions (Wang et al., 2018). The second PROMIS RFA that followed was informed by the strong evidence base for pediatrics that resulted from the first RFA.

Since then, PROMIS has been a formative force behind 300 patient-reported outcome (PRO) measures as well as proxy measures developed, validated, and made readily and freely available for use in research and clinical care (Health Measures, 2025). PROMIS measures were the focus of 4,464 peer-reviewed publications between 2005 and 2025, of which 757

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were pediatric in focus. The PRO measures are available in many languages and are used globally, enabling multiple types of population and health comparisons to be made, which adds immeasurably to our knowledge about diseases.

As with other initiatives spanning NIH ICs, the facilitators of PROMIS are its collaborative design, attention to infrastructure, and sustainable funding (see Table 4-1). The early inclusion of pediatric-relevant scientific questions occurred by happenstance because a single team of pediatric-focused investigators responded to requests for applications. Importantly, the inclusion of pediatric science and populations of children added considerably to the PROMIS program's overall impact.

Pediatric- and adolescent/young adult–focused PROMIS measures have been incorporated into questionnaires for use in cooperative group clinical trials to document the impact of treatment for cancer and have been widely used across a variety of other adult and pediatric diseases to measure the impact of disease-specific treatments (Bense et al., 2023; Dahir et al., 2024; Dovern et al., 2023; Gershon et al., 2010; Hermans et al., 2023; Kliewer et al., 2023; Maurer et al., 2021; Reeve et al., 2017; Walters et al., 2025). These outcomes of the PROMIS adult and pediatric measures in research have increased their usefulness in clinical care in identifying health improvements in patients and in discovering profiles of the patients most in need of supportive care for symptom relief (Cheng et al., 2023; Schuchard et al., 2022; Thissen et al., 2016; Troost et al., 2019; Zigler et al., 2024). Further, the existence of adult- and pediatric-specific PROMIS measures for the same symptoms occurring in the same disease in pediatric and adult patients has advanced our understanding of how a disease evolves across parts of the life course.

PROMIS has been successful in large part because it included considerations of the elements listed in Table 4-1. Its concept and design included collaboration across ICs; the scientific question and implementation recognized the necessity of including pediatric- and lifespan-inclusive populations; early implementation included consideration of pediatric research practices and procedures; and funding includes plans for sustainability.

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5

Supporting Pediatric Research and Addressing Challenges

Pediatric research projects offer unique opportunities to reverse the current pediatric health crisis and support a healthy generation of children, the future health of those children as adults, and, ultimately, a prosperous United States. Despite this, pediatric research is often undervalued compared with adult research (Speer et al., 2023). This undervaluation can be seen in the relatively limited funding allocated to pediatric research, the lower prestige of working in pediatric fields, and the greater difficulty in translating findings into long-term benefits for children (Stoll and Taegtmeyer, 2018; Subasri et al., 2023). Furthermore, the full impact of pediatric research is not always realized because studies often focus on short-term outcomes rather than impacts on the life course (Long and Fink, 2021). This is shortsighted because the health decisions made in childhood have profound consequences on an individual's overall health trajectory, and understanding these long-term impacts is crucial to improving public health.

In addition to being undervalued, pediatric research faces a number of other challenges due both to the complexities of studying children and their environment and also the infrastructure required to support such research. Numerous barriers impede the successful execution and advancement of pediatric research. These barriers are of several types: ethical and legal concerns related to the inclusion of pediatric patients in clinical trials; intrinsic complexities related to the interplay between complex environmental influences and child development; methodological challenges and issues largely faced by researchers; structural and procedural challenges faced by research funders; and financial limitations. There are multiple strategies to overcome these barriers and thus to promote the conduct of important

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pediatric research. In this chapter, both the barriers to and facilitators of pediatric research are discussed, along with recommendations to overcome those barriers and strengthen facilitators.

ETHICAL AND LEGAL IMPEDIMENTS

Despite decades of recognition of the problem, there remains an urgent need to correct the longstanding default approach of "adults first" in research funded by the National Institutes of Health (NIH) (Gitterman et al., 2018). Children's vulnerability to harm has been used as a reason not to conduct pediatric research or to wait for data from relevant studies with adults. While there are some types of research where delayed inclusion of children may be appropriate (e.g., research with new molecular entities that have not yet been tested in humans), evidence suggests that the common practice of using a default of exclusion has harmed the interests of all children, because pediatric clinicians often lack the necessary information about how best to treat their patients. As discussed in Chapter 2, Congress and NIH have taken steps towards the increased inclusion of children in research, most notably releasing an Inclusion of Children policy in 1998 and the Inclusion Across the Lifespan policy in 2019 (NIH, 1998, 2017). Noting that 10–20 percent of research at the time inappropriately excluded children and that most drugs on the market do not have any labeling for use in children, the U.S. Senate concluded that "considerations of justice" call for the greater inclusion of children in biomedical research (NIH, 1998). As the Inclusion of Children Policy explains, "It is the policy of NIH that children (i.e., individuals under the age of 21) must be included in all human subjects research, conducted or supported by the NIH, unless there are scientific and ethical reasons not to include them" (NIH, 1998). The Inclusion Across the Lifespan policy expands on this by clarifying that NIH research should be inclusive for people of all ages, including children and older adults. Yet as discussed in Chapter 3, the promise of these policies to improve the inclusion of children in research has not materialized. Instead, the amount of pediatric research funding at NIH has stalled in real terms since 2020.

One of the reasons that these policies have not dramatically improved the inclusion of children in research may be that they have several exceptions and limited enforcement mechanisms. Investigators submitting grants to NIH are required to include a description of their plans for including individuals across the lifespan and presenting a justification for any exclusion (NIH, 2025). However, investigators deciding whether to include children can use any of six justifications for exclusion provided by these policies (see Table 5-1). The committee was unable to find any data on the frequency of using these justifications for excluding children from research.

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TABLE 5-1 Potential Justifications and Examples of Appropriate Exclusion of Children under Existing NIH Policy

Potential justifications for the exclusion of children from research	Examples
Disease or condition does not occur in children or is not relevant to them	Alzheimer's disease research
Studying children will be redundant of current or previous research	A drug under study has already been studied and approved for use in children
A separate, age-specific study in the excluded age group is warranted and preferable (but consider taking account of age in study design)	A clinical trial designed to promote self-monitoring of blood glucose levels in adolescents with Type 1 diabetes proposes to include only adolescents.
The study will collect or analyze data on pre-enrolled study participants; data for children are not part of this dataset	A study that began prior to implementation of the NIH Policy and Guidelines on the Inclusion of Children proposes follow-up to examine long-term outcomes of individuals with the condition. The original study excluded children, and similar data are not available from a cohort that includes children.
Laws or regulations prohibit including children	Regulations for protection of human subjects allow consenting adults to accept a higher level of risk than are permitted for children.
Study poses an unacceptable risk to the excluded group, such that their participation would be seen as unethical by institutional review boards, peer reviewers, or NIH staff.	Children are excluded from a Phase I study for a treatment that includes significant risk, including death. Evidence suggests the potential benefits to children do not outweigh the risks.

SOURCE: Adapted from NIH (2017).

While the exceptions to justify exclusion are important and reasonable as written, they may be interpreted broadly in a way that unduly restricts the inclusion of children (see Figure 5-1). One example provided is that researchers could exclude children for conditions seen in older adults, such as Alzheimer's disease. However, pediatric research may yield insights into the precursors of Alzheimer's disease (Assuras et al., 2025). Perhaps the broadest potential justification for exclusion is when "[a] separate, age-specific study in children is warranted and preferable" (NIH, 1998). Existing NIH guidance gives clear and specific reasons why this statement may be acceptable, such as if a condition is relatively rare in children and is already being studied in a nationwide pediatric disease research network. However, it is unclear whether this exception is actually used in the relatively narrow way in which it was intended. Additionally, studies that collect even limited information in children may offer insights that can inform future research.

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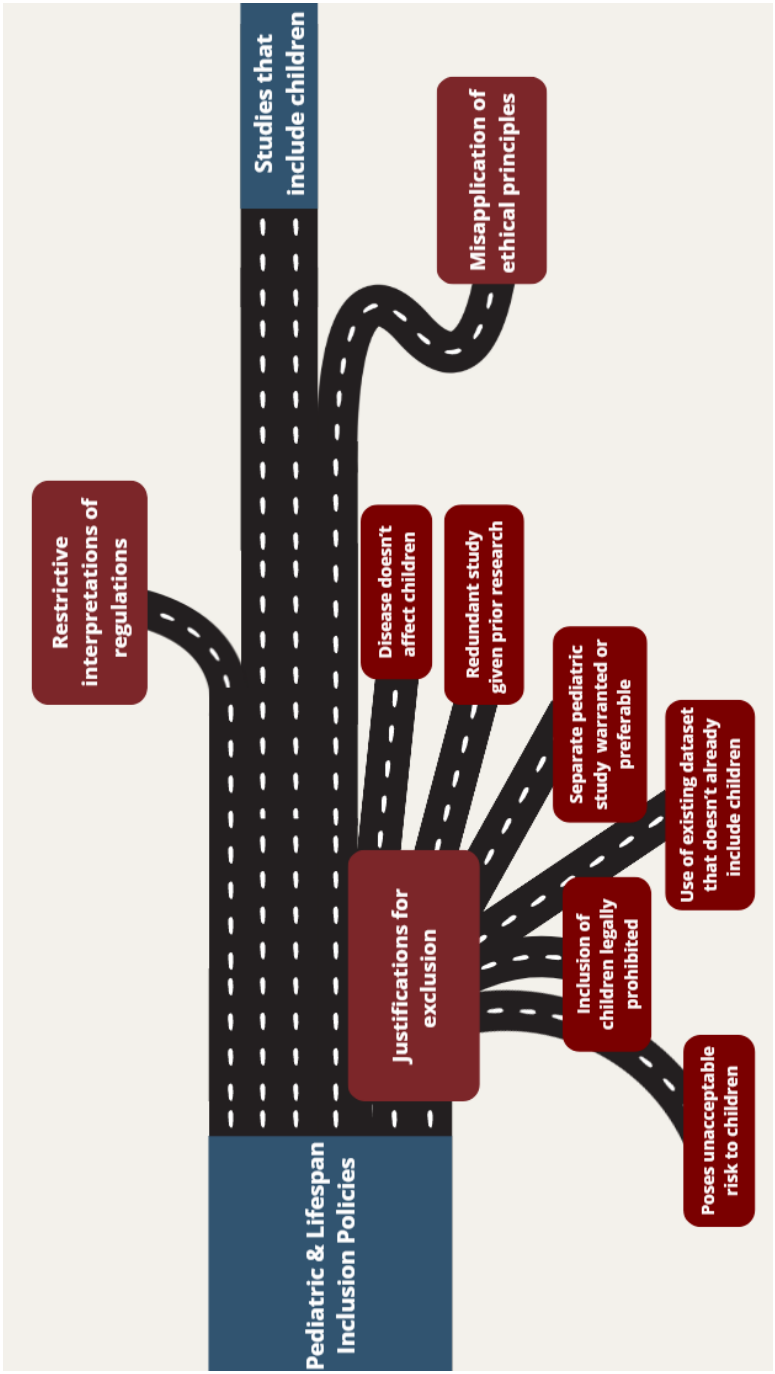

FIGURE 5-1 Off-ramps preventing the inclusion of children in research.

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As will be discussed further below, another justification for the exclusion of children is when pediatric research is not legally or ethically permissible, and the policies provide examples focused on concerns about research risks. While concerns about research risk are sometimes an important reason to exclude children from certain types of research, such as first-in-human research or risky interventions that have limited potential benefit for children, the risk/benefit categories in federal regulations illustrate that even high-risk research can ethically and legally include children in some cases. For instance, risky research may be approvable when it includes children with serious, life-threatening conditions and the research has a high potential of offering benefits not attainable outside of the research.[1] There are some examples of early and responsible inclusion of children in research, several of which were discussed in Chapter 4. As another example, in pediatric rheumatology, researchers recognized the limited therapeutic options available for children. When new biological therapies transformed treatment for adults, researchers collaborated with the Food and Drug Administration to include children early in the research cycle with novel study designs in which each child had the opportunity to respond to the drug being studied and participate in an extension arm if they were benefiting from therapy (Ruperto, 2016).

The inclusion of children in research can therefore be consistent with existing federal regulations in ways that may not always be recognized by applicants or reviewers. Accordingly, further guidance may be needed to ensure that these exceptions are more narrowly understood so that children are more routinely included in research when it is scientifically and ethically appropriate to do so, in a manner consistent with existing policy (see Figure 5-2). It is important that scientific review groups tasked with identifying whether a particular justification is acceptable or unacceptable take care to prevent overuse of these exceptions.

Conclusion 5-1: The National Institutes of Health (NIH) policies requiring the inclusion of children in research related to questions relevant across the lifespan lack adequate mechanisms for enforcement and implementation to ensure their consistency with existing policy. Comprehensive data to monitor the implementation of these policies are not systematically collected.

Beyond their function as exceptions to policies promoting the inclusion of children in research, research ethics principles and regulations may also lead to the undue exclusion of children from research, particularly when they are misapplied or misunderstood (Shah, 2021). U.S. federal regulations

[1] 45 CFR § 46.407 (2025).

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FIGURE 5-2 Proposed approach to the inclusion of children in research.

governing research recognize that it can be important to include children in research and are designed to strike a balance between the inclusion of children and their exposure to risk. These regulations apply to research that involves biomedical interventions, psychological or behavioral research, or social science methods (Shah, 2021).

Notably, the U.S. federal regulations governing research with children have not been updated since their passage in 1983 and have some limitations that could benefit from reform (HHS, 1983; Ross, 2006; Shah and Wendler, 2010). Given the challenges associated with regulatory reform (Berkman et al., 2017), however, this committee focused on other legal and ethical promoters for pediatric research and impediments to pediatric research that may be easier to address. For instance, some regulatory provisions are ambiguous and can be interpreted in different ways by review committees, which can lead to the unnecessary restriction of pediatric research.

The interpretation of requirements that can generate barriers for pediatric research is discussed in greater detail below. First, however, existing

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regulatory requirements for research with children are presented. After discussing barriers created by ambiguity or restrictive interpretations of existing regulations, the section closes with a discussion of ethical safeguards that are commonly used but not required by regulation, highlighting how these can sometimes be impediments to pediatric research that is ethically appropriate.

Regulatory and Legal Requirements that Apply to Research with Children

Regulatory requirements in pediatric research are more stringent than those for adult trials. Children are considered a vulnerable population, which means additional safety, ethical, and legal safeguards must be in place when conducting pediatric research. Children are generally defined by law as individuals who are not old enough to have the capacity to make their own decisions (see Box 5-1). As discussed in that box, U.S. federal regulations defer to state and local law on who is considered a child.

The U.S. federal regulations permit institutional review boards (IRBs) to approve three categories of pediatric research involving low risks or where the benefits outweigh the risks:[2]

- Pediatric research with minimal risk,
- Pediatric research in which the prospect of direct benefit outweighs risk, and
- Pediatric research involving a minor increase over minimal risk with no prospect of direct benefit.[3]

Ambiguity In the Interpretation of Requirements that Impede Pediatric Research

One key source of ambiguity in the regulatory requirements for research with children is the issue of how to identify the risk level. While the regulations define minimal risk as "the risks of everyday life or of routine examinations or tests," they do not define other levels of risk or benefit.[4] As discussed above, to protect pediatric participants federal regulations impose different requirements based on the risks posed by the research and whether the risks are justified by the prospect of direct benefit. Moreover, difficulties

[2] Research involving greater than minimal risk and no prospect of direct benefit to individual subjects but likely to yield generalizable knowledge about the subject's disorder or condition, 45 CFR § 46.406 (2025).

[3] U.S. Federal Regulations: 45 C.F.R. 46, Subpart D.

[4] Definitions for purposes of this policy, 45 CFR § 46.102i (2025).

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in recruiting pediatric participants mean that many studies must enroll at multiple sites in order to obtain enough participants to answer a scientific question definitively. One study found wide variability in IRB judgments about the risks of common biomedical research procedures (Shah, 2004). A more recent study similarly found variability in identifying what counts as minimal risk in certain research procedures involving pregnant people, along with a reluctance to approve studies that would count as minimal risk and be approvable under existing regulations (White et al., 2021). The available data suggest that IRBs tend to use restrictive and variable interpretations of risk and benefit as well as rigid approaches to assent and consent that may not reflect the developmental capacities of pediatric populations (Kodish et al., 2004). As a result, studies may be delayed, redesigned, or prevented altogether. Although waiting to include children may be appropriate for some early studies that pose high risk and uncertain benefit, particularly if they are testing interventions that are not urgently needed for children, these barriers can cause delay when research poses minimal risk and has strong potential for benefit.

Confusion about what is required for research decision making can also lead to delays in including children. Under the U.S. regulations governing research, children should give their agreement to participate ("assent") if they are capable, and parents or guardians are also asked to consent. For anything that does not fit in these categories, the Secretary of the Department of Health and Human Services can convene a special panel that can review and approve the research.[5] For research with older children, the question of when a child can provide their own consent is difficult to determine (Leibson and Koren, 2015). As noted in Box 5-1, the U.S. federal regulations differ from state and local laws on who can consent and allow independent adolescent consent in a range of circumstances, but most state and local laws do not directly address consent to research. This leaves IRBs to decide when the laws that allow adolescents to consent for themselves based on their status, condition, or maturity can be applied to informed consent for research. The existing patchwork of laws and the room left for interpretation can make it challenging to determine when adolescents can participate in research without parental permission, particularly for studies that are conducted in multiple states (Gilbert et al., 2015). This also leads to skewed data from the studies that are conducted, making it challenging to include the populations at greatest risk or in greatest need of intervention and to know whether interventions work for them (Liu et al., 2017).

The vagueness of assent requirements can lead to challenges and delay for pediatric research. A lack of clarity about assent may lead some

[5] Research not otherwise approvable which presents an opportunity to understand, prevent, or alleviate a serious problem affecting the health or welfare of children, 45 CFR § 46.407 (2025).

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BOX 5-1
U.S. Federal Regulations for Pediatric Research Definition of a Child

The U.S. federal regulations governing research involving children as participants define children as "persons who have not attained the legal age for consent to treatments or procedures involved in the research, under the applicable law of the jurisdiction in which the research will be conducted" (45 CFR 46.402[a]). Laws that determine who can consent for themselves to treatments or procedures vary across U.S. states and even cities in some cases (Fogler and Lerman, 2017). In general, jurisdictions define an age of majority when people are considered able to consent for themselves; for most states in the United States, this age is 18 (Guttmacher Institute, n.d.). States typically also have a patchwork of exceptions allowing minors to consent for themselves before they reach the age of majority, for several reasons, including as the importance of ensuring prompt treatment for communicable diseases to promote public health, the need to remove barriers to treating children who are not living with their parents, and the recognition that children do not suddenly become able to make their own decisions on the day they turn 18. The three main categories of exceptions allowing minors to consent are based on: condition (e.g., sexually transmitted infection); status (e.g., marriage); and designation (e.g., a determination by a judge or clinician that a minor is mature enough to make their own decisions or emancipation from one's parents through legal proceedings) (Guttmacher Institute, n.d.). In the United States, states generally do not have laws that specifically address consent for research, and the IRB must determine when laws addressing consent for treatment can be applied to research (HHS, n.d.).

investigators to believe that research with children is more complicated than adult research and may lead to a sense that it is easier to start with research in adults in all cases. Confusion about assent could also cause researchers to obtain assent when it is not necessary. One prominent group of scholars has argued that assent should not be obtained in two cases: (1) the child lacks capacity to assent for various reasons; or (2) "the research offers benefits to the child that are substantially greater than those available outside the research and so joining the study is unequivocally in the child's best interests" (Joffe et al., 2023, p. 464). These authors do note that children should still be given developmentally appropriate information about the research and engaged by research teams, even if they are not capable of assent and it does not make sense to ask them for their agreement to participate (Joffe et al., 2023).

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State and local laws also impose mandatory reporting requirements for certain categories of health professionals that can introduce barriers for pediatric research (Kingsley et al., 2024). These requirements are typically justified by the importance of protecting individuals or the public's health. They range from requiring people designated as mandatory reporters to share information with departments of public health about certain communicable diseases to requiring that information about child abuse or neglect be shared with child protective services (Kingsley et al., 2024). For example, pediatric researchers in Pennsylvania may encounter challenges to conducting research related to adverse childhood experiences (ACEs) due to the state's mandated reporting laws, which apply to all licensed health professionals. This legal obligation can introduce complex ethical and logistical considerations, as inquiring about participants' or their parents' ACE exposure may generate requirements to report to state authorities, thereby complicating both study design and participant engagement.

Additionally, some requirements imposed by agencies such as the Food and Drug Administration (FDA) for adult research may apply to pediatric research and thus raise special challenges. For example, the FDA recommends pregnancy testing for phase I and II clinical trials in any person who has reached menarche, which applies to children who have gone through puberty (FDA, 2025; Thomas and Reeves, 2023). This requirement can be complicated to implement in pediatric research if parents are uncomfortable with their young child undergoing pregnancy testing. The length of follow-up required can also be challenging to determine for pediatric research, depending on the outcome of interest. For instance, studies testing cooling for birth asphyxia and other interventions thought to have neuroprotective effects may require endpoints that are longer in pediatrics in order to capture whether there are developmental delays. Although adult studies sometimes address this issue by developing surrogate endpoints, surrogate endpoints are not available for many pediatric outcomes of interest and may not be appropriate (Green et al., 2019).

Conclusion 5-2: More flexibility in approaches to assent could give investigators the ability to tailor the provision of information and level of engagement to a particular child's abilities.

This variability can be a significant problem for multi-site studies, as it can extend the time needed to conduct research. While single IRBs are now mandated under the Common Rule, there is local variation in familiarity with procedures and relevant variation in state laws, as discussed further below, which may make it hard to determine how much local review is still needed. In some cases, local IRBs may be hesitant to rely on a single IRB and insist on conducting their own review, and IRBs may have differing

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interpretations about the risk level of a given study, which can lead to requirements that vary based on the site (Hu et al., 2022). More work is needed to implement the process of single IRB review for pediatric research, along with additional guidance for local IRBs on ethical issues that are widely misunderstood or are addressed in variable ways. Additionally, the process for approving studies that do not fit into existing risk/benefit categories is slow and cumbersome and could benefit from greater transparency and efforts to increase efficiency (Jennings et al., 2025).

There has been significant controversy concerning whether children whose biological samples were included in biobanks should re-consent to continued use of their samples when they turn 18. While some researchers conducting studies with stored samples opt to re-consent participants who reach the age of majority, others de-identify the sample instead by destroying the information connecting the sample to a particular person. De-identification is one way to preserve privacy but makes it difficult or impossible to share findings that may emerge at a later date, some of which may be clinically actionable or even life saving. In contrast to the standard approaches to stored sample research, some have argued that reconsenting or de-identification may not always be ethically necessary. Parents make many decisions for their children that can influence their lives even as adults, ranging from the schools they attend to the medicine they take, and people who reach the age of majority are not routinely asked to revisit all of these decisions. Given the burdens associated with reconsent, it is unclear that the practice of reconsenting should always be required (Berkman et al., 2018).

