NATIONAL ACADEMIES *Sciences Engineering Medicine*

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

Committee on Strategies to Enhance Pediatric Health Research Funded by NIH

JANUARY 2026

Study Sponsor

- National Institutes of Health

Committee on Strategies to Enhance Pediatric Health Research Funded by NIH

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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.

Statement of Task *(continued)*

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.

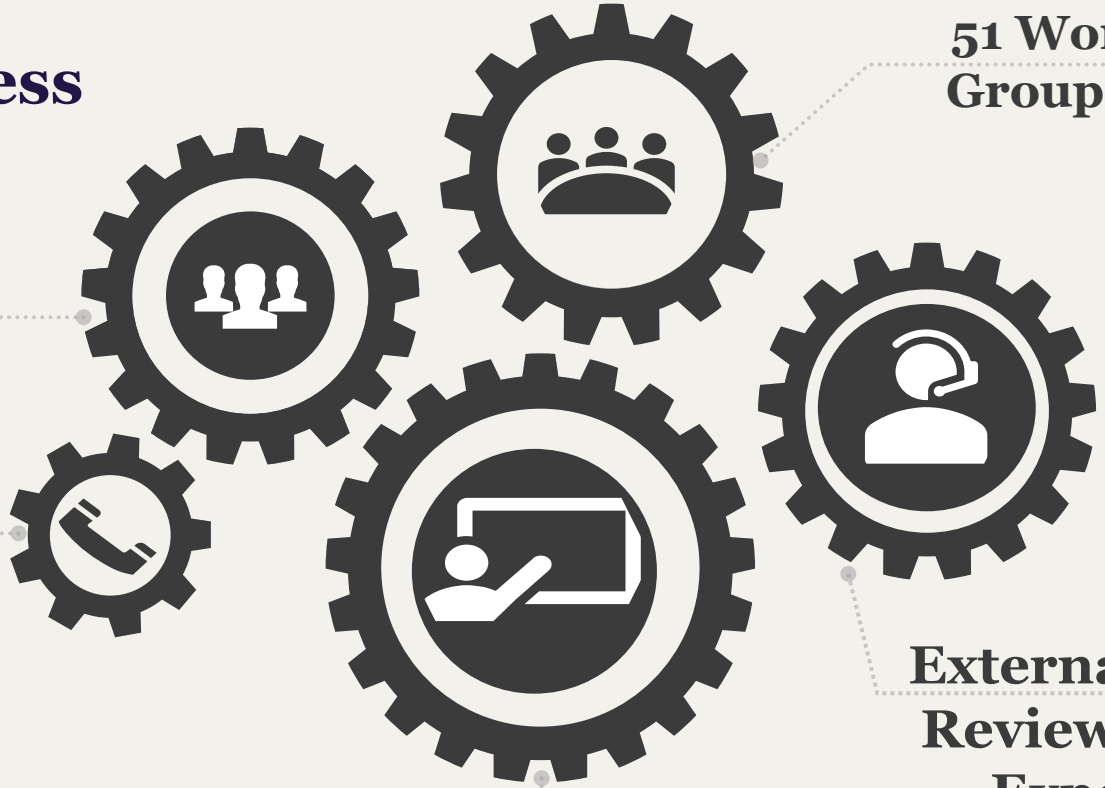
The committee may also consider ways in which the NIH Clinical Center (CC) could be used to advance innovative pediatric research (e.g., whether it would be advantageous to expand the ages eligible for research studies, areas of science uniquely suited to being conducted at the NIH CC, and potential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

What the committee will deliver: Recommendations to strengthen NIH support for child health research, while minimizing burden and cost for researchers and NIH staff

Study Process

51 Working Group Calls

5 Full Committee Meetings



Call For Perspectives

- NIH grant recipients
- Research participants and patients

External Peer Review by 14 Experts

5 Virtual Public Webinars

Committee's Vision and Goals

Make the U.S. the global leader in child health by preventing disease, reversing recent declines, and turning discoveries into better outcomes through an integrated NIH pediatric research program.

Integrate

Integrate pediatric research across NIH programs to grow a multidisciplinary pediatric research community.

Expand

Expand NIH initiatives to meet unmet child health needs across prevention, diagnosis, treatment, and well-being.