Other Ethical Safeguards that Can Stymie Pediatric Research

Another source of ambiguity is deciding when ethical concerns about pediatric participants should be addressed by incorporating additional safeguards, such as extended safety monitoring. These additional requirements may sometimes be justified to protect participants and future pediatric patients but can increase the cost and complexity of pediatric studies. In the worst-case scenario, additional requirements for pediatric research can lead to efforts and funding being directed toward adult-focused research if risks and other ethical complexities are perceived as easier to surmount.

A further contributor to delayed pediatric inclusion may be the common practice of enrolling older children before younger children (i.e., "age de-escalation") without questioning when it makes sense to do so (Harbin et al., 2023). As discussed in Chapter 4, the practice of age de-escalation has been raised for studies even when there is little difference in the risks of research for children across different ages, such as in the All of Us study. Furthermore, the forgone benefit of not receiving access to research may

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be higher for younger children in some cases if they have greater disease severity and few treatment options. Age de-escalation may be an important safeguard in some cases but should not be applied across the board.

One final ethical issue that may stymie pediatric research is the perceived need to limit payment to avoid undue inducement or coercion (Gelinas et al., 2018). Undue inducement is the concern that people deciding on research participation will be so blinded by the money that they disregard the risks or act in ways that go against their values. Coercion is often mistakenly raised as a concern that offering payment will force someone to participate. Offering payment expands the options available to people and does not involve a threat to take away what they are entitled to or a way to force them to do something against their will (Wertheimer and Miller, 2008).

In fact, payments in pediatric research can be valuable to: (1) reimburse caregivers for the costs they incur to participate in research, (2) compensate caregivers and children for their time, or (3) show gratitude to and incentivize caregivers and participants to take on the burdens of research participation (see Figure 5-3). First, reimbursement is important because caregivers may have to pay for childcare, transportation, and parking in order to attend research visits. Without consideration of the costs of research participation, families are essentially being asked to subsidize the research.

Second, paying caregivers and participants more than is needed to cover their costs is not coercive, but it may serve to compensate them for the time spent on research and make it possible for them to choose to contribute to research rather than doing something else. Finally, incentives can be a way to ensure that families feel their contributions are valued and keep coming back to research visits over time (Bierer et al., 2021; Shah, 2021).

While high payments may lead to worries about undue inducement or concerns that parents might pressure their children to participate in research studies, there are other ways to address this concern rather than reducing compensation (Diekema, 2018; Gelinas et al., 2018; Wendler et al., 2002). Research should be reviewed and determined to be ethically acceptable, consent and assent (if appropriate) should be obtained, and dissent should be respected unless the research offers potential therapeutic benefits that the child could not otherwise receive. With those safeguards in place, it may be less important to worry about paying families too much for participation and more important to worry about not paying them enough (Largent, 2017).

As these examples illustrate, the reflexive application of ethical dogmas—as opposed to the thoughtful application of ethical principles— can lead to delayed inclusion of children in research. Exclusion is one response to vulnerability. Another potential response is responsible inclusion. Research can be designed to account for and respect the vulnerabilities and needs

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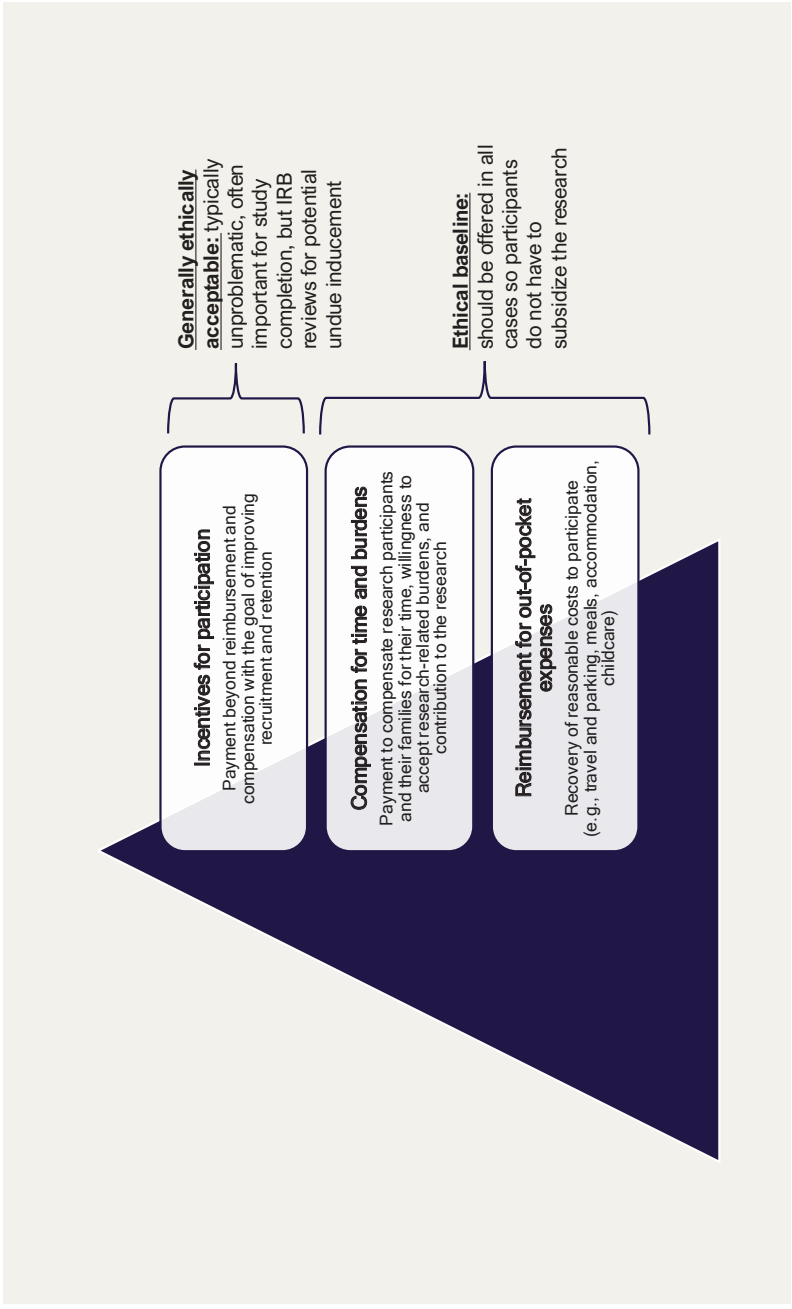

FIGURE 5-3 Payment for participation in research.
SOURCE: Content from Gelinas et al. (2018).

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of different children, implementing safeguards for children based on their vulnerabilities. As has been recognized with other groups previously considered "vulnerable," such as pregnant people, it is time to recognize the importance of protecting children through research, not from it (Lyerly et al., 2021). Ultimately, shifting the default toward inclusion to realize the promise of existing federal policies that promote the inclusion of children, along with ensuring that ethical safeguards are applied appropriately, will help advance the health and well-being of all children.

SCIENTIFIC CHALLENGES TO CONDUCTING PEDIATRIC RESEARCH

Pediatric researchers face numerous scientific challenges due to the inherent physiological and behavioral differences between children and adults, the complexity of diseases that affect children, and the limitations in current research models and scientific approaches in fully unraveling the intricacies of pediatric diseases (EPA, 2025). These barriers hinder the ability to generate accurate and meaningful data and to refine pediatric disease models that are used to understand the mechanisms of diseases affecting children and to test potential therapies. Without the best data and models, it is much more difficult to inform the care and treatment of pediatric populations.

Biological Differences Between Children and Adults

There are significant differences in developmental variability, growth, and maturation between children and adults. Developmental variability impacts infants and young children as well as adolescents, who have developing organ systems, immune responses, and metabolic rates that are different from adults. Infants, children, and adolescents also have developing psychosocial characteristics, including cognitive skill, patterns of social engagement, and perceptual and motor abilities. In the case of disease and injury, the risk, progression, and treatment responses may vary across these developmental stages, making it difficult and sometimes inappropriate to translate adult disease models to pediatric populations. It is also important to take into account growth and maturation in basic science projects and prevention/intervention programming, as pediatric models often need to account for age-specific factors such as hormonal changes, brain development, and skeletal maturation, which may not be well represented in animal models or adult human models. For example, humans accrue approximately 25 percent of peak bone mass during the 2-year period encompassing peak height velocity (Wojtys, 2020). Threats to bone health during that period

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may have very different life-long implications than the same threats a few years later when skeletal maturation is complete.

Limited Availability of Pediatric Participants and Samples

The relatively small size of certain pediatric populations, particularly those with rare diseases or specific genetic conditions, can make it difficult to conduct large-scale studies. Without enough participants, the power of studies is limited because small sample sizes make it challenging to generate statistically significant findings and more difficult to identify trends or differences that might be important in understanding pediatric conditions. Furthermore, small trials may have limited generalizability because findings from a small, homogenous group of children may not apply to the broader pediatric population. Collecting tissue samples or clinical data from children presents significant challenges, particularly for very young children and neonates with heightened vulnerability to the potential risks associated with invasive procedures, which must be carefully addressed in the research design. The combination of small participant pools and limited material for analysis means there may be an insufficient amount of data for accurate modeling. To generate sufficient sample sizes and statistically meaningful data, multi-center studies are not only common but often essential in pediatric research studies. A previous National Academies report drew a similar conclusion:

> Because most children today are healthy, children suffering from serious conditions such as cancer or heart disease are relatively few in number compared with the number of adults who have such conditions. For any one research location, the numbers available for a study are usually quite low. For many pediatric studies, the relative scarcity of potential study participants means that it often takes considerable effort and some creativity to enroll and retain sufficient numbers of children who meet the criteria for study participation. It may also mean that studies must extend for quite long periods just to secure enough participants. (IOM, 2004, p 80)

Genetic and Environmental Variability

Health outcomes in children are shaped by a complex interplay among environmental exposures, family factors, and genetics, and these influences vary significantly across populations. There are numerous genetic diseases that manifest early in life. Pediatric models, thus, need to incorporate a wide variety of genetic mutations, which can make the models complex and difficult to study. Environmental factors (e.g., infections, nutrition, toxins) can influence disease development and response to treatment in children, but

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these factors are difficult to incorporate into animal or laboratory models. Pediatric research must take into account a wide range of environmental determinants—air and water pollution, nutritional access, housing quality, neighborhood safety, the availability of health care services, and so on—which often interact with inherited genetic risk factors in ways that are not yet fully understood. These factors do not operate in isolation; rather, they are embedded within broader social and structural contexts that influence children's development and disease risk. Adverse childhood experiences, which have been established to have long-lasting effects on a child's health into adulthood, are linked to "to the social and economic environments in which some families live" (CDC, 2025). Risk factors for ACEs include, but are not limited to, children with few or no friends, families with low incomes, and families with adults who have low levels of education (CDC, 2025). Collecting data from diverse populations is essential to uncovering how different environmental and genetic factors converge to influence the onset, severity, and progression of pediatric diseases. Such insights are critical not only for improving diagnostic accuracy and therapeutic interventions but also for advancing health equity and tailoring prevention strategies to the needs of specific communities.

Complexity and Rarity of Pediatric Diseases

Based on the limited number of individuals affected, nearly all pediatric diseases can be considered as rare diseases. A rare disease is defined by FDA and the National Cancer Institute as affecting fewer than 200,000 individuals per year (FDA, 2024 ; NCI, n.d.). Furthermore, children are also affected by rare and heterogeneous conditions with complex manifestations. Disorders like childhood cancers, genetic syndromes, or rare metabolic disorders may present differently at different developmental stages, making it difficult to develop a one-size-fits-all model. Pediatric diseases often involve multiple organ systems, making it hard to replicate their full complexity in a single model. This can hinder the development of the treatments needed to address systemic effects.

Challenges in Translating Animal Models to Humans

Basic and translational science studies using animal model organisms, such as simple organisms (e.g., zebrafish, fruit fly, and nematode), mice, or large mammals play a crucial role in advancing pediatric research (Ogden et al., 2021; Tain et al., 2025). While differences exist between species in immune systems, metabolism, and developmental trajectories, these models provide indispensable opportunities to study biological mechanisms that cannot be ethically or practically examined in children. Certain

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investigations, such as the long-term follow-up of early-life interventions, intergenerational impacts, or life course studies, are feasible only in animal models, given the lifespan limitations of human studies.

Animal research has been foundational for numerous pediatric breakthroughs, including prenatal–neonatal therapies, gene therapy for monogenic disorders, interventions for metabolic diseases (Farooqi and Xu, 2024; Tain et al., 2025), necrotizing enterocolitis (Kovler et al., 2020), and cystic fibrosis (Ogden et al., 2021). Even studies in complex neurodevelopmental disorders, when designed carefully, allow researchers to establish cause-and-effect relationships by isolating specific genes or factors before translating findings to children, who are influenced by diverse genetic, environmental, and social contexts.

Although complementary studies in human tissues, cells, or organoids can provide additional insights, organoids mainly reflect early embryonic development and cannot fully capture the developmental stages of childhood organs or the long-term effects of interventions. Therefore, animal models remain essential for determining in vivo effects, safety, and the mechanisms of novel therapies before human trials.

To maximize translational relevance, it is recommended that research teams conducting animal studies include pediatricians or pediatric clinician–scientists. Their expertise ensures that preclinical work aligns with child health priorities and can be effectively translated into clinical interventions. This approach mirrors successful maternal studies funded by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), where inclusion of obstetricians was required to ensure relevance.

Maintaining a robust pipeline from animal research to clinical application has been a distinguishing feature of U.S. pediatric research, contributing to life-saving interventions such as surfactant therapy, nitric oxide treatments, fish oil supplementation, and targeted gene therapies. Continued careful integration of preclinical animal research into pediatric studies is essential to sustain innovation, improve child health outcomes, and uphold the United States' preeminent position in translating basic science into clinical breakthroughs.

Difficulty in Assessing Pediatric Treatment Responses

Children and adults metabolize drugs differently due to differences in liver enzymes, kidney function, and many other physiological factors. Therefore, in many cases pharmacokinetic data from adult models cannot be directly applied to pediatric populations (Fernandez et al., 2011). Furthermore, the tools used to measure disease progression or treatment efficacy in adults may not be suitable for use in children. For example, in

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developmental diseases or pediatric cancers, standard biomarkers or imaging techniques may not be as effective for assessing outcomes in children.

Methodologic Barriers to Conducting Pediatric Research

Developing research studies that include human participants requires navigating a number of methodologic considerations, including participant recruitment (as discussed further in the section addressing financial barriers), the diversity of participants, and data collection and measurement. Each of these takes on an additional degree of complexity when developing pediatric health research. These methodologic challenges are more than important considerations for good study design. Left unaddressed, they generate structural barriers to pediatric representation in NIH-funded research that hinder advances in pediatric health.

Psychosocial Factors

Disease can have a significant impact on normal developmental processes. Chronic illness in childhood affects cognitive, emotional, and social development, and these impacts on development can complicate the study of disease progression and treatment (Turkel and Pao, 2007). The psychological burden of disease may affect children's ability to participate in clinical trials or models. Furthermore, pediatric disease models often need to consider long-term quality-of-life outcomes, which can be harder to measure and predict in young patients and requires long-term follow-up research.

Cognitive Development

Children continuously undergo cognitive development, which complicates research assessment and treatment. When measuring pain, for example, young children have difficulty expressing verbally the level of pain they are experiencing. Infants are unable to do so at all, except through crying and nonverbal cues (Thrane et al., 2016). When measuring depression, children may express depressive thoughts in ways that differ from adult expression (Mullen, 2018). Young children are unable to compare themselves with peers who are functioning with different mental health (Levy-Friedman and Kogut, 2024). Therefore, when gathering data from children that can be used to understand, prevent, and treat their illnesses and diseases, it is often necessary to use innovative, complicated, and creative assessment and treatment strategies, which are frequently more burdensome than those used for adults (Jonsson et al., 2017).

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Inclusion of Rural and Underserved Populations in Pediatric Clinical Studies

Ensuring that the results of pediatric research are generalizable across diverse populations is critical for creating applicable, impactful findings. Children from rural areas and those living in poverty face distinct health risks and barriers to accessing health care. These children are often underrepresented in clinical trials, which means that their specific needs and responses to treatment may not be well understood. Expanding access to clinical trials for these populations is essential to ensuring that all children, regardless of their geographic location or socioeconomic status, are included in research and benefit from advances in pediatric research. To increase the participation of rural and underserved children, pediatric researchers need to meet children in their communities, homes, and local clinics. This may require developing or using infrastructure that is local to these underrepresented populations, such as collaborating with regional hospitals and outpatient clinics, community centers, K–12 schools, and religious establishments, establishing satellite locations and, when feasible, using telehealth and medical devices that can collect, store, and transmit health information securely from participants to researchers.

Ensuring that pediatric research is inclusive and generalizable can be particularly challenging. Trials may unintentionally exclude certain groups, such as children with disabilities or those from underserved communities as well as pregnant women, leading to a lack of representativeness in research findings. The geographic concentration of pediatric clinical studies also contributes to greater direct and indirect costs by requiring a greater number of sites to meet enrollment benchmarks. For pediatric health studies to succeed, they must be provided sufficient funds to meet these challenges to pediatric recruitment. Failing to enable broad representation in studies can lead to gaps in pediatric representativeness, which can cause pediatric harm in real-world clinical practice (Sullivan-Bolyai et al., 2007; Winter et al., 2018).

Family Management Considerations and Gendered Roles

Family dynamics play an important role in pediatric research because the involvement of parents or caregivers is crucial in the recruitment and ongoing participation of children in research. In one study, parents reported that the primary motivators for study participation were health benefits for the child and altruism. The most common barriers to participation were the risk to the child and the burdens of participating (Nathe et al., 2023).

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Gendered Roles and Bias

In many households, socialized expectations and norms about gender and caregiving roles can limit parent participation in research. Across the globe, even in countries well known for policies supporting gender equality, mothers engage in more parenting responsibilities than do fathers (Mark et al., 2025). This reflects a broader trend in which women report bearing more of the mental load when it comes to parenting and household tasks more generally. Parenting, general domestic labor performance, and breakdown among heads of a household can be affected not only by socialized gender roles, but by class status, racialized and ethnic identity, and economic and public policies. Overall, women and mothers are more likely than men to quit their jobs and stay at home for a child with a complex medical condition (Werner, 2023); and at specialty outpatient pediatric clinics, 74 percent of mothers attended with their children compared with 44 percent of fathers (Mark et al., 2025). In a systematic review of paternal involvement in pediatric obesity prevention trials, Morgan and colleagues reported participation rates by fathers as low as 6 percent (Morgan et al., 2017). This evidence base is further limited by the lack of research on historically underrepresented groups. Increased involvement from fathers in pediatric research will provide a more complete picture of child health and increase opportunities for family-centered research.

Impact of Caregiving on Participation

Caregiving responsibilities, especially in the context of complex pediatric conditions, can limit a family's ability to participate in studies (MacNeil et al., 2025; Page et al., 2020). These challenges are exacerbated by social or financial difficulties, including the need for transportation, access to technology, language barriers, childcare, and time away from work. Addressing these barriers requires that researchers consider flexible trial designs that accommodate the varying schedules and needs of families and that funding is sufficient to cover the cost of transportation, childcare, and missed work.

Data Acquisition Challenges

Pediatric researchers face several data collection challenges that are not typically problems for health research involving adults. First, many pediatric research studies require longitudinal data collection to be able to observe relevant outcomes across the developmental course. Creating the capacity for such longitudinal data collection and storage is more labor intensive and costly than the typical R01 (5 years of funding) allows (Flores-Toro et

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al., 2023; Oken et al., 2023). Second, data collection in pediatric studies must evolve as participants transition from childhood to adulthood (Oken et al., 2023; Stepien et al., 2021). Third, many research tools have not been validated across all age groups. As a result, existing research initiatives may not adequately translate to pediatric populations (Collins et al., 2022). Fourth, many outcomes in childhood are best understood within the broader context of family and household factors, where parental health, caregiving practices, and family stressors strongly shape child development and well-being (Masarik and Conger, 2017; Osher et al., 2018; Szatmari et al., 2016; Ward and Lee, 2020). Capturing these interdependencies requires approaches that extend beyond the individual child to include parents, caregivers, or siblings as part of the analytic unit. However, pediatric research lacks established standards for dyad- or family-unit–based data collection. The absence of common frameworks limits comparability across studies, constrains longitudinal follow-up as children mature into adulthood, and hampers the development of interventions that target both the child and family system. National data systems such as Medicare and the Veterans Administration are less available for pediatric populations than for adult populations. Establishing robust methods for dyadic and family data collection—such as harmonized questionnaires, parallel outcome measures across participants, and models that account for interdependent data—will be essential for advancing pediatric research and strengthening its translational impact.

These pediatric-research-specific challenges around data collection and infrastructure complicate the incorporation of pediatric research into NIH-wide efforts that use adult-oriented frameworks. Advancing pediatric health outcomes through NIH-wide initiatives will require accommodating the longer data collection times needed and establishing improved standards for data collection that can accommodate pediatric research needs. For example, surrogate endpoints (i.e., biomarkers) in pediatric research for FDA studies could shorten the time required to collect enough data for the FDA to consider approving a drug for use in children (Green et al., 2019). In addition to modifying data collection standards, the development of standardized measurement protocols for pediatric research will be important for comparing and harmonizing studies as well as for following children across multiple developmental stages. A valuable resource, funded by NIH is the Phenotypes and Exposures (PhenX) toolkit, which includes a pediatric development research domain with a wide variety of freely available protocols (Enlow, 2022; RTI International, 2025). NICHD has a Data and Specimen Hub (DASH) repository that includes protocols, manuals of operation, case report forms, and data. Some of the studies collect family, lifestyle, demographic and other types of data (NICHD, n.d.).

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Conclusion 5-3: There is a need to invest further in pediatric-oriented measurement science at NIH, which would expand the tools available to support data collection for all age ranges in NIH-wide initiatives.

Adjustments to Study Design

Research designs tailored for adults often need to be adjusted for studying children, because the physiology, pharmacokinetics, cognitive skill, and responses to treatment of children differ significantly from those of adults. Developing age-appropriate research requires specialized knowledge and infrastructure. For example, implementing a pediatric physical therapy program may require child-sized equipment, sensory-friendly spaces, and trained staff who can adapt exercises to different developmental stages. Pediatric clinical trials require cautious and careful determination of safety factors and drug dosing to account for the physiological and pharmacokinetic properties of children, which may impact participants' level of risk.

Addressing Barriers to Pediatric Health Research Through Community Engagement

To establish partnerships, researchers have to meet children where they are and collaborate with community organizations to connect with children and their families. Because enrolling participants in pediatric research requires asking parents or guardians to enroll their children in a study that may pose risk, pediatric research arguably requires greater trust in research institutions than does adult research. Entire communities may be deterred from participating in pediatric research if researchers do not, for example, practice cultural humility and linguistically appropriate engagement. The use of interpretation and translation services is essential for engaging children and families who are non-English-speaking or for whom English is not their primary language (Chong et al., 2025). Some communities have historical or ongoing reasons not to trust the health care system and the research enterprise (George et al., 2014). Many of these concerns and issues of mistrust may be mitigated by using community engagement tools (Reber et al., 2025; Sprague Martinez et al., 2018). Meaningfully engaging communities through building lasting partnerships is increasingly recognized as a component of research that is ethically important and that can help to address these barriers to research (Emanuel et al., 2004; Sprague Martinez et al., 2018).

Community engagement in research is not only valuable in and of itself, but it can also serve a number of goals, including increasing trust, transparency, and buy-in; understanding how to minimize risks of research based on the community's understanding of the local context and culture; addressing

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misinformation; and ensuring that research is responsive to community needs (Hood et al., 2010; Shah et al., 2020). Approaches to community engagement vary widely and can be conceptualized on a spectrum from no community engagement to community control (Arnstein, 1969). Sharing information and seeking input with communities (community notification and consultation) are more limited forms of community engagement, while strategies for coproduction and cocreation, such as community-based participatory research, are considered the highest level of community engagement. More than one strategy may be used within a single study, and the level of community engagement needed or even desired by the community may vary from one study to another. More robust engagement in research could help to increase trust and transparency, ensure that researchers can address actual (as opposed to hypothetical) community concerns, and ultimately ensure the success of pediatric research.

The creation of patient and family advisory councils (PFACs, also called patient advisory boards) is one increasingly common approach to community engagement. This approach can be particularly relevant for studies that include children with complex health conditions (Richard et al., 2020). While PFACs can provide essential insights into study relevance, burden, and feasibility, their use in pediatric research remains uneven (Lewis et al., 2025). Some NIH requests for application (RFAs) have added community partners to the applications (e.g., community-partnered nursing research centers, the Environmental Health Sciences Core Centers Program) (NIEHS, 1999, 2024; NINR, 2025). Making the use of PFACs routinely could better align research with the lived experiences and priorities of young participants and their families. Increasing access to guidance on recruiting PFAC members, educating families about the value of pediatric health research, building trust between families and researchers, and providing training for all parties on how to engage in a cooperative research protocol will facilitate the inclusion of PFACs into pediatrics research.

Addressing Contextual Barriers through a Focus on Dissemination and Implementation Research

Lessons from implementation and dissemination research may also offer new ways to lower barriers to the successful implementation of interventions that promote pediatric health. Implementation and dissemination research can help ensure that an intervention accounts for the context in which it will be implemented, whether that is a clinical or community setting; delivered by a clinician, teacher, or caregiver; or differs based on what is developmentally appropriate (Smith and Polaha, 2017). NIH defines implementation science as the "study of methods to promote the adoption and integration of evidence-based practices and interventions into routine

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health care and public health settings to improve our impact on population health" (NCI, n.d.). When investigators imbue their research with a focus on translation, implementation, and dissemination, they can also promote trust-building among the communities with whom they are conducting research, integrating co-creation and co-production approaches into their research, including soliciting feedback on research questions with the intervention context in mind (Israels et al., 2024).

Through contextually tailored dissemination and implementation strategies such as stakeholder engagement, adaptation of interventions to local needs, and iterative evaluation, researchers can enhance the relevance, uptake, and sustainability of pediatric health innovations across diverse populations. Table 5-2 shows that there has been an increase in pediatric dissemination and implementation funding at NIH from fiscal year (FY) 2021 through FY 2024. Incorporating considerations about implementation and dissemination for interventions and treatments for pediatric populations can mitigate gaps between new research findings and actual use in clinical or community settings (Halle, n.d.; Samanta, 2020; Wittmeier et al., 2015).

Conclusion 5-4: The translation, implementation, and dissemination of new research findings into practice and policy is needed to improve the health of all children.

Structural and Procedural CHALLENGES AND Opportunities

Structural and procedural obstacles to conducting pediatric research include challenges involving grant mechanisms, scientific review processes, and administrative hurdles. Conducting pediatric research often requires additional time for recruitment, participant consent, and independent review.

TABLE 5-2 R18 or Pediatric Dissemination and Implementation Funding, FY 2021–FY 2024

FY	# of Eligible Pediatric Projects	Eligible Amount ($M)	% of Total Pediatric
FY 2021	451	187.3	3.43
FY 2022	581	244.7	4.29
FY 2023	700	280.2	4.53
FY 2024	829	318.9	5.15

NOTE: Summary of inclusion criteria: Activity code R18 ("Research Demonstration and Dissemination Projects"), ***OR*** Research, Condition, and Disease Categorization (RCDC) category includes "Dissemination and Implementation Research" or "Dissemination/Implementation."

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The need to establish a network of study sites along with forming collaborations with people and institutions in underrepresented communities can also extend the timeline for a pediatric research study. Additionally, pediatric clinical studies may require extended follow-up periods to track long-term outcomes in children. Given children's developmental trajectories, measuring outcomes at 6 or 12 months after intervention may be inadequate to assess the true effects of an intervention. These extended study timelines often do not align with the typical period of an NIH award.

Grant Mechanisms

Most grant mechanisms have a timeline of 9 or more months from application submission to receipt of a notice of grant award (NCI, 2020; NIAID, 2024). In addition, most initial submissions are not funded, so multiple application submissions are required, which extends the already long timeline to attain extramural support (see Figures 5-4 and 5-5). When support is obtained, several NIH institutes implement procedures for reducing the total individual award so that more awards can be made to additional investigators. Although this increases the number of funded proposals, it reduces the proposed budget for many projects.

The very long review process precludes rapid revision and resubmission. NIH funding requires planning and a significant amount of effort on the part of investigators and their institutions. These issues are experienced by all investigators who seek extramural funding from NIH, not just those conducting pediatric research. However, studies that include children may have an extended period of review because there is a need to address additional safeguards for children as a vulnerable population (Kastner et al., 2015; Stewart et al., 2025). For example, studies that include children often cannot be exempted from IRB review or have an expedited review process (NIH, n.d.). Consent may take longer, and assent may be required. In addition, review bodies generally have a greater level of comfort with research that includes adult participants than they do with research that involves children.

The Scientific Review Process

The Center for Scientific Review (CSR) was established by the NIH in 1946 to oversee a fair and independent review process of grant applications and to ensure that the most promising research is chosen for funding (NIH CSR, 2025). The CSR maintains established expert scientific review committees, colloquially known as "study sections," that evaluate the significance, innovation, rigor, reproducibility, and methodological approach of research proposals from extramural investigators. These study sections are

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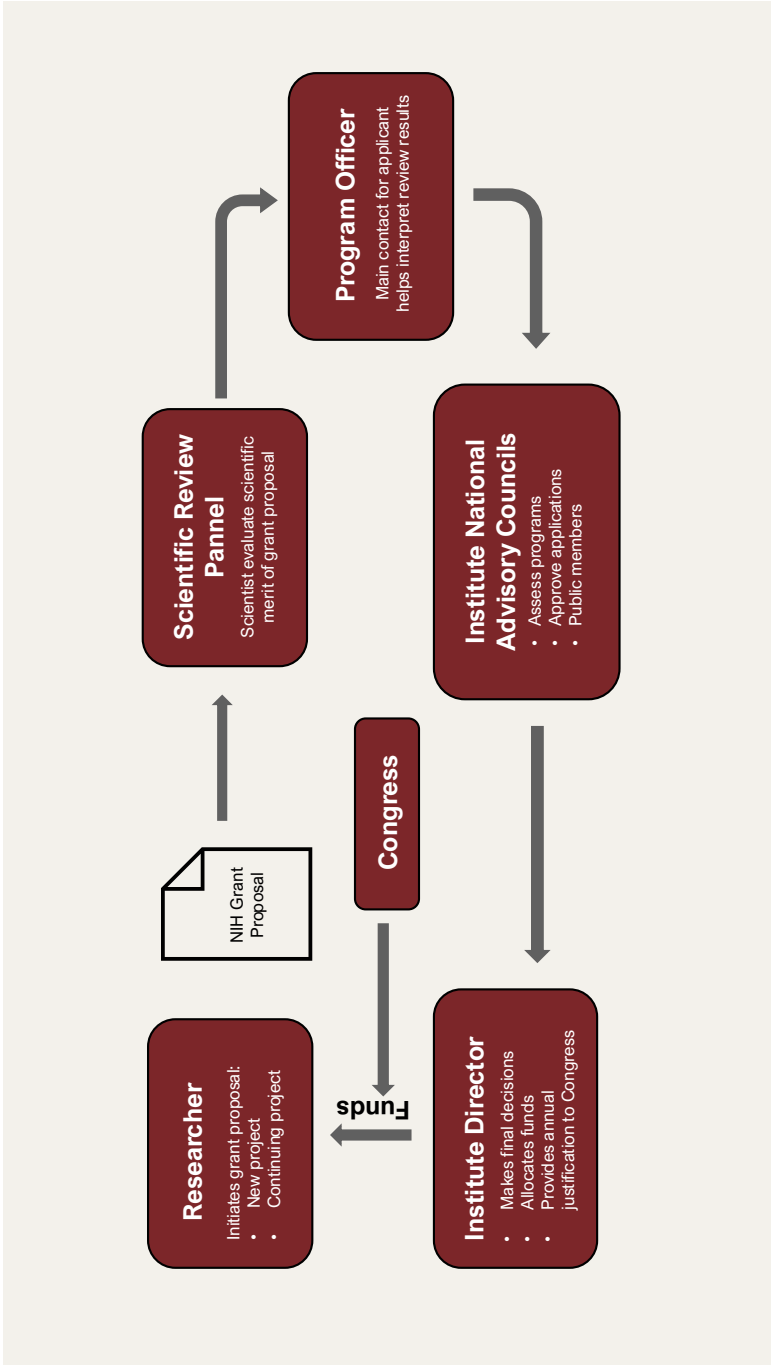

FIGURE 5-4 NIH grant application process.

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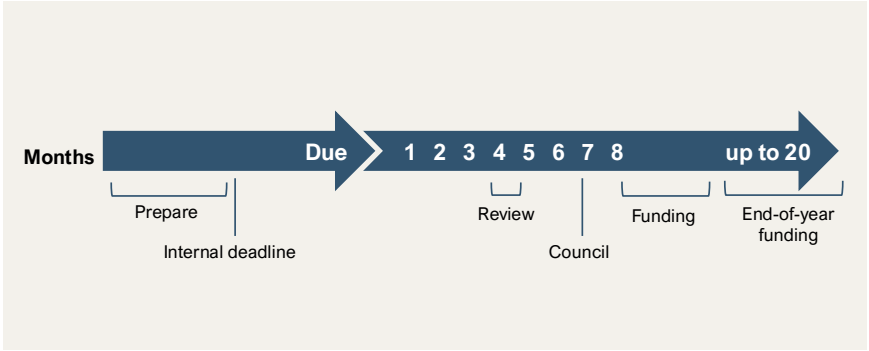

FIGURE 5-5 NIH grant application timeline.
SOURCE: NIH (2019).

composed of established investigators from institutions across the United States.

One obstacle for submitted pediatric-focused research proposals is that while expertise in pediatrics on study section rosters is critical for proposals that include enrollment, interventions, and observations of children, the amount of pediatric expertise can vary widely on a study section. Study section reviewers who lack experience with the intricacies around children as study subjects may be ill equipped to evaluate the merits of pediatric research proposals. The significance and impact of a pediatric study could be missed by a non-pediatric expert. In addition, for basic or translational research proposals, the importance and relevance for a pediatric disease and its mechanisms may not be evident to a reviewer who lacks pediatric research expertise. For these reasons, pediatric-focused clinical research proposals, including clinical trials, require rigorous evaluation by pediatric investigators, ideally clinician investigators.

There is considerable effort among CSR staff to maintain a diverse range of expertise on any given study section, and CSR uses ad hoc reviewers quite liberally to ensure expertise on study sections; however, it is difficult to estimate how *all* award applications involving pediatric research may align with study sections that have adequate pediatric expertise (Durrant, 2025). In an effort to develop a crude estimate of how well pediatrics is represented in study sections, the committee examined the rosters of permanent members of regular standing study sections and special emphasis panels (NIH CSR, 2023). The committee counted study section members as having pediatrics expertise if their primary affiliations were with a department of pediatrics at a medical school or they were employed at a children's hospital. It is worth noting that these percentages do not include individuals with primarily affiliations at hospitals that are primarily adult

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but might include a pediatric unit or those who might have secondary (or other) affiliations in pediatrics departments since it would not have been obvious in their affiliation that they have a pediatric affiliation or expertise.

The data in Tables G-1 and G-2 in Appendix G also very likely underrepresent what the committee would consider pediatric expertise, such as study sections focused on prevention science, which would inherently have both membership expertise in pediatrics and research applications heavily skewed towards child and adolescent populations, as was noted by Dr. Valerie Durrant in an open session with the committee (Durrant, 2024). The committee also did not have access to the number of pediatric-related applications in each study section and whether the applications reviewed in the study section required pediatric expertise. Additionally, Table G-1 does not include the count of ad hoc reviewers with pediatric expertise, so the percentage of reviewers with pediatric expertise on the study section may be slightly inexact. It is also worth noting that the two tables include two different timeframes with Table G-1 consisting of standing members participating in meetings for 1 year while Table G-2 includes panel members participating in one meeting occurrence. Special emphasis panels may use aggregate rosters, which can skew the proportion of reviewers with pediatric expertise, as these rosters include reviewers from various panels. Despite these clear limitations to this evaluation, the results offer an initial look at where pediatric expertise could be more significantly represented. Among the 187 active (as of April 2025) regular study sections, pediatric representation was low, with almost half (88/187; 47 percent) of them having no pediatric expertise represented in their primary affiliation (see Table G-1 in Appendix G). Only 24 study sections (13 percent) had three or more members representing pediatrics. The Reproductive, Perinatal, and Pediatric Health study section had the greatest number of pediatrics experts: nine of its 20 members (45 percent). Among the 3,459 permanent members of study sections at NIH, 204 (6 percent) represented pediatrics. Pediatrics was also poorly represented among the special emphasis panels, with 6 percent of the standing members (324 of 5,702 members) having pediatric expertise. Among the 138 special emphasis panels, 37 of them (27 percent) had no representatives from pediatrics (see Table G-2 in Appendix G). The potential lack of pediatrics expertise on study sections and special emphasis panels could make it difficult for pediatrics-focused submittals to be evaluated fairly if reviewers cannot fully understand the merits and mechanics of the proposals (see Box 5-2). Consequently, the distribution of pediatrics research across ICs could be affected, and the representation of pediatrics-focused principal investigators on NIH-wide initiatives could be lessened.

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BOX 5-2
The Challenge of Having Insufficient Pediatric Representation on Study Sections

"From a process perspective, NIH review panels may not always include reviewers with pediatric expertise, which can lead to undervaluation of the unique scientific and clinical significance of pediatric-focused proposals."

– Early career researcher and recipient of NIH grant funding

"Additionally, the smaller sample sizes and longer follow-up periods often required for pediatric studies can be perceived as limitations during peer review (and these longer follow-ups are associated with significant costs). From a process perspective, the current structure of study sections can disadvantage pediatric applications when they are reviewed by panels lacking pediatric expertise. This can lead to lower enthusiasm scores and reduced appreciation for the nuances of conducting rigorous research in neonatal or pediatric populations."

– Neonatal pediatric researcher and recipient of NIH grant funding

"Review processes seem dominated by scientists/researchers with an adult focus. These reviewers may not appreciate the significance and impact of pediatric-focused projects. Research review of pediatric projects by pediatric-focused scientists would be better."

– Pediatric oncologist who received grant funding from NIH

SOURCE: These quotes were collected from the committee's call for perspectives from NIH grant applicants; responses were collected by staff and synthesized by theme and anonymized to the committee (see Appendix C for the questions posted in the call for perspectives).

Conclusion 5-4: Representation of pediatric expertise on study sections is variable, which can create circumstances in which pediatric research proposals are reviewed by panels lacking pediatric expertise, potentially leading to inadequate reviews and lower prioritization.

Administrative Hurdles

Administrative burdens, including complex federal requirements, associated with the application processes for funding can be significant. NIH could improve processes to better support pediatric researchers to assist with regulatory requirements that are more challenging in pediatrics, including informed consent and assent. Given the special challenges facing

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pediatric researchers described above, further education of investigators and engagement with NIH program officers is key. "Navigators" could assist investigators who possess varying levels of expertise. Navigators could assist students, postdoctoral fellows, junior faculty, mid-level faculty, and senior faculty with respect to opportunities and processes. In addition, they could advise on the ever-changing landscape for proposals and on compliance with and adherence to policies and procedures, especially with regard to how they impact children and pediatric research. The navigators could better educate investigators on how to best use the resources that lead to success (engagement with program officer, the RFA process, etc.). The navigators would have expertise relative to pediatric research and could better guide applicants through the process. NICHD previously conducted a young investigator meeting with highlighted speakers and a mock study section; the feedback was very positive, and this could be a potential path for young investigator engagement.

In conclusion, pediatric research faces significant scientific and methodological barriers, such as difficulties in translating animal models to bedside care, challenges recruiting diverse sets of study participants, and the ecosystem of care which consists of medical, health, social and environmental aspects of child growth and development. Overcoming these limitations requires a multi-faceted strategy that will include improving recruitment and inclusion efforts, developing more accurate models of pediatric health, and valuing the long-term impact of pediatric research for both individuals and society. In Chapter 6 the committee expands on these ideas, presenting a vision of what pediatric health research could become both at NIH and via extramural research funded by NIH.

Recommendation 5-1: The National Institutes of Health (NIH) should work to create a culture of ethical inclusion of children in research, focused on protecting children *through* research rather than *from* research. To this end, NIH should develop measurable strategies and mechanisms to better implement and enforce existing policies to ensure inclusion of children at the project and proposal level. Strategies and mechanisms should include:

- **a. Updating the simplified review framework by explicitly considering the inclusion of children and its implications for health during the life course as a component of evaluating a proposal (i.e., its significance) during review.**
- **b. Continuing to support the NIH Center for Scientific Review in implementing and monitoring structural changes to support pediatric research:**

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- **Requiring and monitoring that pediatric representation is consistent with the number of applications on all relevant standing study sections.**
- **Ensuring provision of adequate justification for exclusion of children during grant review: If there is a scientific, legal, or ethical reason for the exclusion of children, this justification should be clearly articulated in the grant application and assessed and documented during the grant review process.**
- **Elevating prioritization of implications of research on the life course during scientific review: NIH should identify the impact of pediatric research on the life course as an important component of a proposal's significance section and should do so directly within reviewer guidance. In addition, training for study section reviewers should encourage them to consider whether a proposal adequately addresses the importance of the research for the life course when evaluating the significance or impact of extramural grant applications.**

c. **Improving transparency on inclusion: The Office of the Director of NIH should annually track and report justifications provided for the exclusion of children. This information should be included in the NIH annual report and published on the NIH Pediatric Research Consortium (NPeRC) website, or an NPeRC equivalent website, to track progress on the improved inclusion of children across NIH activities over time.**
d. **Providing clearer institutional guidance about the ethical inclusion of children in research: NPeRC (or NPeRC equivalent), in consultation with the NIH Clinical Center's Department of Bioethics, should develop a widely accessible toolkit and guidance on ethical issues in pediatric research that have the potential to unduly impede research if misunderstood, such as when to include children in research, provisions for assent, adolescent independent consent, age de-escalation, and payment for research participation.**

FINANCIAL BARRIERS FACED BY PEDIATRIC HEALTH RESEARCHERS

Insufficient Funding to Cover the Higher Costs of Pediatric Research

Funding for pediatric research often does not align with the actual costs required for pediatric trials, which can be more expensive and logistically

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complex than adult trials. (See Box 5-3 for some perspectives from recent NIH award applicants when asked to discuss their experience regarding any structural impediments to securing NIH funding for pediatric research; see Appendix C for the full list of questions posed in the call for perspectives.)

Pediatric Recruitment and Retention Challenges

For several reasons, recruiting and retaining children in clinical trials is more difficult than recruiting and retaining adults. First, parents or guardians are essential participants in recruitment and study processes. Caregiver distrust of academic research can challenge pediatric recruitments and often requires educating parents and fostering trust in the validity and value of clinical pediatric research (Cunningham-Erves et al., 2019; Rajakumar et al., 2009). Second, the participation of children is often contingent on the availability of family support, which can limit who can participate in studies. Third, pediatric clinical research often happens at select urban academic medical centers, which can make it difficult for families living in rural and underserved areas to participate (Lim et al., 2011). Fourth, inclusion of non-English-speaking participants is essential to ensuring a representative sample. This requires multi-lingual recruitment strategies (Beauchemin et al., 2024), especially since 22 percent of children live in homes in which a language other than English is spoken (Annie E. Casey Foundation, 2025). Fifth, it is simply difficult to obtain enough participants in pediatric studies for several reasons.

The pediatric population available for study for most specific diseases is significantly smaller than the available population for adult studies since most children are healthy. For example, while about half of adults in the United States have hypertension, it affects only about 4 percent of children. Thus, most pediatric research studies, especially for rarer conditions or diseases, require multiple study sites to recruit enough participants of the eligible population. Rare disease studies are a cornerstone of pediatric research, but low prevalence precludes recruitment and enrollment targets on par with standards in adult populations (Batshaw et al., 2014; Rees et al., 2019).

More centers mean greater infrastructure costs. A multi-site study may also require a consortium agreement among the institutions, which also increases cost and study complexity. Including more institutions in studies requires an increase in baseline infrastructure costs and necessitates multi-center IRBs, which are costly. As an example, the Chronic Renal Insufficiency Cohort (CRIC) study run by the National Institute of Diabetes and Digestive and Kidney Diseases included 13 clinical sites and enrolled more than 5,625 adults (NIDDK, 2024), compared with the concurrent Chronic Kidney Disease in Children (CKID) study, which required 50 clinical sites to enroll 1,100 children over a nearly 20-year period (NIDDK, 2025) (see Figure 5-6). Hospitals and research centers must contend with financial

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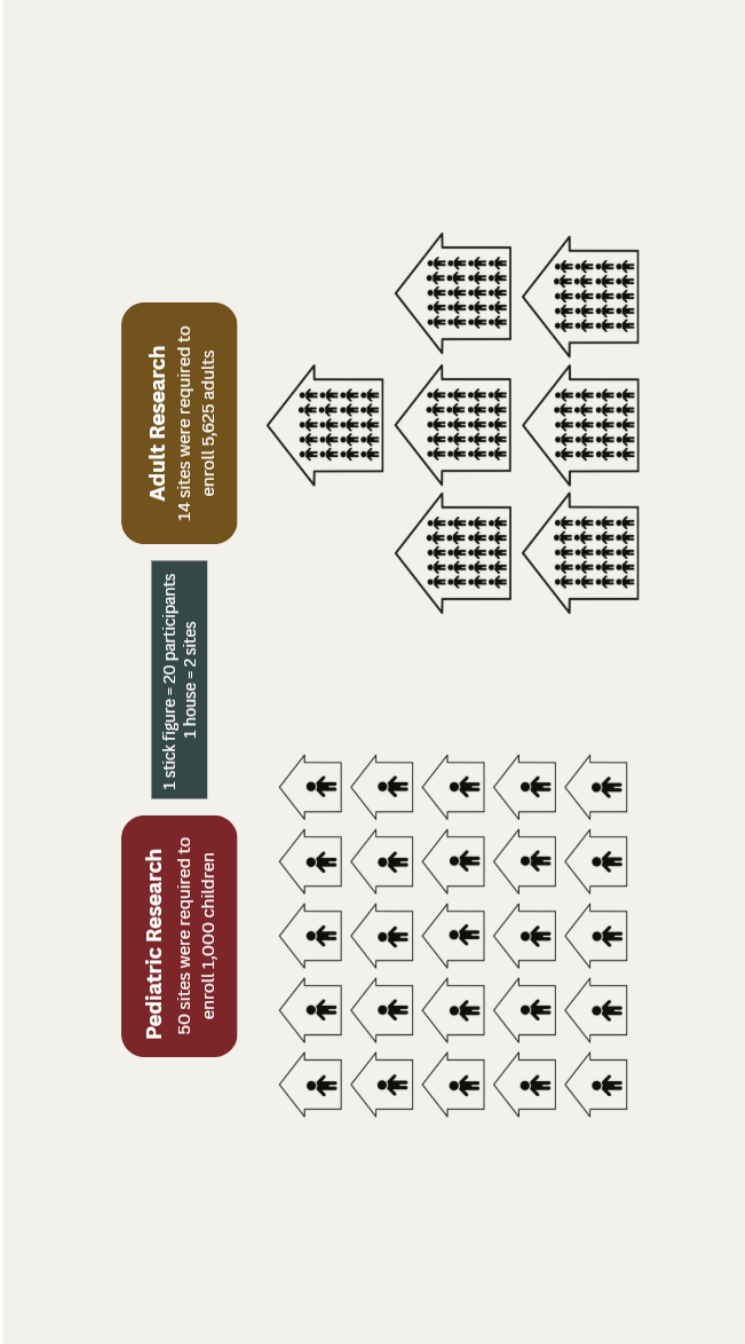

FIGURE 5-6 Comparison of sites needed for adult versus child enrollees in concurrent studies about renal disease.
NOTE: CKID = Chronic Kidney Disease in Children; CRIC = Chronic Renal Insufficiency Cohort.
SOURCE: Data from or derived from CRIC and CKID studies.

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BOX 5-3
Financial Impediments

"Investigator-initiated R01s for children's health-related research often get assigned to NICHD, but then the low paylines of the NICHD mean even if it was well-scored, it will not get funded."

– Recipient of NIH funding for 30 years from the National Institute on Drug Abuse (NIDA), National Institute on Alcohol Abuse and Alcoholism, and NICHD

"NIH has had the same cap on R03, R21, and R01 modular grants for >20 years despite inflation. This is absurd. Applicants can propose less and less research in order to fit the budget caps. These caps need to be raised to be more realistic. NIH needs to stop pretending research costs haven't risen in 20 years!"

– Experienced researcher (preclinical research in developmental neuroscience) with grants from the National Institute on Environmental Health Sciences, National Institute on Mental Health, and NIDA

"Conducting clinical trials in pediatrics is extremely difficult with the current NIH caps on direct costs. Since RA [research assistant], coordinator, grad student, technician salaries have increased over time, it leaves less and less money to conduct the research, provide stipends for participants, and ensure that there are robust facilities and infrastructure to support this work. Unless we figure out a way to share resources across an institution or between institutions, it will become more and more difficult to conduct clinical trials or interventions that can change lifestyle behaviors that promote a healthier life."

– Researcher studying childhood obesity treatment, parenting interventions, and related topics for over 15 years with NIH grant funding

SOURCE: These quotes were collected from the committee's call for perspectives from NIH grant applicants; responses were collected by staff and synthesized by theme and anonymized to the committee (see Appendix C for the questions posted in the call for perspectives).

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constraints when implementing large-scale research studies, further limiting their capacity to compensate parents for their time and potential lost wages. Timelines and endpoints may face a critical window depending on the age of recruitment with regard to a child's development and the need for longer follow-up that accounts for developmental changes. A 12-month follow-up may be appropriate for many adult studies, but this is often inadequate for children. This is particularly true for studies of drugs (IOM, 2004). Delays in funding or interrupted funding may limit the ability to follow up study cohorts.

Due to the accelerated pace of development in childhood and the fact that child development has critical periods that create the potential for delayed outcomes from interventions, advancing the science of pediatric medicine at times may require multiple sequential studies to ensure safety and efficacy across pediatric populations. For example, the NICHD Neonatal Research Network's whole-body cooling trial for hypoxic–ischemic encephalopathy demonstrated significantly reduced death and disability in infants (Shankaran et al., 2005) but could not confirm whether the benefits would persist at school age, a critical downstream outcome. To answer this important question, additional funding was obtained from NICHD for a 7-year follow-up following the results at 18 to 22 months. This additional investment confirmed the benefits of whole-body cooling in reducing mortality in 6- to 7-year-old children with hypoxic–ischemic encephalopathy (Shankaran et al., 2012).

Another financial barrier for pediatric research is the lengthier decision-making process as compared with adult research. For example, the informed consent process requires that at least one parent, and sometimes both parents and guardians, agree to the child participating in the research. If only one parent is present at a time, that parent may need to ask the other parent and may solicit advice from family members and friends before consenting on behalf of the child (Nelson et al., 2013). Further time may be needed for child assent, which is the process of explaining the study to the child and obtaining the child's agreement to participate. Another expense in pediatrics research is compensating parents or caregivers for missed-work time and travel to the enrollment site. Additional research time may be needed if the data collection includes parents, siblings, and the children enrolled in the study, which is often the case for pediatric studies. This too adds to the cost of the research.

One example of a need for extended follow-up was the Antenatal Late Preterm Steroid (ALPS) study from the Maternal Fetal Medicine Units Network, which showed that the administration of antenatal steroids (betamethasone) significantly reduced the need for respiratory treatment among infants born at late preterm (34–36 weeks of gestation) (Gyamfi-Bannerman et al., 2016). However, low blood sugar, or hypoglycemia, was more

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common in the infants exposed to betamethasone, raising a concern over safety. The initial proposal for the ALPS study included a 2-year follow-up, but both funding challenges and the uncertain outcome of the parent trial prevented this plan from coming to fruition. Participants in the parent trial were asked to consent to the possibility of future contact with the researchers (Gyamfi-Bannerman, 2024). Given the desire for long-term follow-up to monitor low blood sugar and its impacts on children who received betamethasone before delivery, funding was secured for further assessment, and no differences were found at 6 years of age between the exposed and unexposed children (Gyamfi-Bannerman et al., 2016, 2024). These results offered reassuring data regarding the lack of impact of hypoglycemia on the study participants. The ALPS study and the whole-body cooling trial are good examples of why it is necessary to have supplemental funds available for long-term follow-up assessment of efficacy and safety in pediatric clinical studies.

Conclusion 5-5: Pediatric research faces unique barriers that increase the duration, cost, and complexity of studies. These barriers include, but are not limited to, the need for extended monitoring to capture developmental outcomes, multi-site collaboration to ensure representative and adequately sized samples, and, at times, challenges arising from misunderstood or misapplied ethical standards, such as the common practice of enrolling older children before younger ones ("age de-escalation") without considering when it serves as a protection and when it does not. These barriers contribute to a culture of overprotection and undue delay in the inclusion of children in research.

To address these additional hurdles faced by pediatric research, structural solutions to accelerate research become even more important. One such attempt to do so is awards made by the National Center for Advancing Translational Science (NCATS). NCATS has a Clinical and Translational Science Awards (CTSA) program that supports a network of institutions working on the translation of research discoveries into improved care (NCATS, 2025). While information provided by NCATS shows that nearly all CTSAs engage in pediatric research, only 5 to 7 percent of trainees and scholars focus on pediatric research, and merely 20 percent of pilot awards involve pediatric research. There are 16 CTSA program hubs which are led by principal researchers or co-principal investigators who are also pediatricians. Ninety-one percent of the hubs include children's hospitals. However, only about 6 percent of the CTSA publications are pediatric in nature (Llewellyn et al., 2020). Given that children make up 22 percent of the U.S. population, it would seem that more resources should be directed towards additional pediatric studies in the CTSA program. It was

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difficult for the committee to discern the actual dollar amounts that are directed towards pediatric research through the CTSA program. The current program announcement asks for inclusion or exclusion of children as required by statute to determine justification in term of scientific goals and research strategy (e.g., HHS, 2025). The guidance for the reviewers states, "Women, members of racial and ethnic minority groups, and individuals across the lifespan (including children and older adults) must be included in all clinical research studies, as appropriate for the scientific goals of the work proposed" (NIH, 2025). The reviewers are asked to justify inclusion as acceptable or unacceptable. Stressing the importance of inclusion of pediatric research to reviewers of CTSA programs, along with financial incentives for pediatric research, may encourage greater inclusion of pediatric research.

Recommendation 5-2: National Institutes of Health (NIH) institutes and centers should award pediatric research grants and budgets that support the specific needs of pediatric studies. In addition, to address the mismatch between study timelines and funding cycles, NIH should:

- **a. Allow for extended or extendable award timelines and/or renewable grant mechanisms specifically designed for pediatric research, allowing investigators to sustain long-term studies that align with children's developmental trajectories;**
- **b. Provide higher budget maximums to account for the need for representative recruitment and multi-center studies, when scientifically justified;**
- **c. Create supplemental funding opportunities specifically designed for longitudinal or follow-up data collection, which would enhance learning across the life course while capitalizing on previously funded and ongoing studies;**
- **d. Provide funding supplements to support multilingual research and community engagement to facilitate representation for generalizable results; and**
- **e. Increase access and usage of Clinical and Translational Science Awards as a mechanism for promoting pediatric research.**

Appropriations for NIH Funding

There are research projects that may require 10- to 15-year-long financial commitments from NIH. Mechanisms for such long-term funding are not yet in place. There would need to be some assessments over time to ensure that milestones are being met by the investigators.

NIH funding is dependent on timely appropriations. When appropriations are delayed, as has been the case for most of the last two decades,

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institutes and centers (ICs) are hesitant to fund grants above a certain percentile (known as the payline) in an effort to ensure fairness over the fiscal year and to stay within their eventual budget. An additional impact of delayed appropriations is that many ICs impose reductions in the applicant's requested budget. For example, in 2024 the average budget reduction to an R01 from NICHD was approximately 14 percent (NICHD, 2024). Since NICHD was the largest funder of pediatric research in 2023 ($993 million), these project budget reductions can disproportionately affect NICHD's pediatric grantees. Delays in appropriations also slow the onset of award notices and, in turn, research projects, which hampers potential discoveries and delays their clinical and therapeutic benefits. A mechanism that guarantees yearly funding of ICs at the start of the fiscal year would make research more efficient and streamlined and would likely accelerate the pace of discovery. An additional challenge is that many NIH ICs have reductions in the requested budgets they submit to Congress.

THE STRATEGIC IMPORTANCE OF INVESTING IN PEDIATRIC RESEARCH

Investing in pediatric research is a crucial precursor to advancing pediatric health, itself a strategic necessity for the United States. A healthy generation of children is the foundation of a resilient, innovative, and secure nation, directly influencing the state of the nation's future workforce, military readiness, economic productivity, and societal stability. A robust pediatric research enterprise will enable the kind of preventive interventions, early diagnostics, and targeted therapies that can dramatically alter life course trajectories, improving short- and long-term health outcomes (Halfon et al., 2022; NASEM, 2024).

As introduced in Chapter 1, U.S. children face nearly universal risk for chronic disease and mental health issues when compared with peers in other high-income nations (Forrest et al., 2025). This is a sobering indicator of systemic vulnerabilities in U.S. health care and social support systems, which are discussed in detail in the recent National Academies report *Launching Lifelong Health by Improving Health Care for Children, Youth, and Families* (NASEM, 2024).

According to a recent U.S. military report, more than 70 percent of 18-year-olds are ineligible for service without a waiver due to chronic health and mental conditions (DOD, 2022). Moreover, half of all chronic mental disorders begin at 14 years of age, and 80 percent of obese adolescents will become obese adults (Bibbins-Domingo et al., 2007; Kessler et al., 2005; Simmonds et al., 2016). Chronic diseases and mental health conditions that begin in childhood often persist into adulthood, compounding health care costs and reducing quality of life. Without a healthy youth

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population, the nation's capacity to maintain a robust military, respond to global challenges, and sustain economic competitiveness is compromised.

Investment in evidence-based health and social services and, importantly, the research needed to create them benefits children and their families and leads to greater adult productivity by way of a healthier labor force (NASEM, 2024). Children who grow up without the burden of chronic disease are more likely to thrive academically, socially, and professionally (Maslow et al., 2011). This is especially critical in an era when precision medicine and genetic therapies are rapidly advancing fields in which pediatric research has already yielded groundbreaking successes, such as curative gene therapy for spinal muscular atrophy (Finkel et al., 2017). The impact of NIH-supported pediatric research and its discoveries cannot be overstated: lifespan-extending treatments for cystic fibrosis, vaccines that have prevented countless deaths and hospitalizations, and tailored therapies leading to a 90 percent cure rate for acute lymphoblastic leukemia. For example, a Centers for Disease Control and Prevention analysis found that among approximately "117 million children born during 1994–2023, routine childhood vaccinations will have prevented approximately 508 million lifetime cases of illness, 32 million hospitalizations, and 1,129,000 deaths, at a net savings of $540 billion in direct costs and $2.7 trillion in societal costs" (Zhou et al., 2024).

The benefits of investment in pediatric research are exponential in the number of increased and healthier life-years across the life course to which they contribute. Healthier children and adolescents will become the next generation of Americans.

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6

Implementing the Committee's Vision for the Future of Pediatric Health Research at NIH

"As the mother of a child who died from diffuse intrinsic pontine glioma (DIPG), my experience with NIH-funded pediatric research is shaped by both deep gratitude and painful frustration. I'm grateful for the foundational discoveries NIH has made possible, such as the identification of the H3K27M mutation, which helped define DIPG as a distinct disease. . . My son at age 4 participated in four early-phase clinical trials. These were not easy decisions; they were desperate ones. The research offered the only possibility of hope. Even though those trials didn't save his life, we chose them because doing nothing was not an option. We did it to push science forward and to help the children who would come after him-because that is the only way we are going to find a treatment."

— Parent of young child NIH research participant

Our future society is already present among us in the form of children. Imagine a future where every child in the United States is given not just the chance to survive, but to thrive as a child and future adult, bolstered in a health care system rooted in discovery, access to all, and innovation. In this future, National Institutes of Health (NIH)-funded research into child health is not an afterthought but a national priority, built on investments that rival our most ambitious scientific endeavors. In this future, children, from the first days of life, benefit from our comprehensive understanding of the biological, environmental, and social factors that shape life course development of health, well-being, and resilience.

Prevention lies at the heart of this vision. NIH-supported breakthroughs in genomics, environmental monitoring, and digital health converge to allow researchers and clinicians to identify risks before disease takes hold.

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Predictive models help families and clinicians intervene early to prevent asthma, diabetes, mental illness, and other chronic conditions long before symptoms appear. Childhood becomes the foundation for a healthy adulthood, with early interventions that transform the trajectory of lives and eliminate disparities that have persisted for generations.

In addition, a new era of therapeutics redefines what is possible for children with serious illness. Gene editing, immune modulation, artificial intelligence (AI), and regenerative medicine lead to cures, not just symptom control, for conditions that once led to lifelong disability or early death. Preventative strategies, treatments, and cures become personalized, safe, and developed from their inception with children in mind. The research and discovery that NIH supports today fuels a tomorrow in which no child's potential is limited by biology, birthplace, or the inequities of our past systems.

Children today have more opportunities for health and longevity than at any point in history. Yet rising rates of chronic conditions, including obesity, anxiety, and other health challenges signal that this progress is uneven and fragile. NIH has a unique opportunity to lead a new era of research that illuminates how rapidly evolving exposures and experiences shape child health trajectories. This includes studying the effects of pervasive electronic device use, information ubiquity, and shifting food environments as well as emerging environmental and social determinants. By investing in rigorous, child-centered science on these modern exposures, NIH can generate actionable knowledge that empowers families, clinicians, and communities to foster resilience, prevent disease, and optimize lifelong health.

To build this future, the nation will need to act with urgency and intention. Success will require us to center children in the NIH research agendas and foster collaborations that unite families, communities, and researchers. The reward is profound: A generation of children who grow up healthier, stronger, and more fully able to contribute to the United States and the world around them. This chapter reiterates recommendations made in the preceding three chapters, organized under the committee's four goals discussed in Chapter 1, and describes how this future could look if the recommendations are successfully implemented.

GOALS AND RECOMMENDATIONS FOR NIH-SUPPORTED PEDIATRIC RESEARCH

Pediatric health is a cornerstone of our national well-being. Increases in the U.S. average lifespan during the 20th century were in great part due to declines in child mortality caused by infectious diseases, prematurity, trauma, cancer, and other common problems. These advances would never have happened without the significant and consistent federal investment,

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led by NIH (and supported by Congress), in basic, translational, and clinical research conducted by intramural NIH investigators and investigators across the nation in our universities, children's hospitals, and biotechnology and pharmaceutical industries.

NIH is one of the most prestigious and revered research institutions in the world. As the world's leading funder of biomedical research, it has the opportunity and responsibility to also be the world's leader in advancing and innovating pediatric health research. This report provides the evidence and recommendations for how NIH, with the support of Congress, the executive branch, and partner federal agencies, can best fund, support, and coordinate research in pediatric health. This closing chapter recapitulates those recommendations in the context of the committee's vision and goals.

More than ever before, federal and federally funded biomedical research is under heightened scrutiny. Debates over the size, scope, and organization of NIH and calls for increasing fiscal restraint pose significant challenges to long-term planning, infrastructure development, and the pursuit of bold scientific goals to improve the health of U.S. children. Perhaps paradoxically, these pressures also create an opportunity to reaffirm the unique value of pediatric research to the health and well-being of children and, because our children are our future, to demonstrate the significant return on investment our nation gains in health, prosperity, opportunity, innovation, and growth by prioritizing pediatric health research that is comprehensive, equitable, and groundbreaking.

When investments in pediatric health research are designed with rigor, focused on outcomes, and grounded in the needs of our children and the nation, they should command broad support across the federal government and the public. When these investments are visibly linked to national priorities such as educational readiness, workforce development, economic productivity, national security, and family well-being, there should be support for our children by funding and carrying out basic, clinical, and translation research. Children experience rapid, complex developmental, physical, and psychosocial changes with lifelong implications for their health, well-being and resilience. The United States needs a new vision for pediatric research that positions pediatric research as a dynamic, essential driver of population health across the life course, rather than one limited to the pediatric years. This chapter presents the committee's vision, in which there is a NIH-led pediatric research enterprise that is coordinated, data-enabled, and impact-oriented, designed to deliver lasting health benefits for all children while maximizing efficiency, accountability, and scientific relevance in an era of constrained resources. In this envisioned future, pediatric research that is efficiently stewarded and adequately resourced by NIH generates knowledge that is urgently needed, broadly applicable, and visibly impactful.

The committee was tasked by NIH to examine its portfolio of pediatric health research, and to make recommendations on how NIH could improve

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the support, management, and coordination of pediatric research throughout its institutes and centers (ICs). This report has outlined the committee's areas of focus and has detailed its approach to assessing the effectiveness, strengths, and opportunities of the current NIH pediatric research portfolio. The committee developed a vision for where U.S. pediatric health research should head, and, to actualize that vision, the committee created a set of four goals (see Box 6-1). Figure 6-1 shows the downstream impacts that would occur with the achievement of the four goals.

The committee was tasked with making recommendations "focused on improving NIH's overall support of child health research" while maintaining strong consideration for the burden on researchers and NIH staff. The committee's recommendations serve as a series of action points that, if implemented, would help achieve the goals and ultimately improve pediatric health outcomes.

BOX 6-1
The Committee's Vision for Pediatric Research at NIH

The United States will be the global leader in promoting child health, preventing childhood diseases and morbidity, reversing recent declines in child health, and using scientific discoveries, through a robust, dynamic, and integrated pediatric research program at the National Institutes of Health (NIH). These goals will ensure that all children are supported, resilient, and thrive across the lifespan. To accomplish this vision, NIH would:

GOAL 1: Integrate pediatric research throughout NIH research programs and initiatives, thereby broadening the community of multidisciplinary pediatric health researchers.

GOAL 2: Expand NIH initiatives and programs to address unmet research needs in child health, focusing on prevention, diagnosis, and treatment of illness and injury while also advancing new strategies that promote and optimize children's overall health and well-being.

GOAL 3: Strengthen the impact of pediatric health research on child and adult health outcomes by optimizing the translation, dissemination, and implementation of research into practice.

GOAL 4: Build trust in pediatric health research through communication about and dissemination of rigorous research findings that improve health trajectories across the lifespan for all.

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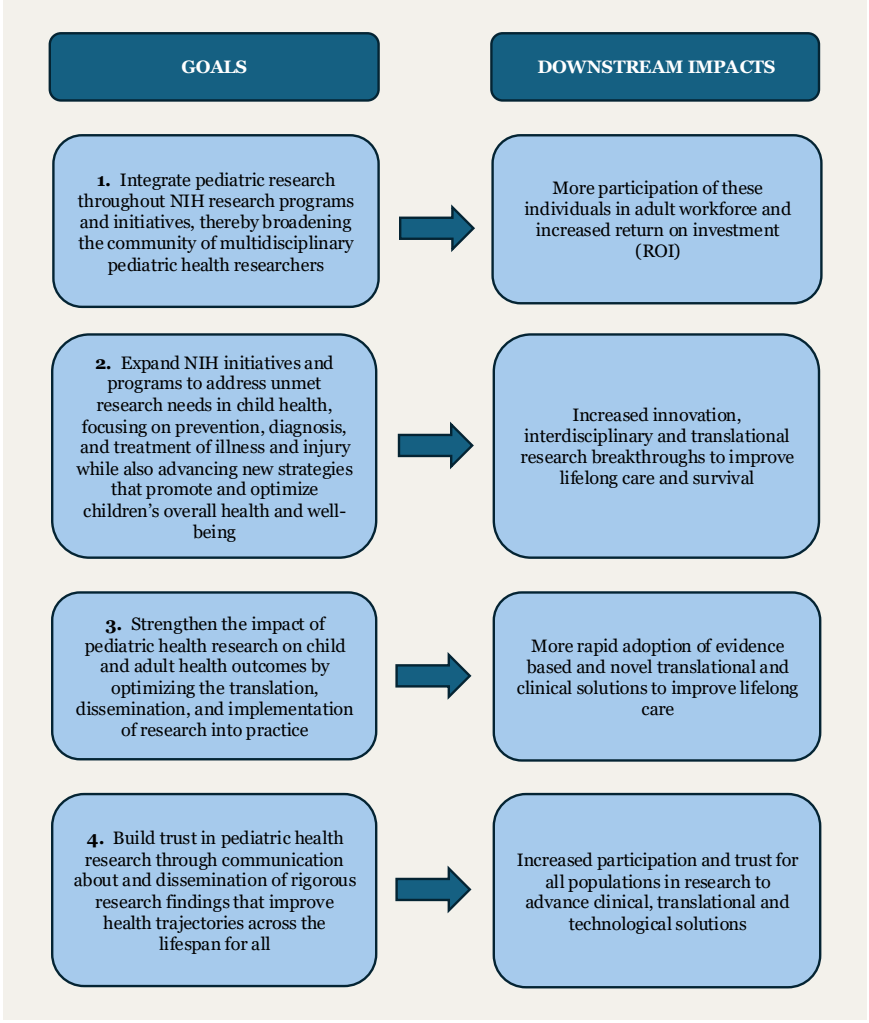

FIGURE 6-1 Committee goals and downstream impacts.

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Goal 1: Integrate pediatric research throughout NIH research programs and initiatives, thereby broadening the community of multidisciplinary pediatric health researchers.

Recommendation 3-1: The National Institutes of Health should adopt and apply a consistent agency-wide definition of pediatric research. The definition should recognize that pediatric research may include studies that: (1) involve data or specimens from or about infants, children, adolescents, or young adults; (2) inform the prevention, diagnosis, or treatment of conditions affecting these populations; (3) advance the health, well-being, or resilience of children, adolescents, or young adults; or (4) investigate childhood origins of adult health conditions.

Historically, NIH has not consistently defined pediatric research across ICs. The committee recommends that NIH adopt the definition above to standardize what research is categorized as pediatric and to promote consistency in reporting. Pediatric research may encompass several distinct areas of study: research in which participants do not specifically exclude children; research in which participants specifically include children; or research on children with a clinical or public health relevance focus on conditions or developmental processes that typically originate and manifest in childhood.

Recommendation 3-2: The National Institutes of Health (NIH) should improve the Research, Condition, and Disease Categorization (RCDC) system to better assess funding for pediatric research by (a) adding the new spending categories and (b) improving the Categorical Spending webpage in RePORT.

The following new spending categories should be updated:

a. **Add pediatric developmental stages: during pregnancy, neonate (≤28 days), infant (<1 year old), early childhood: age 1–2 years (1- to 2-year-olds), age 3–5 years (3- to 5-year-olds), middle childhood (6- to 11-year-olds), adolescence (12- to 17-year-olds), and young adults (18- to 24-year-olds).**
b. **Add a Basic Science spending category and ensure its validity.**
c. **Add new terms for pediatric health conditions to better reflect the breadth of pediatric health conditions. The process to determine these new terms should include child health expertise from each institute and center (IC).**
d. **To improve specificity of diagnostic labeling, consider switching eponyms to indicate diseases—e.g., change Cooley's Anemia to Beta Thalassemia Major".**

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To improve the Categorical Spending (RCDC) webpage in RePORT, NIH should:

- **a. Publish a reference paper that clearly and comprehensively describes the RCDC classification methodology with sufficient detail that it can be replicated or a similar assessment can be done by the scientific community. Update the methodology report whenever the methodology changes.**
- **b. Evaluate the validity of key RCDC terms, such as "pediatric" and update the classification to improve accuracy and remove possible biases.**
- **c. Allow more than one RCDC term to be exported in a specific project. (This functionality currently exists in RePORTER, but not RCDC.)**
- **d. Enable download of all projects funded by the NIH for a given year.**
- **e. Add a flag to clearly identify extramural, intramural, and, specifically, Clinical Center research and ensure that this information is available for all research projects.**
- **f. Add a search field that links projects to their respective notice of funding opportunity, program announcement, program announcement with special review, request for application, notice of special interest, or request for proposal.**

Currently, it is challenging to study NIH funding of pediatric research because of deficiencies in the RCDC system that is used to categorize funding. The two recommendations above add transparency and clarity into the types of funded projects. Grant information in the future will be coded with more specific pediatric codes denoting the developmental stages and common pediatric health conditions that are currently missing in RCDC, making NIH-funded research more transparent and easier to track, thus helping to improve efficiency and accountability. RCDC will also have a code for basic research making it possible to distinguish it from translational and clinical research.

Recommendation 4-1: All directors of National Institutes of Health institutes and centers that fund research should explicitly incorporate pediatric health and potential downstream life course impacts into their strategic plans, as childhood chronic conditions, exposures, and experiences may have disproportionate health impacts into adulthood. Include metrics such as those focused on investigators (e.g., funding rates and career advancement), scientific discovery (i.e., publications and innovations), impact on child health (e.g., morbidity, mortality), and downstream life course impacts (e.g., premature cardiovascular disease) to measure success in meeting their goals.

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The committee recommends that each IC explicitly and transparently consider pediatrics and plan for the ways that they will fund research to improve child health. Our recommendation would result in all of the 27 NIH ICs including pediatric research in their strategic plans, which act as a roadmap for establishing goals and research priorities. Including pediatrics in the written strategic plan will also help ICs and the NIH Pediatric Research Consortium (NPeRC) identify gaps and redundancy across the NIH's global plan for pediatric research.

Recommendation 4-2: The National Institutes of Health (NIH) director should:

- **a. Elevate the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to the Office of the Director, with membership including senior leaders within each institute or center (IC) with pediatric health and life course expertise, who systematically review all relevant components of strategic plans, including progress and impact made in the past year. Each senior leader should then report back to their IC and to the NIH director.**
- **b. Provide appropriate infrastructure funding and adequate resources (including personnel) to enable NPeRC (or its equivalent) to accomplish its goals and for its members to participate in its work, which includes (1) identifying and addressing research gaps, (2) enabling a nimble response to emerging threats to pediatric health, (3) promoting greater efficiency and reach of ongoing initiatives through data harmonization, (4) spearheading novel partnerships, (5) providing consultation to ambitious inter-IC initiatives, and (6) ensuring representation and visibility at national scientific meetings and societies.**
- **c. Have NPeRC's membership (or its equivalent) include expertise in pediatric bioethics. NPeRC should also partner with patient advocacy or community representatives and develop a communications plan to disseminate pediatric research findings to both the scientific community and the general public. NPeRC (or its equivalent) should be prepared to coordinate the NIH-wide response to any emerging pediatric research needs.**

The inclusion of children in NIH-wide and inter-IC initiatives will be facilitated by an empowered NPeRC or its equivalent. Historically, it has been difficult to identify what NPeRC has done to support and enable NIH-funded pediatric research. Only through extensive requests for information and follow-up questions has the committee been able to identify many of the vitally important contributions NPeRC has made. This recommendation

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would lead to an NPeRC that is overseen by the Office of the Director and is given sufficient resources and authority to be able to bring ICs together to foster the inclusion of pediatric research in all NIH-wide initiatives and to sponsor the development of new initiatives focused specifically on children.

Recommendation 5-2: National Institutes of Health (NIH) institutes and centers should award pediatric research grants and budgets that support the specific needs of pediatric studies. In addition, to address the mismatch between study timelines and funding cycles, NIH should:

- **a. Allow for extended or extendable award timelines and/or renewable grant mechanisms specifically designed for pediatric research, allowing investigators to sustain long-term studies that align with children's developmental trajectories;**
- **b. Provide higher budget maximums to account for the need for representative recruitment and multi-center studies, when scientifically justified;**
- **c. Create supplemental funding opportunities specifically designed for longitudinal or follow-up data collection, which would enhance learning across the life course while capitalizing on previously funded and ongoing studies;**
- **d. Provide funding supplements to support multilingual research and community engagement to facilitate representation for generalizable results; and**
- **e. Increase access and usage of the Clinical Translational Science Awards as a mechanism for promoting pediatric research.**

"Pediatric research should not be seen as a niche within adult medicine, but as foundational science that informs health across the lifespan. Prioritizing child health research is not only an investment in vulnerable populations, it is a strategic imperative for addressing long-term public health, as healthy children grow up to be adults."

– Neonatal pediatric researcher and recipient of NIH grants

Currently, most studies do not include children. Unfortunately, this is despite updated requirements for providing justification for any exclusion of children. In the committee's vision, it is unacceptable to exclude children from a clinical study simply because it is somewhat difficult for the investigators. The committee envisions that the NIH adopts and rigorously applies the following policy: children will be included in all research funded by NIH, unless including them is not scientifically justified. Integrating this policy change into NIH begins with weaving it into the fabric of each IC's strategic plan. This perspective then guides the development of programs within the ICs, funding opportunity announcements, and intramural research programs at each IC.

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> "We are challenged by time for pediatric research. To acquire enough data on enough patients within a 5-year funding mechanism is very hard. It is even more challenging when you are wanting to track change over time, which is important in child development. Longer-term funding mechanisms would be very appropriate for many studies of pediatric conditions that require longitudinal surveillance."
>
> – NIH grant recipient

Every adult was once a child, and children are our country's future. Research to support the current and future health of children must be prioritized. The committee envisions that the NIH extramural research program will be organized to ensure that pediatric research is appropriately prioritized. Since most diseases in children are much less common than in adults, pediatric research studies will routinely require multicenter involvement to generate scientifically adequate sample sizes. The extra cost of many such studies is not feasible under the current $500,000 direct-cost annual cap for most R01-level grants, and, thus, this will be increased to enable rigorous pediatric research to be conducted. The committee proposes that the NIH use incentives to allow for longer research timelines to accommodate for developmental changes as they pertain to recruitment and follow-up.

Goal 2: Expand NIH initiatives and programs to address unmet research needs in child health, focusing on prevention, diagnosis, and treatment of illness and injury while also advancing new strategies that promote and optimize children's overall health and well-being.

Recommendation 4-3: Initiatives within and across the National Institutes of Health (NIH) institutes and centers should coordinate with the NIH Pediatric Research Consortium (NPeRC) (or its equivalent) to include children and/or the downstream life course impact of childhood chronic disease, exposures, and experiences in studies in a strategic and deliberate way. NPeRC (or its equivalent) should support the enforcement of existing policies requiring that studies not exclude children or childhood downstream impacts on the life course without clear and adequate justification.

Initiatives across the institutes and centers of the NIH can lead to transformations in the diagnoses, treatment, and prevention of disease and the enhancement of health. The committee envisions that inter-IC initiatives will coordinate with NPeRC to include pediatrics in research from the beginning of studies unless there is a scientific justification for excluding

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children. Because ethical considerations are sometimes highlighted as a barrier to the inclusion of children in research, the committee envisions that the NIH will provide strong leadership in developing and disseminating foundational ethical guidance for research on pregnant women and children. This includes guidance about when it is acceptable that pregnant women and children are excluded from research. The committee envisions that the NIH be a leader nationally in the translation of this ethical guidance into appropriate regulations, thereby helping researchers conduct ethical, rigorous research with children.

As the NIH develops foundational guidance on the ethical considerations of research in pediatrics, the committee recommends that the Office of Human Research Protections (OHRP) develop and disseminate clear guidance on when it is and is not appropriate to ask for assent from children participating in research and that it provide clarity on what state laws may prohibit adolescents from consenting to treatment. Developing a clear foundational ethical framework will support researchers across the United States who work with regulatory boards that have different levels of experience with issues involving children. The development of a national framework will also protect and support the nation's children who will benefit from participating in and contributing towards safe research.

Recommendation 3-3: The National Institutes of Health (NIH) Clinical Center (CC) should continue to prioritize the current level of pediatric research, particularly on topics and using methods that are difficult to pursue in extramural environments (e.g., longitudinal studies of rare diseases and early-stage interventional trials) as well as on the long-term outcomes of interventions. Potential mechanisms include:

- **a. Increasing capacity while ensuring safety by overcoming operational constraints at the CC. Younger and more acute patient populations should be supported in early-phase interventional and observational protocols.**
- **b. Continued, expanded, or new partnerships with children's hospitals, academic medical centers, schools, public health departments, and other large health centers across the country to best support pediatric populations.**
- **c. Offering pediatric education and training opportunities at the CC to continue to develop the specialized workforce needed to support this research.**
- **d. Collecting and presenting data in the annual report to the NIH Pediatric Research Consortium (or its equivalent) on the proportion of pediatric research studies, support, and usage by each institute and center at the CC.**

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A core part of NIH is the Clinical Center. The committee envisions that changes at the Clinical Center to accommodate pediatric research will be accelerated. These will include lowering the age for patients to allow admission of neonates and building the infrastructure to care for critically ill children. While some of the changes can occur in the Clinical Center on the NIH campus, the committee notes that it will be advantageous and efficient for other studies to be done in collaboration with research-intensive children's hospitals, which can conduct the study protocols. The committee recommends that the Clinical Center continue to build up its expertise in all pediatric subspecialties to accomplish its expanded pediatric mission. Importantly, this will include the expansion of subspecialty fellowship programs at the Clinical Center. Additionally, the Clinical Center will collect data on the proportion of pediatric research studies, support, and usage by each institute and center and report these findings back to NPeRC (or its equivalent).

Goal 3: Strengthen the impact of pediatric health research on child and adult health outcomes by optimizing the translation, dissemination, and implementation of research into practice.

Recommendation 5-1: The National Institutes of Health (NIH) should work to create a culture of ethical inclusion of children in research, focused on protecting children *through* research rather than *from* research. To this end, NIH should develop measurable strategies and mechanisms to better implement and enforce existing policies to ensure inclusion of children at the project and proposal level. Strategies and mechanisms should include:

- **a. Updating the simplified review framework by explicitly considering the inclusion of children and its implications for health during the life course as a component of evaluating a proposal (i.e., its significance) during review.**
- **b. Continuing to support the NIH Center for Scientific Review in implementing and monitoring structural changes to support pediatric research:**
  - **Requiring and monitoring that pediatric representation is consistent with the number of applications on all relevant standing study sections.**
  - **Ensuring provision of adequate justification for exclusion of children during grant review: If there is a scientific, legal, or ethical reason for the exclusion of children, this justification should be clearly articulated in the grant application**

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and assessed and documented during the grant review process.

- **Elevating prioritization of implications of research on the life course during scientific review: NIH should identify the impact of pediatric research on the life course as an important component of a proposal's significance section and should do so directly within reviewer guidance. In addition, training for study section reviewers should encourage them to consider whether a proposal adequately addresses the importance of the research for the life course when evaluating the significance or impact of extramural grant applications.**

c. **Improving transparency on inclusion: The Office of the Director of NIH should annually track and report justifications provided for the exclusion of children. This information should be included in the NIH annual report and published on the NIH Pediatric Research Consortium (NPeRC) website, or an NPeRC equivalent website, to track progress on the improved inclusion of children across NIH activities over time.**

d. **Providing clearer institutional guidance about the ethical inclusion of children in research: NPeRC (or NPeRC equivalent), in consultation with the NIH Clinical Center's Department of Bioethics, should develop a widely accessible toolkit and guidance on ethical issues in pediatric research that have the potential to unduly impede research if misunderstood, such as when to include children in research, provisions for assent, adolescent independent consent, age de-escalation, and payment for research participation.**

"Additionally, the smaller sample sizes and longer follow-up periods often required for pediatric studies can be perceived as limitations during peer review (and these longer follow-ups are associated with significant costs). From a process perspective, the current structure of study sections can disadvantage pediatric applications when they are reviewed by panels lacking pediatric expertise. This can lead to lower enthusiasm scores and reduced appreciation for the nuances of conducting rigorous research in neonatal or pediatric populations."

– Neonatal pediatric researcher and recipient of NIH grants

Applications to fund pediatric research are at a marked disadvantage when the review committees do not include experts in pediatric research. Having pediatric representation on these panels will ensure that members of the panel understand the unique needs, methods, and features of pediatric

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research and can advocate to other review committee members on behalf of these pediatric research issues.

> "Pediatric research often involves complex regulatory requirements, including parental consent and assent from minors, which can slow down recruitment and add logistical hurdles not encountered in adult-focused research."
>
> – Neonatal pediatric researcher and recipient of multiple NIH grants

The timeline for the translation of research findings supported by NIH to affect clinical care, practice, and policy is long, and the road to this translation is uneven. One of the barriers to translation in pediatric research arises from the fact that many pediatric diseases are classified as rare (affecting fewer than 200,000 individuals), which reduces the financial incentive for industry to develop therapeutic options for these diseases. This is aggravated by the reluctance to enroll children in clinical trials to test the efficacy and adequacy of these therapies. Another barrier, which impacts implementation, is that more than 50 percent of patients in our children's hospitals are insured only by Medicaid, which often fails to adequately reimburse for the cost of care provided by hospitals and clinicians. This barrier to implementation is compounded by the fact that a majority of U.S. children (53 percent) are of non-White race and ethnicity, which has been the case since 2020. Despite the efforts of health care professionals to provide equal care for all, children who are of minority race and ethnicity,[1] poor, and from families with limited English proficiency continue to experience access barriers to timely, high-quality health care in the United States. Finally, advanced pediatric care is increasingly concentrated in urban centers, creating barriers for the one in five children living in rural communities.

Globally, under-age-5 child mortality has been cut in half in the last 25 years, thanks both to the use of new technologies and the adaptation of effective treatments to low-resource settings. A key to this has been the development of the new field of implementation science, defined by NIH as the "study of methods to promote the adoption and integration of evidence-based practices and interventions into routine health care and public health settings to improve our impact on population health." (NCI, n.d.). For example, at the National Cancer Institute, one goal is to "integrate implementation science into research across the cancer control continuum to improve the relevance to health care systems and population and public

[1] "Minority" as used here refers to the entire U.S. population's demographic statistics, not just that of U.S. children. U.S. Census Bureau; https://www.census.gov/library/visualizations/interactive/race-and-ethnicity-in-the-united-state-2010-and-2020-census.html (accessed October 8, 2025).

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health" (NCI, 2022). If NIH were to apply a similar effort to integrate pediatric research into practice by using implementation science, it could help to lower the aforementioned barriers, leading to significant improvements in the health of all children.

Goal 4: Build trust in pediatric health research through communication about and dissemination of rigorous research findings that improve health trajectories across the lifespan for all.

Recommendation 4-2: The National Institutes of Health (NIH) director should:

c. Have NPeRC's membership (or its equivalent) include expertise in pediatric bioethics. NPeRC should also partner with patient advocacy or community representatives and develop a communications plan to disseminate pediatric research findings to both the scientific community and the general public. NPeRC (or its equivalent) should be prepared to coordinate the NIH-wide response to any emerging pediatric research needs.

As a part of this recommendation, the committee suggests that NPeRC have bioethics representation to address concerns with consent and assent in pediatric research and patient advocacy representation to ensure that patients' needs are understood and addressed. NPeRC's communications staff will provide the expertise to disseminate pediatric research findings to a range of audiences, thereby promoting trust in pediatric health research, its findings, and the clinical guidelines, therapies, and devices that arise from the research.

For children to thrive, families and communities must be able to trust the research that informs their care. Building that trust starts with making sure that the science itself is rigorous and then sharing it in ways that are clear, non-political, and grounded in fact. When parents, pediatricians, teachers, and policy makers hear directly and consistently about research findings that improve children's health (whether about nutrition, asthma, vaccines, or mental health), they are better equipped to support the choices that set children on healthier paths. Trust in experts and physicians is not just a professional issue; it is a cornerstone of a healthy nation. When people believe that the information they are receiving is reliable and free of political bias, they are more likely to act on it in ways that benefit children, families, and the broader society.

The challenge today is that the landscape of health information looks very different than it did even a generation ago. Thirty years ago, parents

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might have gone to the library and pulled a medical reference book from a card catalogue; today, they are more likely to type a question into a search bar and find themselves scrolling through a flood of websites, videos, AI-generated answers, and social media posts. Because many of these sources lack the imprimatur of subject-matter experts, it can be difficult and frustrating to know what information to trust. This means that investigators must both do excellent science and also communicate it in ways that meet families and communities where they are. To build trust in pediatric health research, the NIH has an important role in shaping communication strategies that are fact-based, transparent, and accessible, ensuring that credible knowledge rises above the noise and ultimately improves the health of children across their entire life course.

A CODA

The future of pediatric research will be shaped not only by scientific innovation, but also by the ability of leaders to steward public resources wisely, build powerful cross-sector coalitions, and keep children firmly at the heart of our national health priorities. NIH is uniquely positioned to lead this effort, but leadership in today's climate means a sharpened focus, aligning research to public concerns, and demonstrating and communicating impact from the laboratory to the community. Renewing our commitment to child health research is not simply an expansion or a transformation per se; instead, it is a reorientation towards prevention and a future in which every child can flourish. America's children represent our most profound opportunity for shaping a better and healthier society. The earlier in the lifespan that we invest in child health, the greater the return in long-term health, well-being, and vitality throughout the life course of individuals, through generations of families, and in communities across the United States. Investing in pediatric health through prevention, targeted therapeutics, and cures not only improves lifelong health outcomes but also yields significant societal and economic benefits by reducing the burden of disease and fostering healthier communities and a more productive and vital society. Our national security, U.S. competitiveness, and future generations of Americans depend on our having healthy children.

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Appendix A

Biographical Sketches of Committee Members

Frederick P. Rivara, M.D., M.P.H., (*Co-Chair*) is the holder of the Seattle Children's Guild Association Endowed Chair in Pediatric Research, vice chair and professor of pediatrics and adjunct professor of epidemiology at the University of Washington (UW). He was founding editor-in-chief of *JAMA Network Open* (2018–2024) following 17 years as editor-in-chief of *JAMA Pediatrics*. He served as founding director of the Harborview Injury and Research Center in Seattle and has devoted his career to studying injury and injury prevention. He has contributed to landmark studies and global initiatives aimed at reducing injury-related deaths, especially among children and adolescents. He also served as a founding board member of the Washington State Academy of Science. He has received numerous honors including the Charles C. Shepard Science Award from the Centers for Disease Control and Prevention, the American Public Health Association, Injury Control and Emergency Health Services Section Distinguished Career Award, and the American Academy of Pediatrics, Section on Injury and Poison Prevention, Physician Achievement Award, the UW School of Public Health Distinguished Alumni Award, and the UW Medicine Minority Faculty Mentoring Award, and he delivered the William T Fitts, Jr. Lecture in Trauma in 2009. Rivara was elected to the Institute of Medicine (now National Academy of Medicine) in 2005. He was awarded the Joseph St. Geme, Jr. Leadership award from the Federation of Pediatric Organizations in 2021. Dr Rivara was a member of the Advisory Council for the Eunice Kennedy Shriver National Institute of Child Health and Human Development (2015–2018) and most recently chaired the Committee on Pediatric

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Subspecialty Workforce, National Academies of Science, Engineering and Medicine, May 2022–July 2023.

Phyllis A. Dennery, M.D., FAAP, (*Co-Chair*) is the Sylvia Kay Hassenfeld Professor and chair of pediatrics at the Warren Alpert Medical School; pediatrician-in-chief, Rhode Island Hospital; medical director, Hasbro Children's Hospital; and professor of molecular biology, cell biology, and biochemistry at Brown University. She served as the division chief in neonatology at Children's Hospital of Philadelphia/University of Pennsylvania before being recruited to lead the Department of Pediatrics at Brown in 2015. Dr. Dennery is the recipient of many awards and honors, including election to the American Society for Clinical Investigation, the National Academy of Medicine, and the Association of American Physicians, some of the top honors in Medicine. She is also the recipient of the Mentor of the Year award from the Eastern Society for Pediatric Research. Dr Dennery is an active member of many professional and scientific societies and has led many of these including the International Pediatric Research Foundation, the Society for Pediatric Research, the International Perinatal Collegium and the Society for Redox Biology. Her research is funded by the National Institutes of Health, and she publishes on mechanisms of neonatal hyperoxic lung injury, in particular the role of senescence and senescence-associated secretory factors in altering lung metabolism and architecture in top-tier journals. Her clinical interests focus on bronchopulmonary dysplasia and long-term consequences of prematurity. She is the steering committee chair for several multi-center grants funded by the National Heart, Lung, and Blood Institute (NHLBI) and is the data and safety monitoring board chair for a multi-site study funded by NHLBI. She was a member of the National Academy of Medicine (formerly Institute of Medicine) committee that wrote *The CTSA Program at the NIH: Opportunities for Advancing Clinical and Translational Research*, published in 2013. She also serves on many national boards, including the March of Dimes. Dr Dennery obtained her B.S. in biology from McGill University and her medical degree from Howard University; she completed a residency in pediatrics at Children's Hospital National Medical Center and a fellowship in neonatology at Case Western Reserve University.

Alexander G. Bassuk, M.D., Ph.D., is currently the chair and departmental executive officer of the University of Iowa (UI) Stead Family Department of Pediatrics and the physician-in-chief of UI Stead Family Children's Hospital. Previously, he served in various roles related to pediatric neurology and clinical leadership at the University of Iowa's Roy J. and Lucille A. Carver College of Medicine. Dr. Bassuk's primary expertise lies in pediatric neurology, focusing on childhood neurological disorders, education, and

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the development of future clinician–scientists. He has been instrumental in training medical students, residents, and postdoctoral fellows, and he leads the Child Health Research Career Development Award program as principal investigator. Additionally, he co-directs the Clinical and Translational Science Award K–12 program, aimed at advancing clinical and translational research in pediatric health. Dr. Bassuk is a member of several prominent organizations, including the American Academy of Neurology, the Child Neurology Society, and the American Pediatric Society. He earned his M.D. and Ph.D. from the University of Chicago.

Glenn Flores, M.D., FAAP, is a professor and the chair of pediatrics, senior associate dean of child health, and the George E. Batchelor Endowed Chair in Child Health at the University of Miami Miller School of Medicine. He is also physician-in-chief at Holtz Children's Hospital in the Jackson Health System. Dr. Flores previously served on the U.S. Preventive Services Task Force and held leadership roles in several national committees related to pediatric health policy. Dr. Flores's research and clinical expertise focus on pediatric health equity, addressing racial/ethnic disparities in children's health, and health services research. He has contributed significantly to policy efforts on insuring the uninsured, social determinants of health, and childhood obesity. He has received numerous prestigious awards, including the 2006 American Academy of Pediatrics Outstanding Achievement Award, the 2019 American Public Health Association's David P. Rall Award for Advocacy, and the 2024 Reinhardt Distinguished Career Award from AcademyHealth. He is also a member of several professional societies, including the American Pediatric Society and the Academic Pediatric Association and a member of the Board of First Focus on Children. He earned his M.D. from the University of California San Francisco School of Medicine and has published over 260 research articles.

Christopher Forrest, M.D., Ph.D., is a pediatrician and health services and outcomes researcher. He is the director of PEDSnet (pedsnet.org), a national pediatric learning health system that includes 11 of the nation's premier pediatric health systems. PEDSnet has created a longitudinal electronic health record database for more than 15 million children, and the network conducts observational research and pediatric clinical trials across all pediatric subspecialty areas. Dr. Forrest serves as the director of the Children's Hospital of Philadelphia Applied Clinical Research Center, which is the institutional home for PEDSnet, and the program director for PEDSnet Scholars, a faculty development program funded by the Agency for Healthcare Research and Quality (AHRQ) and the Patient-Centered Outcomes Research Institute (PCORI). His research group has developed numerous pediatric patient-reported outcome measures, both child self-report and

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parent-proxy versions. He co-edited the new *Handbook of Life Course Health Development*, which has been downloaded over 1 million times from the Springer web site. He is a member of the National Academy of Medicine and serves on the board of regents for the National Library of Medicine from September 2023–2027. Dr. Forrest received his B.A. and M.D. degrees from Boston University and his Ph.D. (health policy and management) from Johns Hopkins University. Dr. Forrest previously served as a member of the Committee on the Pediatric Subspecialty Workforce and its Impact on Child Health and Well-Being (2022–2023). Dr. Forrest receives grant support from the National Institutes of Health, AHRQ, PCORI, the Centers for Disease Control and Prevention, the Food and Drug Administration, and industry sponsors (e.g., UCB Pharmaceuticals, Sanofi Aventis, Moderna, and Otsuka).

Vittorio Gallo, Ph.D., is the chief scientific officer and senior vice president at Seattle Children's Hospital, leading the Seattle Children's Research Institute and serving as professor of pediatrics at the University of Washington. He previously held leadership roles at Children's National Hospital, including director of the Center for Neuroscience Research and interim chief academic officer, as well as associate dean for child health research at George Washington University. Dr. Gallo's research focuses on brain development and developmental disabilities, particularly the impact of neonatal brain injuries and neurodevelopmental disorders. He has directed numerous National Institutes of Health (NIH)–funded pediatric research training programs and has mentored many faculty and trainees. His honors include being named an EMBO, Fogarty, and NATO fellow, and he received the prestigious National Institute of Neurological Disorders and Stroke Javits Award for his contributions to neuroscience. He has been an editor for *The Journal of Neuroscience*. He was elected to the American Pediatric Society and has served on several NIH review panels, including chairing two study sections. Dr. Gallo earned his Ph.D. in biochemistry and neurobiology from the University of Rome, with postdoctoral training at the MRC Developmental Neurobiology Unit and the NIH.

Rosemary Higgins, M.D., is the associate vice president for research and sponsored programs at Florida Gulf Coast University, joining in 2022. Previously, she was senior associate dean for research at George Mason University's College of Health and Human Services and spent over 16 years at the National Institutes of Health (NIH) as a medical officer and program officer. At the NIH, she served as the program scientist for the NICHD Neonatal Research Network, overseeing landmark studies that advanced care for premature and sick infants. Before her NIH tenure, Dr. Higgins had over 12 years of academic medicine experience at New

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York University and Georgetown University, where she led a basic science lab investigating retinopathy of prematurity using cell culture and animal models. She served as the neonatal–perinatal medicine fellowship program director at Georgetown. Dr. Higgins also served as the neonatology editor in chief of the *American Journal of Perinatology* and as a member of the Pediatric Eye Disease Investigator Group (PEDIG), Data and Safety Monitoring Committee of the National Eye Institute at NIH. Her expertise spans neonatal–perinatal medicine, prematurity, substance use, and public health, with a strong background in clinical trials and longitudinal studies. Dr. Higgins has received multiple NIH director awards, awards of merit, and teaching honors. Her current work focuses on public health issues across the lifespan. She received her M.D. from Georgetown University School of Medicine, completed her pediatrics training at the University of Virginia and Neonatal–Perinatal Medicine fellowship at the University of Rochester.

Pamela Hinds, Ph.D., M.S.N., is the director of the Department of Nursing Science, Professional Practice, and Quality; the William and Joanne Conway Endowed Chair in Nursing Research; the research integrity officer at Children's National Health System; and a professor of pediatrics at the George Washington University School of Medicine and Health Sciences. Previously, she served as the director of nursing research at St. Jude Children's Research Hospital. Dr. Hinds' research focus has been on the pediatric cancer experience, patient treatment tolerability, and patient reported outcomes. Dr. Hinds is an Oncology Nursing Society Distinguished Nurse Researcher and the Association of Pediatric Oncology and Hematology Distinguished Nurse Researcher, the 2020 recipient of the HPNA Distinguished Nurse Researcher Award in Palliative Care, the 2020 Nightingale Award from the American Nurses Association and the *Washington Post*, and the 2024 recipient of the Larry M. Rosen Research Award. Dr. Hinds received her Ph.D. and M.S.N. from the University of Arizona. She served on the Institute of Medicine (IOM) committee on End-of-Life and Palliative Care for Children in America, the IOM committee on Dying in America, the National Academies of Sciences, Engineering and Medicine Roundtable on Quality Care for People with Serious Illness, and the National Cancer Institute Moonshot Tolerability Steering Committee.

Shafali Spurling Jeste, M.D., is a behavioral child neurologist specializing in autism and related neurodevelopmental disorders (NDDs). She is a professor and the Mattel Executive Endowed Chair in the Department of Pediatrics at the David Geffen School of Medicine at the University of California, Los Angeles (UCLA). Dr. Jeste is also the executive medical director of pediatrics in the UCLA health system and the executive director of the UCLA Children's Discovery and Innovation Center. She was a professor of pediatrics

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and neurology at the University of Southern California's Keck School of Medicine; the Las Madrinas Chair, chief of neurology, and the co-director of the Neurological Institute at Children's Hospital Los Angeles. Her research focuses on improving precision in the diagnosis and treatment of NDDs through studies on early detection, early intervention, and clinical trial readiness, particularly in genetic NDDs, with focus on electrophysiological biomarkers and clinical endpoints. Her work is funded by the National Institute of Health, the Department of Defense, the Dup15q Alliance, and the Simons Foundation. She holds leadership positions on the boards of directors for the National Organization for Rare Disorders and the TSC Alliance, and she serves on the steering committee for the ARIA Initiative to accelerate autism research. She sits on a data safety and monitoring board for Ionis Pharmaceuticals' Angelman Syndrome trial. Dr. Jeste consulted for Roche Pharmaceuticals on clinical trial design for a genetic neurodevelopmental disorder called Dup15q syndrome. Dr. Jeste was awarded the Presidential Early Career Award for Scientists and Engineers in 2019 and the 2024 Child Neurology Society Martha Bridge Denckla Award. She earned a B.A. in philosophy from Yale University in 1997 and an M.D. from Harvard Medical School in 2002, followed by residency and fellowship training at Boston Children's Hospital. Dr. Jeste previously served as a member of the Committee on the Pediatric Subspecialty Workforce and its Impact on Child Health and Well-Being (2022–2023).

Leah Kottyan, Ph.D., is a professor at Cincinnati Children's Hospital Medical Center and the University of Cincinnati, where she leads a National Institutes of Health–funded research laboratory focused on the genetic etiology of immune system diseases. Dr. Kottyan also serves as the leader of the Cincinnati Children's Basic Science Research Committee, which represents hundreds of faculty members conducting foundational research to discover new biological insights. Her research includes the discovery of genotype-dependent disease risk mechanisms and studies on the clinical return of individualized polygenic disease risk.

Brendan Lee, M.D., Ph.D., is a pediatrician and geneticist who specializes in structural birth defects and inborn errors of metabolism. He has published over 320 peer-reviewed papers and more than 85 invited reviews, chapters, and books. Dr. Lee has secured continuous National Institutes of Health (NIH) funding over his 25-year career as an independent investigator. He currently leads several NIH-funded initiatives, including the BCM Undiagnosed Diseases Network Clinical Site, the RE-JOIN Consortium site, the Brittle Bone Disorders Consortium, and the All of Us Evenings with Genetics Scholars Program. Dr. Lee has served on numerous nonprofit boards and advisory panels, including the Advisory Committee to the Director

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of the NIH. He was previously an investigator at the Howard Hughes Medical Institute and is currently the chair of the Department of Molecular and Human Genetics at Baylor College of Medicine, a leading global program integrating research, clinical services, and molecular pathology. His work extends into drug discovery and gene therapy, with multiple patents and licensed technologies currently in industry-sponsored clinical trials for osteoarthritis, osteogenesis imperfecta, and maple syrup urine disease, and he receives royalties from companies such as Pacira and Sanofi for gene therapies. He also earns royalties through licensing of discoveries from Baylor College of Medicine for Osteogenesis imperfecta clinical trials. Additionally, Dr. Lee sits on data safety and monitoring boards and consults for pharmaceutical companies such as Kyowa Kirin. Dr. Lee has also served as the president of the American Society for Human Genetics and the Society for Pediatric Research.

Mary B. Leonard, M.D., MSCE, is the Arline and Pete Harman Professor and chair of the Department of Pediatrics at Stanford University, physician-in-chief of Lucile Packard Children's Hospital Stanford, and director of the Stanford Maternal and Child Health Research Institute. She has led a distinguished career in clinical and translational research, with a primary focus on the impact of chronic pediatric diseases on growth, skeletal development, nutrition, and physical function. Dr. Leonard has served as principal investigator for numerous National Institutes of Health grants, including multiple R01s, and has mentored junior investigators across various pediatric specialties. She has authored over 220 peer-reviewed research papers and has been recognized with numerous awards, including the 2024 Maureen Andrew Mentor Award from the Society of Pediatric Research and the National Kidney Foundation's Donald W. Seldin Award. Dr. Leonard received her M.D. from Stanford University in 1989 and completed her pediatrics residency and nephrology fellowship at the Children's Hospital of Philadelphia. She also holds a master's degree in clinical epidemiology from the University of Pennsylvania.

Keila N. Lopez, M.D., M.P.H., is an associate professor of pediatrics and the director for faculty development in the Heart Institute at Colorado Children's Hospital. Dr. Lopez is also a Child Health Policy Fellow at the Farley Center for Health Policy. She was formerly an associate professor of pediatrics in the Section of Pediatric Cardiology at Texas Children's Hospital and Baylor College of Medicine, where she also served as the creator and medical director of the first in-person pediatric cardiology transition program, designed to improve the transfer of care from pediatric to adult services. Additionally, Dr. Lopez remains an adjunct assistant professor in sociology at Rice University and a co-investigator for the Texas Children's Policy

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and Advocacy Center. Dr. Lopez's research focuses on health disparities, public health initiatives, and health care access, using large databases and geographic information system mapping to explore how sociodemographic factors influence health outcomes. Her National Institutes of Health (NIH)–funded work has included developing a mobile application to reduce health disparities during the transition process for patients with congenital heart disease, and her more recent NIH R01 grant explores novel care models for underserved young adults with congenital heart disease. She has received several accolades, including the 2024 American Academy of Pediatrics DEI Excellence Award and the 2024 Texas Children's Research Mentor Award. Currently, Dr. Lopez is the immediate past chair of the American Heart Association's Young Hearts Congenital Cardiac Defects Committee and serves on the medical advisory board of the Children's Heart Foundation and Make-A-Wish Foundation. She earned her medical degree from Rush Medical College and completed her pediatric residency at the University of Chicago, completed her pediatric cardiology training at Texas Children's Hospital, and obtained her master of public health in minority health policy at Harvard T.H. Chan School of Public Health.

Kristy Murray, D.V.M., Ph.D., is the chief research officer at Children's Healthcare of Atlanta and professor and executive vice chair for research in the Department of Pediatrics at Emory University. Prior to her current role, she served as vice chair for research and professor of pediatrics at Baylor College of Medicine and Texas Children's Hospital, where she also held leadership positions at the National School of Tropical Medicine. Dr. Murray is an expert in the epidemiology and pathogenesis of emerging infectious diseases, with a focus on pediatric populations. Her National Institutes of Health– and Centers for Disease Control and Prevention–supported research has addressed diseases like West Nile virus, dengue, Zika virus, and COVID-19, with field studies conducted in global health settings in Nicaragua, Belize, and the Philippines. She has received numerous awards, including the Bailey Ashford Medal from the American Society of Tropical Medicine and Hygiene and the Texas A&M College of Veterinary Medicine's Distinguished Alumni Award. Dr. Murray earned her D.V.M. from Texas A&M University and her Ph.D. in preventive medicine from the University of Texas Medical Branch. She has authored over 170 scientific papers and serves as an associate editor for *Emerging Infectious Diseases* and *PLOS Neglected Tropical Diseases*.

Abby Rosenberg, M.D., M.S., M.A., is the chief of pediatric palliative care at the Dana-Farber Cancer Institute, director of palliative care at Boston Children's Hospital, and an associate professor of pediatrics at Harvard Medical School. She holds advanced degrees in epidemiology and

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biomedical ethics in addition to clinical training in pediatric palliative care and pediatric hematology–oncology. Dr. Rosenberg's research focuses on developing and implementing programs to enhance resilience and improve the mental health and quality of life for children with serious illnesses and their families. She has been continuously funded by the National Institutes of Health (NIH) throughout her career and has published over 250 peer-reviewed manuscripts on pediatric and adolescent/young adult psychosocial and supportive care. She is the chair for the Lifestyle and Health Behaviors Standing Study Section at NIH and reviews population-level research focused on health and disease outcomes in this role. Dr. Rosenberg has received numerous honors, including the 2021 University of Washington Faculty Mentoring Award, the 2022 American Cancer Society Lifetime Achievement Award, the 2023 Pathfinder in Palliative Care Award, and the 2024 Palliative Care Visionary Award from the American Academy of Hospice and Palliative Medicine. Dr. Rosenberg began her professional career as a social worker for children with HIV. Dr. Rosenberg earned her medical degree from Stanford University School of Medicine and completed her two master's degrees at the University of Washington. She completed her pediatric residency and fellowship training at Seattle Children's Hospital. In 2024, Dr. Rosenberg was elected as a volunteer pediatrician to the American Society of Clinical Oncology board of directors (2024–2027), an organization that advocates for cancer-related funding and programs worldwide.

David C. Schwebel, Ph.D., M.A., is the vice president of research at the University of Iowa. Dr. Schwebel was formerly university professor of psychology and associate vice president for research facilities and infrastructure at the University of Alabama at Birmingham (UAB), where he led the UAB Youth Safety Lab. He has extensive expertise in child safety, with a particular focus on understanding and preventing unintentional injuries in children. Dr. Schwebel has authored over 400 peer-reviewed manuscripts and secured more than $12 million in research grants, primarily from the National Institutes of Health. Dr. Schwebel served on a strategic planning working group for the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) in 2018, which led to the current NICHD strategic plan that was released in 2020. He has received significant recognition for his work, including the 2019 Award for Distinguished Professional Contributions to Applied Research from the American Psychological Association. Dr. Schwebel is also a Woodrow Wilson Scholar, a Fulbright Award recipient, and a fellow of the American Psychological Association. He was a consultant for Proctor & Gamble on topics related to child safety and injury. In the past 5 years he has also done paid consulting work on cases related to child safety and injury for Slocumb Law Firm, LLC; Cohen, Placitella & Roth; Gray, Rust, St. Amand, Moffett & Brieske,

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LLP; Allison & Ward Attorneys at Law; and Arthur Chapman Kettering Smetak & Pikala, P.A. Dr. Schwebel also served on the board of scientific advisors for the National Center for Injury Prevention and Control of the Centers for Disease Control and Prevention. He earned his B.A. in psychology from Yale University and his M.A. and Ph.D. in clinical psychology from the University of Iowa, followed by a clinical internship at the University of Washington School of Medicine. In addition to his research, he published the popular book *Raising Kids Who Choose Safety* in 2022.

Seema K. Shah, J.D., HEC-C, is a professor of pediatrics at Northwestern University Medical School and the Founder's Board Professor of Medical Ethics at Lurie Children's Hospital. She is also the director of research ethics and leads the Pediatric Research Ethics and Policy Program at Lurie Children's Hospital. Dr. Shah's research focuses on pediatric and global health research ethics, with particular attention to ethical and regulatory issues in pediatric pandemic preparedness and controlled human infection studies. Dr. Shah has served as chair of an NIH panel on the ethical considerations of Zika virus human challenge trials, currently sits on the National Advisory Allergy and Infectious Disease Council for the National Institute of Allergy and Infectious Diseases (NIAID) at NIH and serves as an expert advisor for the World Health Organization. She is an elected fellow of the Hastings Center and a recognized leader in the field of bioethics. Shah earned her law degree from Stanford Law School.

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Appendix B

Public Meeting Agendas

COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Thursday, December 5, 2024 (1:30–3 p.m. ET)
Session 1 - Open

1:30–1:40 p.m.	**Welcome and Introduction** **Frederick Rivara,** *Committee Co-Chair* **Phyllis Dennery,** *Committee Co-Chair*
1:40–2:05 p.m.	**Charge to the Committee: Perspective of the Study's Sponsor Representatives** **Rohan Hazra,** *Director, Division of Extramural Research, NICHD* **Tracy King,** *Associate Director for Prevention and Medical Officer, Child Development and Behavior Branch, NICHD* **Sarah Glavin,** *Deputy Director Science Policy Analysis and Communication; Chief, Science Policy and Evaluation, NICHD, Co-chair of NIH's RCDC Governance Board*

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2:05–2:30 p.m.	**Committee Q&A Session with Sponsor Representatives**
2:30–2:45 p.m.	**Coalition for Pediatric Medical Research (CPMR) Speakers** **Tina Cheng,** *Chief Medical Officer, Cincinnati Children's* **Dennis Durbin,** *President, Abigail Wexner Research Institute at Nationwide Children's* **Sallie Permar,** *Chair, Department of Pediatrics at Weill Cornell Medicine* **Carl Allen,** *Chair in Pediatric Oncology, Texas Children's Hospital*
2:45–3:00 p.m.	**Committee Q&A Session with Coalition Representatives**
3:00 p.m.	**Adjourn Open Session**

COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Thursday, March 13, 2025 (9:00-11:00 a.m. ET)
Public Session Part 1 Day 1 - Open

9:00–9:10 a.m.	**Welcome and Introduction** **Frederick Rivara,** *Committee Co-Chair* **Phyllis Dennery,** *Committee Co-Chair*
9:10 a.m.	**Brief Introduction to the Session** **Mary Leonard,** *Committee Member*
9:10–10:10 a.m.	**Perspectives from Larger NIH Initiatives** **Gaya Dowling,** *ABCD Project* **Matthew Gilman,** *Environmental Influences on Child Health Outcomes Program* **Sara van Driest,** *All of Us Research Program* **Walter Koroshetz,** *BRAIN Initiative* **Tiina K. Urv,** *Rare Diseases Clinical Disease Network* **David Goff** and **Gail Pearson,** *RECOVER*

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10:10–11:00 a.m. **Committee Q&A with Panelists**

11:00 a.m. **Adjourn**

COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Friday, March 14, 2025 (9:00-11:00 a.m. ET)
Public Session Part 1 Day 2 - Open

9:00–9:10 a.m. **Welcome and Introduction**
Frederick Rivara, *Committee Co-Chair*
Phyllis Dennery, *Committee Co-Chair*

9:10 a.m. **Brief Introduction to the Session**
Brendan Lee, *Committee Member*

9:10–9:25 a.m. **NIH Center for Scientific Review**
Valerie Durrant, *NIH Center for Scientific Review*

9:25–9:45 a.m. **Committee Q&A**

9:45 a.m. **Brief Introduction to the Session**
Rosemary Higgins, *Committee Member*

9:45–10:10 a.m. **Perspectives from NICHD**
Diana Bianchi, *Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)*
Rohan Hazra, *Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)*

10:10–10:30 a.m. **Committee Q&A**

10:30 a.m. **Brief Introduction to the Session**
Pamela Hinds, *Committee Member*

10:30–10:45 a.m. **NIH Clinical Center**
Colleen Hadigan, *NIH Clinical Center*

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

10:45–11:00 a.m. **Committee Q&A**

11:00 a.m. **Adjourn**

COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Thursday, March 27, 2025 (9:30-10:35 a.m. ET)
Public Session Part 2 Day 1 - Open

9:30–9:35 a.m. **Welcome and Introduction**
Frederick Rivara, *Committee Co-Chair*
Phyllis Dennery, *Committee Co-Chair*

9:35 a.m. **Brief Introduction to the Session**
Glenn Flores, *Committee Member*

9:35–10:05 a.m. **Disease Categorization and Analysis at NIH**
Mara Olenick, *Division of Scientific Categorization and Analysis, Office of Research Reporting and Analysis, Office of Extramural Research, NIH*
Sarah Glavin, *Science Policy, Planning, and Evaluation Branch, Office of Science Policy, Reporting, and Program Analysis, NICHD*

10:05–10:35 a.m. **Committee Q&A**
Moderator, Glenn Flores

10:35 a.m. **Adjourn**

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COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Friday, March 28, 2025 (9:00-11:00 a.m. ET)
Public Session Part 2 Day 2 - Open

9:00–9:10 a.m.	**Welcome and Introduction** **Frederick Rivara,** *Committee Co-Chair* **Phyllis Dennery,** *Committee Co-Chair*
9:10–10:15 a.m.	**Perspectives from NIH Institutes, Centers, and Offices on Pediatric Research** **Michael Chiang,** *National Eye Institute (NEI)* **Gaya Dowling,** *National Institute on Drug Abuse (NIDA)* **John Glod and Brigitte Widemann,** *National Cancer Institute (NCI)* **Kimberly Gray,** *National Institute of Environmental Health Sciences (NIEHS)* **Amelia Karraker,** *National Institute on Aging (NIA)* **Kelly King,** *National Institute on Deafness and Other Communication Disorders (NIDCD)* **George Koob,** *National Institute on Alcohol Abuse and Alcoholism (NIAAA)* **Jeanne Marrazzo,** *National Institute of Allergy and Infectious Diseases (NIAID)* **Susan Mendley,** *National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)* **Eliseo Pérez-Stable,** *National Institute on Minority Health and Health Disparities (NIMHD)* **Ann Poduri,** *National Institute of Neurological Disorders and Stroke (NINDS)* **Jennifer Webster-Cyriaque,** *National Institute of Dental and Craniofacial Research (NIDCR)*
10:15–11:00 a.m.	**Committee Q&A**
11:00 a.m.	**Adjourn**

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

COMMITTEE ON STRATEGIES TO ENHANCE PEDIATRIC HEALTH RESEARCH FUNDED BY NIH

Virtual Public Session

Monday, April 21, 2025 (3:00-4:00 p.m. ET)
Public Session Part 3 - Open

3:00–3:05 p.m.	**Welcome and Introduction** **Frederick Rivara,** *Committee Co-Chair*
3:05–3:50 p.m.	**Disease Categorization and Analysis at NIH** **Sarah Glavin,** *Science Policy, Planning, and Evaluation Branch, Office of Science Policy, Reporting, and Program Analysis, NICHD*
3:50–4:00 p.m.	**Committee Q&A** **Moderator: Christopher Forrest**
4:00 p.m.	**Adjourn** **Phyllis Dennery,** *Committee Co-Chair*

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Appendix C

Calls for Perspectives Questions

CALL FOR PERSPECTIVES: QUESTIONS FOR RESEARCH FUNDING APPLICANTS

The committee developed a list of questions for research funding applicants designed to offer some illustrative examples and perspectives on the funding application process. The questions were posted in April 2025 and listed until July 25, 2025, and distributed widely via National Academies listservs and social media and other email distribution lists relevant to pediatric health researchers.

1. Please describe your relationship and experience with NIH in terms of the research you conduct, including the type of grants you have applied for and/or received, from which institute(s) or center(s), and how long you have been receiving them. Please avoid sharing details that might identify you.
2. If you have received funding that was from multiple ICs or part of a trans-NIH initiative, please describe in further detail. Additionally, if you have worked with the NIH Pediatric Research Consortium (NPeRC), please describe how it supported pediatric research or how it may yet be used to do so. Please avoid sharing details that may identify you.
3. What do you consider to be the most significant structural or process impediments to securing NIH funding for pediatric research? How could the NIH streamline the grant application and review

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process to better support pediatric researchers? Please avoid sharing details that might identify you.

4. Are there unique challenges faced by early-career investigators specializing in pediatric research that you have personally faced or witnessed? Please avoid sharing details that might identify you.
5. What administrative and review processes could NIH change to facilitate extramural research, while maintaining accountability and oversight? Are there existing processes that have been successful that could be replicated elsewhere? What can NIH do to respond quickly with necessary funding to emerging pediatric issues (for example, Zika virus and microcephaly)?
6. Are there NIH funding mechanisms or RFAs that should be adjusted or created to better support pediatric research?
7. Are there particular basic or applied research areas, for which pediatric research has been or is being relatively overlooked and underfunded? Are there basic or applied research areas for which pediatric research has been overly concentrated? If so, please comment on the implications.
8. What do you believe are the successful ways NIH has engaged with the pediatric research community to establish research priorities? What have been unsuccessful ways?
9. Please comment on any challenges or opportunities related to conducting research on disorders or risk factors that originate in childhood but involve participants who are outside the pediatric range.
10. Please share any additional information relevant to the statement of task that you would like the committee to consider. Please avoid sharing details that might identify you.

CALL FOR PERSPECTIVES: QUESTIONS FOR RESEARCH PARTICIPANTS AND THEIR FAMILIES

The committee also developed a list of questions for individuals and families of individuals who had participated in NIH-funded pediatric research which were designed to offer some illustrative examples and perspectives on the funding application process. The questions were posted in May 2025 and listed until July 25, 2025, and distributed widely via National Academies listservs and social media and other email distribution lists relevant those who may have participated in such research.

1. Please describe how NIH funding has been important in supporting pediatric research?
2. What has been your experience with NIH-funded pediatric research?

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3. Please provide personal examples of how NIH research funding has improved child health care, child health, and child well-being.
4. What benefits do you see in participating in NIH-funded pediatric research?
5. What concerns, if any, do you have about NIH-funded research directly involving children?
6. If you have any additional thoughts, stories, or ideas you would like to share about the impact of pediatric research, please feel free to do so below.

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Appendix D

History of Pediatric Health; NIH Structure and Organization

EARLY HISTORY OF PEDIATRIC HEALTH AND RESEARCH

To survive as a species, humans require healthy children who will grow to procreate and sustain the species as well as to produce food, create shelter, and defend their communities. The history of humans caring about pediatric health, therefore, dates arguably to pre-history.

Early History: 1700–1911

Children in the colonial United States faced a multitude of health threats. Prominent among them was smallpox, responsible for dreaded and devastating outbreaks in 1721, 1752, 1764, and 1775. The 1775 epidemic killed almost 8 percent of citizens living in Boston at the time, and survivors faced immense suffering and permanent scarring (Hasselgren, 2020). Inoculation and variolation were commonly practiced worldwide through the 18th century, including among George Washington's Revolutionary War troops (and his British foes), but Edward Jenner's experimentation with smallpox vaccination starting in 1796 triggered a transformation of children's health both in Britain and subsequently in the United States, as vaccination slowly became widespread. Although Jenner's approach to research likely would not meet research ethics standards today, the disease was eradicated in the United States by the middle of the 20th century and worldwide by 1980.

The history of organized pediatric health research in the United States began most clearly, however, with Dr. Abraham Jacobi, whom many medical

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historians credit for creating the medical specialty of pediatrics in the late 19th century. Jacobi's notion was revolutionary at the time, as previously medical specialists had focused solely on particular organ systems, with child health considered as part of obstetrics. In 1880, Dr. Jacobi helped found and served as the first president of a new section of the American Medical Association focused on diseases among children and then did the same when he founded the American Pediatric Society in 1888.

Concurrent with Jacobi's efforts, the first children's hospitals were formed in the United States. Modeled after comparatively recent institutions in Europe, Children's Hospital of Philadelphia opened in 1855, and others followed in Boston, Washington, New York, and several other cities over the subsequent two decades.

The development of professional pediatric societies and children's hospitals occurred simultaneously with key scientific discoveries that impacted the care of children's illnesses and injuries. In 1854, John Snow recognized that a cholera outbreak in London was caused by contaminated water at public water sources, which led to the removal of water pump handles, ending the outbreak, and ultimately, over time, triggering the creation of clean public water supplies. Over the 1850s, 1860s, and 1870s, Louis Pasteur and others published groundbreaking work demonstrating the germ theory of disease, offering the first scientific evidence that some diseases were caused by microscopic pathogens. This discovery led to disease prevention strategies including clean food and water supplies, safe sewage disposal, and the development and dissemination of vaccines.

Early 20th Century: 1912–1945

Following dramatic medical advances in the late 1800s, the U.S. government took action to address children's health in the country through the creation of the Children's Bureau in 1912, the first government agency devoted to children's health and development (Children's Bureau, 2024). The small agency—in its first year it funded a staff of just 15—was charged with a broad agenda but focused especially in its early years on pediatric health topics such as infant mortality, orphaned children, juvenile delinquency, and child labor. It was initially led by Julia Lathrop, a Hull House colleague of Jane Addams and the first woman appointed to lead a U.S. government bureau.

As the world faced economic depression following the 1929 stock market crash, a landmark conference was held in the United States, the 1930 White House Conference on Child Health and Protection. That conference led to the release of the Children's Charter, a proclamation for the rights of all children in the United States that had long-term effects to promote children's health through the depression and into World War II. Additional

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influential White House conferences on children were held decennially in 1940 and 1950.

NIH STAFF ROLES

National Institutes of Health (NIH) staff are civil servants who play a major role in carrying out the mission of NIH to enhance health, lengthen life, and reduce illness and disability (NIH, 2025). The staff roles are integral to grants management (NIH, 2024). NIH staff are tasked with reviewing, prioritizing, funding and monitoring grant and contract activities, and closing out awards. In addition, they perform other roles associated with their positions, including administrative tasks, conferences, planning, and other functions. NIH staff with expertise in pediatrics and pediatric–related disciplines are ideal to oversee pediatric research projects and programs.

Program officers (POs) are responsible for scientific guidance based on their area of expertise as well as for management of grants, the development of research initiatives including notices of funding opportunities, input for funding decisions, and interaction with the extramural community. POs are the point of contact for applicants and grantees. They can advise applicants whether their proposed topic or research idea is suitable as well as on programmatic and scientific matters. POs are permitted to attend peer review study section meetings as observers but have no formal role in the peer review of applications. They can advise applicants about funding status and potential resubmission of an application following receipt of their summary statement or critique of their application.

Grant management specialists and officers (GMSs and GMOs) are responsible for business management, grant administration, and providing budgetary technical assistance to applicants and recipients. The GMO is the NIH official responsible for business management and other non-programmatic aspects of the award. There are many required grants administration policies, and the GMO is responsible for overall compliance in collaboration with the GMS and PO.

Scientific review officers (SROs) manage the study section or peer review for scientific and technical merit of applications. SROs actively match reviewers to the science of the applications being submitted, so expertise in any given area may vary from cycle to cycle depending on what applications are received. Study sections are composed of experts outside of NIH who review the technical and scientific merits of proposals. They are vetted for real and apparent conflicts of interest to assure the integrity of the review process. The SROs play a key role in ensuring that adequate pediatric expertise is available to review applications for pediatric research.

SROs are responsible for selecting scientists and experts to evaluate the applications. They are responsible for managing conflicts of interest. SROs

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facilitate the meetings and are responsible for ensuring fairness and impartiality as part of the review process. They prepare summary statements which reflect the evaluations of the applications that are sent to the applicants.

Visibility of NIH Staff, Especially Program Officers

The importance of visibility of NIH staff, particularly program officers, should be acknowledged. Program officers should be public facing to encourage dialogue between applicants and the NIH. This can be accomplished by attendance at appropriate disciplinary scientific meetings, via outreach, and through opportunities to connect with program officers on a local, state, national or internal level. NIH-conducted conferences or workshops are also a valuable way to connect with program officers and NIH staff. One former program officer authored a paper reflecting on his NIH career, in which he succinctly described the role of the program officer as well as offering suggestions to novices on how to have NIH grant success (Raju, 2019).

NIH CLINICAL CENTER

The NIH Clinical Center (CC) is composed of two major facilities: the Warren Grant Magnuson Clinical Center, opened in 1953, and the Mark O. Hatfield Clinical Research Center, added to the original facility in 2004 (NIH Office of Extramural Research, 2010). A unique feature of the Magnuson Building when it first opened was the co-location of patient care rooms and laboratories where clinical scientists conducted their research to confirm diagnoses, characterize disease processes, and explore effective treatments (NIH Clinical Center, n.d.-b). Conceived originally as a facility focused almost entirely on inpatients, the CC's outpatient infrastructure was added in the 1980s (NIH Clinical Center, n.d.-c).

Background on the NIH Clinical Center

> "[Pediatrics] has really been in the building as long as the building has been there. . . . [W]e have a 71-year history in the clinical center, and there has been the presence of pediatrics as part of the research portfolio, including early groundbreaking work in leukemia, inborn errors in metabolism, [and] even cardiac surgery."
>
> – Dr. Colleen Hadigan, NIH Clinical Center, presented testimony to the committee at the March 14 open session

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In 1944, the Public Health Service Act[1] authorized the establishment of the NIH CC to provide a "model environment for clinical research, patient care and training future generations of clinical scientists" (NIH Clinical Center, n.d.-a). The CC is the largest hospital devoted entirely to clinical research in the world and has historically been involved in groundbreaking research across multiple disease areas, including cancer, behavioral health, and infectious diseases (Gallin, 2011; NIH Almanac, 2024). CC successes include "pioneering the cure of cancerous solid tumors with chemotherapy; the use of nitroglycerin to treat heart attacks; identifying a genetic component in schizophrenia; conducting the first successful replacement of a mitral valve to treat heart disease; and creating blood tests to identify both acquired immune deficiency syndrome (AIDS) and hepatitis" (NIH Clinical Center, 2024). Many practices that began on the basis of NIH research at the CC have become the standard of care worldwide (Gandhi, 2016). The CC is also considered to be a premier institution for training the next generation of clinician scientists and researchers (NIH Office of Extramural Research, 2010; NIH Scientific Management Review Board, 2014).

The CC is designed to support the broad missions of intramural research programs throughout the NIH. Initially, "each of the several NIH Institutes was assigned a proportional fraction of the new beds, with the National Cancer Institute and what became the National Heart Lung and Blood Institute holding the lion's share" (Nathan and Nathan, 2016, p. 2389). In 2025, ICs continue to drive the scientific agenda and clinical protocol development at the CC, with the CC managing resources and infrastructure (e.g., staff, facilities, and systems) for safe and high-quality patient care in support of clinical research studies and responding to usage requests from ICs (NIH, 2021).[2] As the CC is a research facility, only patients meeting the specific requirements of approved research protocols are admitted, but, unlike other hospitals, they do not have to pay fees for treatment and care. Most patients are referred by their clinicians, but approximately one-third self-refer via the Internet (NIH, 2021).

[1] Public Law 78-410. *Public Health Services Act*, 78th Cong. (July 1, 1944).

[2] "Doing research in the NIH IRP differs from doing research in academia in that IRP scientists are not obligated to write and submit grant applications (indeed, they cannot serve as principal investigators of or derive salary support from federal grants); their salary and laboratory or research clinic support are provided by the intramural budgets of their respective NIH institutes. Instead of being funded by individual grants awarded for individual projects, their whole research and training program is evaluated approximately every four years by their institute's Board of Scientific Counselors (BSC) and outside scientists appointed by the director of their respective institute. Generally based on the recommendations of the BSC, the Scientific and/or Clinical Director determines the level of funding and staffing they will receive over the ensuing four years for their entire research program" (Schor, 2023, p. 41).

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Relevant Historical Changes to the Clinical Center

Since the CC opened in 1953, it has undergone many changes that affected both its operations overall and pediatric research performed at the CC specifically. A summary of selected historical changes to the CC over the past few decades can be found in Box D-1.

BOX D-1
Selected Recent Historical Events at the Clinical Center

May 1994 — A multi-institute unit designed and staffed for children opens.

November 1996 — A board of governors is appointed by the Secretary of the Department of Health and Human Services, marking a new governing system for the Clinical Center.

2004 — As recommended by the NIH director's *Blue-Ribbon Panel on the Future of Intramural Clinical Research*, the former Clinical Center board of governors assumes a new and larger identity, becoming the NIH Advisory Board for Clinical Research. The board oversees all intramural clinical research, while continuing its oversight of Clinical Center resources, planning, and operations.

February 2012 — The Clinical Center established a memorandum of understanding allowing NIH intramural clinical studies of children under the age of 2 in the Clinical and Translational Science Award clinical unit at Children's National Medical Center in Washington, DC.

January 2017 — Dr. James K. Gilman serves as the first chief executive officer of the NIH Clinical Center.

2022 — The NIH Clinical Center expands pediatric services, looking at early interventions that may cure diseases and prevent negative outcomes that can occur in some rare diseases. The hospital's Pediatric Consult Service became the Department of Pediatrics, and one of the first initiatives was to establish the Pediatric Hospital Medicine (PHM) service.

2019 – The Clinical Center Strategic Plan (2019), "People, Places, Capabilities: The NIH Clinical Center at 65," was released.

2023 – The Pediatric Research Strategic Plan (PRSP) was released in October 2023, established to shape the future of pediatric research at CC and identify scientific opportunities with the greatest potential for impact at the CC.

SOURCE: Data from NIH Almanac (2024); NIH Clinical Center (2023, n.d.-c).

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Appendix E

PI Department Classifications

TABLE E-1 Principal Investigator Department Classifications

Department Type	Organization Departments Included
Basic Science	Anatomy/Cell Biology, Biochemistry, Biology, Biomedical Engineering, Biophysics, Chemistry, Engineering (all types), Microbiology/Immunology/Virology, Neurosciences, Other Basic Sciences, Physics, Physiology, Social Sciences, Veterinary Sciences, Zoology
Non-Pediatric Clinical	Anesthesiology, Biostatistics and Other Mathematical Sciences, Dentistry, Dermatology, Emergency Medicine, Family Medicine, Genetics, Internal Medicine/Medicine, Neurology, Neurosurgery, Nutrition, Obstetrics and Gynecology, Ophthalmology, Orthopedics, Other Clinical Science, Non-Pediatrics, Other Health Professions, Otolaryngology, Pathology, Pharmacology, Physical Medicine and Rehabilitation, Plastic Surgery, Psychiatry, Psychology, Public Health and Preventive Medicine, Radiation–Diagnostic/Oncology, Surgery, Urology
Pediatrics	Pediatrics
None (Excluded)	(BLANK), Administration, Miscellaneous, No Code Assigned, None

NOTE: This table refers to contact PIs and does not include the multiple appointments that some researchers may have. Additionally, children's hospitals do not have department information that is reported to NIH, and not all institutions report their departments.

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Appendix F

NPeRC Agenda Items

NPeRC Agenda Items, 2020-2024

NOTES: If a topic was discussed multiple times in the same year, it is only listed once. Many discussions were confidential in nature (e.g., potential funding opportunities); in these cases, a discussion of the topic is noted but specifics are omitted.

2024 Meetings

NASEM study: The Pediatric Subspecialty Workforce and Its Impact on Child Health and Well-Being
Opportunities to Leverage PCORnet® Data Resources to Support Patient-Centered CER in Pediatrics
NICHD Training and Career Development Working Group Recommendations
Using National Institutional Training Programs to Grow Research in a Clinical Specialty
External Feedback on Pediatric Research Training (e.g., report out on meetings with AMSPDC)
NIH Pediatric Research Report
NIH Primary Care Research Network (later called CARE for Health)
All of Us Research Program pediatrics update
A New Approach to NICHD Network Clinical Studies: Two Years Later
Pediatric Medical Devices Public-Private Partnerships
Reissue of a funding opportunity (topic redacted)
NIMH Pediatric Research Portfolio

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NASEM study: Strategies to Enhance Pediatric Health Research Funded by NIH
Future topic: Including youth voices in research

2023 Meetings
All of Us Research Program pediatrics update
Pediatric Medical Device Subgroup Public Private Partnership update
HEAL Pediatric Pain concept development
NCI Childhood Cancer Data Initiative
Surgeon Scientist Funding Across NIH
NIH Clinical Center Pediatric Research Strategic Plan
RCDC Pediatric Research Category
Pediatric to Adult Healthcare Transition NOSI (Notice of Special Interest)
Agency for Healthcare Research and Quality
Introduction to PCORI Support of Research Activities to Improve Children's Health
Children and Media Research Advancement (CAMRA) Act
Tackling Acquisition of Language in Kids (TALK) Initiative
NIH-wide Maternal and Pediatric Pharmacology subgroup
NIH Maternal and Child Health Collaborations with the Bill and Melinda Gates Foundation
Canadian Institute for Health Research-NIH meeting on the Pediatric Impacts of the Pandemic in Canada and the United States

2022 Meetings
RECOVER updates
AAP Committee on Infectious Diseases
NIEHS Pediatric Research Portfolio
HEAL Pediatric Pain concept
Inclusion of Children in Research
NIH Lasker Scholar presentations (pediatric-focused)
Pediatric Provisions in the FY 2022 Omnibus Appropriations
Anticipating the Future of Pediatric Physician–Scientist Training and Development
NIH Firearms and Violence Research
Social Media and Youth Development/Mental Health
A New Approach to NICHD Network Clinical Studies
AAP Council on Injury, Violence, and Poison Prevention
Pediatric Disaster Science
NASEM study updates: Pediatric Subspecialty Workforce and Its Impact on Child Health and Well-Being

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2021 Meetings
Lost in Transition (to Adult Healthcare) Workshop follow-up and potential funding opportunity
Shaping and Leveraging the Pediatric Medical Devices Ecosystem
COVID-19 Updates (Post-acute sequelae workshop and NIH-wide initiative)
Pediatric Neurological Effects of SARS-CoV-2 Infection
NINDS Pediatric Research Portfolio
EPA Office of Children's Health Protection
RADx-UP Return to Schools Initiative
AAP Committee on Pediatric AIDS
NIDDK Pediatric Research Portfolio
Pediatric Research in the Clinical Center
NCCIH Pediatric Research Portfolio
NIAAA Pediatric Research Portfolio

2020 Meetings
Pediatric Training at NIH/Pediatric Training Analysis
Pediatric Research Training Programs (Pediatric Scientist Development Program; Child Health Research Career development Award)
Pediatric Transition to Adult Healthcare workshop
STRIDES initiative; Gabriella Miller Kids First Data in STRIDES
Bill and Melinda Gates Foundation meeting recap (maternal and child health subgroup)
NIH ECHO program
HEAL pediatric research projects
COVID-19 – Maternal/Child Health subgroup
Inclusion Across the Lifespan II (workshop)
NIDCD Pediatric Research Portfolio
AAP Committee on Pediatric Research
All of Us Research Program
Workshop updates (Addressing Neuroimaging Challenges Across Populations and Settings; COVID-19 in Pregnancy: Clinical, Research, and Therapeutics Updates; Toward the Use of Buprenorphine in Infants)
Federal Interagency Forum for Child and Family Statistics
NCI Pediatric Research Portfolio

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Appendix G

Pediatric Representation on Scientific Review

TABLE G-1 Pediatric Representation within NIH Study Sections, as of April 24, 2025

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Reproductive, Perinatal, and Pediatric Health	20	9	45%
Advancing Therapeutics - B	9	3	33%
Developmental Brain Disorders	23	7	30%
Human Studies of Diabetes and Obesity	18	5	28%
Immunobiology of Transplantation and Alloimmunity	15	4	27%
Lung Immunology and Infection	16	4	25%
Neurogenesis and Cell Fate	16	4	25%
Pregnancy and Neonatology	20	5	25%
Basic Biology of Blood, Heart and Vasculature	21	5	24%
Psychosocial Development, Risk, and Prevention	21	5	24%
Molecular and Structural Immunology	17	4	24%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Therapeutic Approaches to Genetic Diseases	17	4	24%
Population-Based Research in Infectious Disease	13	3	23%
Cancer and Hematologic Disorders	17	3	18%
Child Psychopathology and Developmental Disabilities	23	4	17%
Digestive and Nutrient Physiology and Diseases	18	3	17%
Nutrition and Metabolism in Health and Disease	18	3	17%
Surgery, Anesthesiology, and Trauma	24	4	17%
Digestive System Host Defense, Microbial Interactions, and Immune and Inflammatory Diseases	19	3	16%
Interdisciplinary Clinical Care in Specialty Care Settings	19	3	16%
Innate Immunity B	19	3	16%
Hemostasis, Thrombosis, Blood Cells, and Transfusion	20	3	15%
Mechanisms of Cancer Therapeutics B	20	3	15%
Pathobiology of Kidney Disease	20	3	15%
Addiction Risks and Mechanisms	14	2	14%
Development - 1	7	1	14%
Interspecies Microbial Interactions and Infections	15	2	13%
Clinical Neuroimmunology and Brain Tumors	23	3	13%
Basic Mechanisms of Diabetes and Metabolism	16	2	13%
Pulmonary Injury Remodeling and Repair	17	2	12%
Genetics of Health and Disease	26	3	12%
Cell Signaling and Molecular Endocrinology	18	2	11%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
HIV Comorbidities and Clinical Studies	18	2	11%
Kidney and Urological Systems Function and Dysfunction	18	2	11%
Viral Dynamics and Transmission	18	2	11%
Atherosclerosis and Vascular Inflammation	19	2	11%
Clinical Informatics and Digital Health	19	2	11%
Lifestyle Change and Behavioral Health	19	2	11%
Science of Implementation in Health and Healthcare	19	2	11%
Bacterial–Host Interactions	20	2	10%
Biobehavioral Medicine and Health Outcomes	20	2	10%
Cancer Cell Biology	20	2	10%
Cardiovascular and Respiratory Diseases	20	2	10%
Gene Regulation in Cancer	20	2	10%
Hepatobiliary Pathophysiology	20	2	10%
Immunity and Host Defense	20	2	10%
Motor Function, Speech, and Rehabilitation	20	2	10%
Pathophysiology of Obesity and Metabolic Disease	20	2	10%
Pathogenic Eukaryotes	20	2	10%
Environmental Determinants of Disease	21	2	10%
Health Services: Quality and Effectiveness	21	2	10%
Clinical Neuroplasticity and Neurotransmitters	22	2	9%
Molecular Cancer Diagnosis and Classification	22	2	9%
Skeletal Biology Development and Disease	23	2	9%
Clinical Oncology	24	2	8%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Molecular and Cellular Biology of Virus Infection	15	1	7%
Anti-Infective Resistance and Targets	16	1	6%
Cellular Immunotherapy of Cancer	16	1	6%
HIV Coinfections and HIV-Associated Cancers	16	1	6%
Macromolecular Structure and Function C	16	1	6%
Neuroscience of Basic Visual Processes	16	1	6%
Therapeutic Immune Regulation	16	1	6%
Mechanisms of Cancer Therapeutics C	17	1	6%
Skin and Connective Tissue Sciences	17	1	6%
Therapeutic Development and Preclinical Studies	17	1	6%
Basic Mechanisms in Cancer Health Disparities	18	1	6%
Clinical Management in General Care Settings	18	1	6%
Development - 2	18	1	6%
Organization and Delivery of Health Services	18	1	6%
Pulmonary Vascular Disease and Physiology	18	1	6%
Skeletal Muscle and Exercise Physiology	18	1	6%
Viral Pathogenesis and Immunity	18	1	6%
Adaptive Immunity	19	1	5%
Bacterial Virulence	19	1	5%
Community Influences on Health Behavior	19	1	5%
Auditory System	20	1	5%
Brain Injury and Neurovascular Disorders	20	1	5%
Cancer Genetics	20	1	5%

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TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Drug Discovery and Molecular Pharmacology C	20	1	5%
Etiology, Diagnostic, Intervention, and Treatment of Infectious Diseases	20	1	5%
Biobehavioral Mechanisms of Emotion, Stress and Health	20	1	5%
Molecular Neurogenetics	20	1	5%
Pathophysiological Basis of Mental Disorders and Addictions	20	1	5%
Social Psychology, Personality, and Interpersonal Processes	20	1	5%
Tumor Host Interactions	20	1	5%
Cellular and Molecular Biology of Glia	21	1	5%
Clinical Translational Imaging Science	21	1	5%
Drug and Biologic Therapeutic Delivery	21	1	5%
Healthcare and Health Disparities	21	1	5%
Chronic Dysfunction and Integrative Neurodegeneration	22	1	5%
Clinical Data Management and Analysis	22	1	5%
Emerging Imaging Technologies and Applications	22	1	5%
Mechanisms of Autoimmunity	22	1	5%
Mechanisms of Cancer Therapeutics A	22	1	5%
Integrative Myocardial Physiology/ Pathophysiology A	22	1	5%
Advancing Therapeutics A	23	1	4%
Biomaterials and Biointerfaces	23	1	4%
Integrative Myocardial Physiology/ Pathophysiology B	23	1	4%
Population and Public Health Approaches to HIV/AIDS	23	1	4%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Aging, Injury, Musculoskeletal, and Rheumatologic Disorders	15	0	0%
Adult Lifespan Psychopathology	17	0	0%
Aging Systems and Geriatrics	20	0	0%
Analytics and Statistics for Population Research Panel A	17	0	0%
Analytics and Statistics for Population Research Panel B	13	0	0%
Biochemistry and Biophysics of Membranes	18	0	0%
Basic Cancer Immunobiology	10	0	0%
Biochemical and Cellular Oncogenesis	19	0	0%
Biology and Development of the Eye	20	0	0%
Biodata Management and Analysis	20	0	0%
Brain Injury and Neurovascular Pathologies	19	0	0%
Behavioral Neuroendocrinology, Neuroimmunology, Rhythms, and Sleep	20	0	0%
Biobehavioral Regulation, Learning and Ethology	13	0	0%
Bioengineering and Tissue Engineering for Neuroscience	19	0	0%
Bioengineering, Technology and Surgical Sciences	19	0	0%
Chemical Biology and Probes	18	0	0%
Clinical Integrative Cardiovascular and Hematological Sciences	17	0	0%
Cellular Mechanisms in Aging and Development	21	0	0%
Cellular, Molecular, and Integrative Reproduction	16	0	0%
Cellular and Molecular Biology of Neurodegeneration	18	0	0%
Cellular and Molecular Technologies	23	0	0%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Cancer Prevention	17	0	0%
Chemical Synthesis and Biosynthesis	18	0	0%
Cell Structure and Function-1	20	0	0%
Cellular Signaling and Regulatory Systems	20	0	0%
Drug and Biologic Disposition and Toxicity	15	0	0%
Drug Discovery and Molecular Pharmacology A	20	0	0%
Drug Discovery and Molecular Pharmacology B	20	0	0%
Enabling Bioanalytical and Imaging Technologies	21	0	0%
Genomics, Computational Biology, and Technology	20	0	0%
Genetic Variation and Evolution	16	0	0%
Human Complex Mental Function	19	0	0%
HIV/AIDS Intra- and Inter-Personal Determinants and Behavioral Interventions	19	0	0%
HIV Immunopathogenesis and Vaccine Development	15	0	0%
Health Promotion in Communities	20	0	0%
HIV Molecular Virology, Cell Biology, and Drug Development	19	0	0%
Integrative and Clinical Endocrinology and Reproduction	12	0	0%
Imaging Guided Interventions and Surgery	21	0	0%
Innovations in Nanosystems and Nanotechnology	21	0	0%
Imaging Probes and Contrast Agents	18	0	0%
Interventions to Prevent and Treat Addictions	21	0	0%
Instrumentation and Systems Development	22	0	0%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Imaging Technology Development	22	0	0%
Imaging Technology for Neuroscience	14	0	0%
Integrative Vascular Physiology and Pathology	21	0	0%
Kidney, Endocrine, and Digestive Disorders	11	0	0%
Language and Communication	18	0	0%
Lifestyle and Health Behaviors	11	0	0%
Learning, Memory, and Decision Neuroscience	18	0	0%
Modeling and Analysis of Biological Systems	19	0	0%
Molecular and Cellular Neuropharmacology	16	0	0%
Molecular Genetics	23	0	0%
Maximizing Investigators' Research Award A	14	0	0%
Maximizing Investigators' Research Award B	14	0	0%
Maximizing Investigators' Research Award C	15	0	0%
Maximizing Investigators' Research Award D	15	0	0%
Maximizing Investigators' Research Award E	12	0	0%
Maximizing Investigators' Research Award F	14	0	0%
Musculoskeletal Rehabilitation Sciences	19	0	0%
Macromolecular Structure and Function A	18	0	0%
Macromolecular Structure and Function B	19	0	0%
Musculoskeletal Tissue Engineering	20	0	0%
Neurotoxicology and Alcohol	19	0	0%
Neuronal Communications	20	0	0%

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TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Neurodifferentiation, Plasticity, Regeneration, and Rhythmicity	18	0	0%
Neuroscience of Interoception and Chemosensation	13	0	0%
Neurobiology of Motivated Behavior	21	0	0%
Neurological, Mental, and Behavioral Health	20	0	0%
Neural Oxidative Metabolism, Mitochondria, and Cell Death	23	0	0%
Neural Basis of Psychopathology, Addictions, and Sleep Disorders	21	0	0%
Neurobiology of Pain and Itch	19	0	0%
Oral, Dental, and Craniofacial Sciences	19	0	0%
Prokaryotic Cell and Molecular Biology Study Section	16	0	0%
Pathophysiology of Eye Disease 1	20	0	0%
Pathophysiology of Eye Disease 2	20	0	0%
Radiation Therapeutics and Biology	24	0	0%
Skeletal Biology Structure and Regeneration	22	0	0%
Social and Environmental Determinants of Health	16	0	0%
Sensory–Motor Neuroscience	15	0	0%
Social Sciences and Population Studies A	17	0	0%
Social Sciences and Population Studies B	17	0	0%
Tumor Evolution, Heterogeneity, and Metastasis	18	0	0%
Translational Immuno-Oncology	16	0	0%
Transmission of Vector-Borne and Zoonotic Diseases	19	0	0%
Vaccines Against Infectious Diseases	20	0	0%
Cognitive Disorder and Brain Aging	7	0	0%

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TABLE G-1 Continued

Study Section Description	No. of Standing Committee Members	No. of Pediatrics-Specific Members	Percentage
Clinical Neurodegeneration Translational Neuroscience	25	0	0%
	3459	**204**	**6%**

NOTE: The committee included study section members as having pediatrics expertise if their primary affiliation listed was with a department of pediatrics at a medical school or they were employed at a children's hospital. This method excludes study section members who may have a secondary appointment or whose pediatric expertise is not otherwise evident and thus should be considered illustrative as opposed to a fully representative picture. The committee also did not have access to the number of pediatric-related applications in each study section, and whether the applications reviewed in the study section required pediatric expertise. Additionally, the table does not include the count of ad hoc reviewers with pediatric expertise, so the percentage of reviewers with pediatric expertise on the study section may be slightly inexact. This table includes standing members participating in meetings for 1 year.

TABLE G-2 Pediatric Representation within NIH Study Sections/ Special Emphasis Panels, as of April 24, 2025

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
NIH Director's New Innovator Award Program	229	11	5%
RFA-RM-24-004: NIH Director's Transformative Research Awards	176	11	6%
Member Conflict: Respiratory Sciences	59	10	17%
Member Conflicts: Respiratory Topics	59	10	17%
Member Conflicts - Pulmonary Diseases	59	10	17%
NIH Director's New Innovator Award Program (DP2)	228	9	4%
RFA-RM-24-002: 2025 NIH Director's Pioneer Award Review	210	9	4%
Member Conflict: Topics in Urology and Nephrology	66	9	14%
Member Conflict: Liver Diseases, Pharmacology and Toxicology	66	9	14%
Member Conflict: Topics in Gastroenterology	66	9	14%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Member Conflict: Cognition, Speech, Language, and Mental Health	62	9	15%
Biobehavioral Mechanisms of Health	62	9	15%
RFA-AR-24-007: HEAL Initiative - HEAL KIDS	62	9	15%
Member Conflict: Topics in Biobehavioral Research	62	9	15%
RFA Panel: NIH Director's Early Independence Award Review	188	7	4%
Advancing Therapeutics	32	7	22%
Mechanisms of Autoimmunity and Inflammation	48	6	13%
Member Conflict: Topics in Transplantation, Immunology, and Autoimmunity	48	6	13%
Topics in Bacterial Pathogenesis and Molecular Host Interactions	48	6	13%
PAR-23-064 Program Projects: Interactions Between HIV-1 and Opioids	48	6	13%
Cardiovascular Differentiation and Development SEP	23	6	26%
Cardiovascular Differentiation and Development	23	6	26%
Topics in Health Services Research: Maternal, Reproductive, and Child Health and Substance Use	38	5	13%
Member Conflict: Musculoskeletal Sciences	57	4	7%
Member Conflict: Skeletal Muscle and Rehabilitation Sciences	57	4	7%
Member Conflict: Skin, Oral, and Dental Sciences	57	4	7%
PAR Panel: Fogarty Global Brain and Nervous System Disorders Research across the Lifespan	51	4	8%
Member Conflict: Special Topics in Neural Disorders and Clinical Neuroscience	38	4	11%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Nucleic Acid Therapeutic Delivery (NATD)	40	3	8%
Topics in Health Services Research: Big Data, Health Information Technology, and Clinical Informatics	39	3	8%
Topics in Clinical Data Management, Analysis, Informatics, and Digital Health A	36	3	8%
Clinical Topics in Neuroimmunology and Neuroinflammation	33	3	9%
RFA Panel: NIH Research Software Engineer (RSE) Award (R50)	33	3	9%
Special Topics: Noninvasive Neuromodulation and Neuroimaging Technologies	30	3	10%
Collaborative Applications: Clinical Studies of Mental Illness	29	3	10%
Topics in Mechanisms of Autoimmunity	19	3	16%
Special Topics in Psychosocial Development, Risk, and Prevention	19	3	16%
Member Conflict: Bioengineering, Surgery, Anesthesiology, and Trauma	61	2	3%
Member Conflict: Medical Imaging Investigations	61	2	3%
Topics in Clinical Data Management, Analysis, Informatics, and Digital Health B	45	2	4%
Member Conflict: Cancer Biology	41	2	5%
Member Conflict: Cancer Biology	41	2	5%
Cancer Biology AREA/REAP Review	41	2	5%
Member Conflict: Topics in Basic Neuroscience	31	2	6%
Cellular and Molecular Aspects of the Blood–Brain Barrier and Neurovascular System and Therapeutic Strategies	31	2	6%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Member Conflict: Hypertension and Thrombosis	30	2	7%
Member Conflict: Atherosclerosis, Vascular Pathobiology, and Hematopoiesis	30	2	7%
PAR-24-167 and PAR-24-259 Panel for R24 and S15 Instrumentation	30	2	7%
Topics in Neurobehavior, Neuropsychology, and Neurodevelopment	29	2	7%
Member Conflict: Immunity and Host Defense	28	2	7%
Member Conflict: Disease Control and Applied Immunology	28	2	7%
Special Topics in Transmission of Vector-Born and Zoonotic Diseases	28	2	7%
IIA Innate Immunity-A	24	2	8%
PAR Panel: Innovative Research in Cancer Nanotechnology	24	2	8%
Special Topics in Social Influences and Environmental Determinants of Health	24	2	8%
Member Conflict: Topics in Endocrinology, Metabolism, and Reproductive Systems	23	2	9%
Member Conflict: Health Services and Systems	21	2	10%
RFA Panel: CounterACT Cooperative Agreements for Therapeutic Development and Basic Research on Chemical Threats Affecting the Nervous System	20	2	10%
PAR Panel: Centers for Precision Disease Modeling (U54)	19	2	11%
Societal and Ethical Issues in Research	19	2	11%
RFA-NS-22-025: Helping to End Addiction Long-Term (HEAL) K99	12	2	17%
PAR: Enhancing Mechanistic Research on Precision Probiotic Therapies	9	2	22%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Myalgic Encephalomyelitis–Chronic Fatigue Syndrome	92	1	1%
Member Conflict: Auditory, Visual, and Cognitive Neuroscience	92	1	1%
Member Conflict: Pain, Olfactory, and Sensorimotor Neuroscience	92	1	1%
Member Conflict: Alcohol and Motivated Behavior	92	1	1%
Member Conflict: Sleep, Rhythms, Social Behaviors, and Neuroimmunology	92	1	1%
PAR Panel: Fogarty HIV Research Training Program for Low- and Middle-Income Country Institutions	51	1	2%
Member Conflict: Topics in HIV Comorbidities, Coinfections, and Associated Cancers	51	1	2%
Member Conflict: Topics in HIV Research	51	1	2%
Training in Veterinary and Comparative Medicine	43	1	2%
Member Conflict: Advancing Therapeutics	43	1	2%
PAR Panel: Molecular and Cellular Underpinnings and Integrative Neuropathophysiology of Alzheimer's Disease and Related Dementias (ADRD)	39	1	3%
Special Topics: Bioanalytical, Molecular, and Cellular Sciences and Technologies	38	1	3%
AREA/REAP: Musculoskeletal, Skin, and Oral Sciences	38	1	3%
Neuroimmune, Neuroinflammation, and Metabolic Factors Involved in Neurodegenerative Disorders	37	1	3%
Member Conflict: Cardiovascular Sciences	35	1	3%
Special Topics in Bioengineering and Instrumentation	32	1	3%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Infectious Disease Drug Development and Molecular Pharmacology	32	1	3%
Member Conflict: Topics in Bacterial Virulence	31	1	3%
Member Conflict: Topics in Pathogenic Eukaryotes	31	1	3%
Neuro Informatics, Computational and Data Analysis	31	1	3%
CSR Special Emphasis Panels	29	1	3%
Member Conflict: Epidemiology and Population Health	27	1	4%
PAR Panel: The NCI Transition and Community Oncology Career Development Awards, Institutional Research Training, Research Education Grants	26	1	4%
PAR Panel: Intervention Research to Improve Native American Health	26	1	4%
Member Conflict: Molecular Genetics	25	1	4%
PAR Panel: Animal and Biological Material Resources and Centers	25	1	4%
Immunology–Oncology	24	1	4%
PAR: HIV/AIDS Behavioral Interventions and Health Outcomes	21	1	5%
RFA-OD-23-017: Tobacco Regulatory Science Panel A	21	1	5%
Human Genetics and Genomics Topics	20	1	5%
Member Conflict: Bioengineering, Biodata, and Biomodelling Technologies	18	1	6%
Research Enhancement Awards: Cell and Molecular Biology	18	1	6%
PAR-23-281: Research Projects to Enhance Applicability of Mammalian Models for Translational Research	18	1	6%
Innate Immunity A	18	1	6%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Member Conflict: Neurotechnology and Vision	18	1	6%
Biology of Aging	17	1	6%
Member Conflict: Topics in Adaptive and Innate Immunity	13	1	8%
RFA-OD-25-002: Investigation of Co-Occurring Conditions across the Lifespan to Understand Down Syndrome (INCLUDE)	6	1	17%
Special Topics: Pathophysiology of Eye Disease	5	1	20%
Clinical Care and Health Interventions	72	0	0%
RFA-OD-23-017: Tobacco Regulatory Science (TRS)	72	0	0%
Substance Use and Prevention and Treatment of Addiction	72	0	0%
RFA-NS-24-021: HEAL Initiative—Understanding Individual Differences in Human Pain Conditions	72	0	0%
Topics in Health Services Research, Aging, and Workforce Issues	43	0	0%
PAR Panel: Metabolic, Cerebrovascular, Environmental, and Sleep Factors in Alzheimer's Disease and Related Dementias (ADRD)	36	0	0%
Imaging and Bioengineering Technology for Visual Systems (IBV)	36	0	0%
Special Topics in Biodata Management and Analysis	35	0	0%
RFA Panel: Building Sustainable Software Tools for Open Science (R03) B	34	0	0%
RFA Panel: Building Sustainable Software Tools for Open Science (R03) A	33	0	0%
PAR Panel: Topics in Neurodegeneration and Brain Injury	29	0	0%

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TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Collaborative Applications: Clinical Studies of Mental Illness	28	0	0%
PAR Panel: Maximizing Investigators' Research Award (R35)	28	0	0%
PAR-23-145: Maximizing Investigators' Research Award (MIRA) for Early-Stage Investigators	27	0	0%
Topics in Bacterial-Host Interactions	26	0	0%
Special Topics in Community-Level Interventions and Prevention Research	25	0	0%
RFA-NS-23-023: BRAIN Initiative: Targeted BRAIN Circuits Planning Projects	24	0	0%
Special Topics in Biomaterials, Nanoscience, and Drug Delivery	23	0	0%
Member Conflict: Cell Signaling, Structure, and Function	22	0	0%
Member Conflict: Developmental Biology	22	0	0%
Member Conflict: Biology of the Eye	22	0	0%
Psychosocial Risk and Interpersonal Processes of Health	22	0	0%
PAR Panel: International and Cooperative Projects for the Emerging Global Leader Award	21	0	0%
Member Conflict: Macromolecular Biophysics and Biochemistry	20	0	0%
Special Topics in Social Sciences and Population Studies	20	0	0%
Special Topics in Health Interventions and Public Health Approaches in HIV/AIDS	19	0	0%
PAR Panel: The Cellular and Molecular Biology of Complex Brain Disorders	17	0	0%

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Strategies to Enhance NIH-Funded Pediatric Research: Optimizing Child Health

TABLE G-2 Continued

Study Section Description	No. of Panel Members	No. of Pediatrics-Specific Members	Percentage
Special Topics in Brain and Neural Injury	17	0	0%
Member Conflict: Applied Immunology and Disease Control	17	0	0%
Member Conflict: Neurodegeneration	16	0	0%
Imaging Guided Interventions and Surgery	14	0	0%
USU—Center for Health Services Research	14	0	0%
Special Topics in Transmission of Vector-Borne and Zoonotic Diseases	13	0	0%
RFA-NS-22-034: HEAL Initiative	13	0	0%
Mature Synchrotron Resources for Structural Biology	11	0	0%
Academic Research Enhancement Awards	10	0	0%
Topics in Etiology, Diagnostic, Intervention, and Treatment of Infectious Diseases	9	0	0%
	5,702	**324**	**6%**

NOTE: Special emphasis panels may use aggregate rosters, which can skew the proportion of reviewers with pediatric expertise, as these rosters include reviewers from various panels. This table includes panel members participating in one meeting occurrence.

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