Translate

Strengthen impact by improving translation, dissemination, and implementation into practice.

Build Trust

Build trust through clear communication and dissemination of rigorous evidence that improves lifelong health.

Background

- 20th-century U.S. progress - Life expectancy rose by >30 years
- Global progress (Past 25 years) - Under-5 mortality has been cut ~in half since 2000
  - Achieved through new technologies and by adapting proven interventions to low-resource settings
- The current challenge (Now) - U.S. children are falling behind peer nations
  - U.S. child health indicators worsened over the last 25 years, with rising rates of chronic physical, mental, and neurodevelopmental conditions, functional limitations, and symptom burden
  - Children face high incidence of chronic diseases such as obesity, asthma, and diabetes

Historic gains in child survival were driven by federally funded biomedical research; today's child health burden is shifting to chronic disease and widening gaps.

NIH-Funded Pediatric Achievements

Prevention	Earlier detection	Treatment & survival
>99% decrease **Vaccine-preventable childhood infections** NIH-supported vaccine development and trials	**Hours, not days** **Newborn screening enables rapid intervention** NIH-supported validation of newborn screening	**>85% 5-year survival** **Childhood cancer** NCI pediatric cooperative groups and combination chemotherapy trials
50% reduction **Infant deaths from SIDS** NIH-supported SIDS epidemiology and sleep-position	**Noninvasive** **Cell-free prenatal screening** NIH-supported development of cell-free fetal DNA testing	**Survival at 22 weeks** **Modern neonatal intensive care** NIH foundational investments in NICU care, surfactant, and antenatal steroids
		60+ years **Cystic fibrosis life expectancy** NIH-enabled CFTR discovery and targeted therapies

Decades of **discovery → trials → implementation** improves health in children through prevention, early detection, and treatment

Two concepts that frame discussion of pediatric research funding

Life course development

Sensitive/critical periods - early hits matter more

Cumulative exposure - risk accumulates

Sequence/feedback - early exposure changes later vulnerability

This is why pediatric research funding can produce outsized lifelong health returns

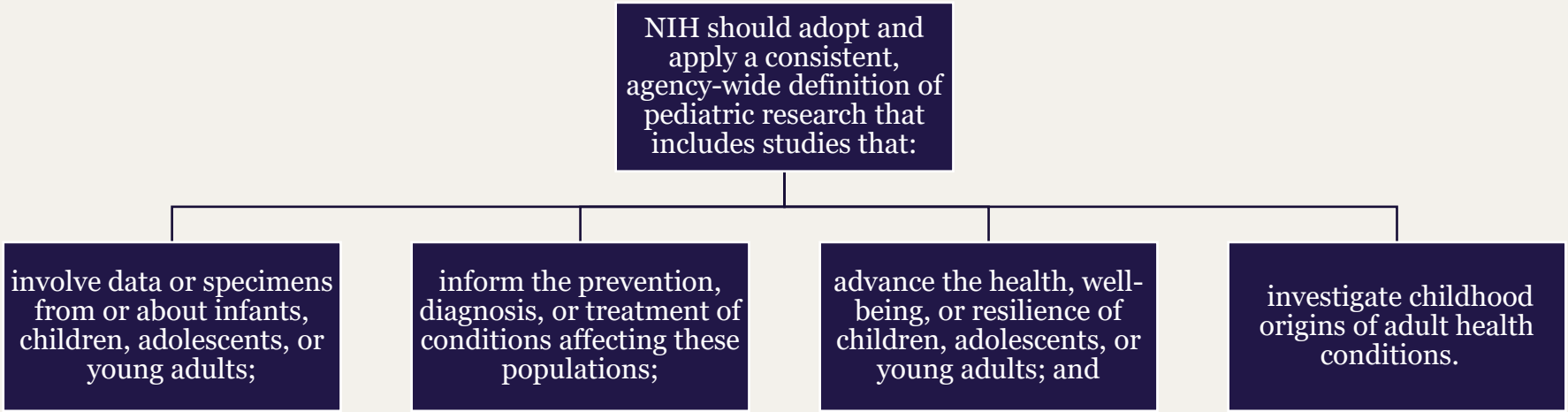
Pediatrics: definitions differ slightly

NIH's definition of a child: <18 years

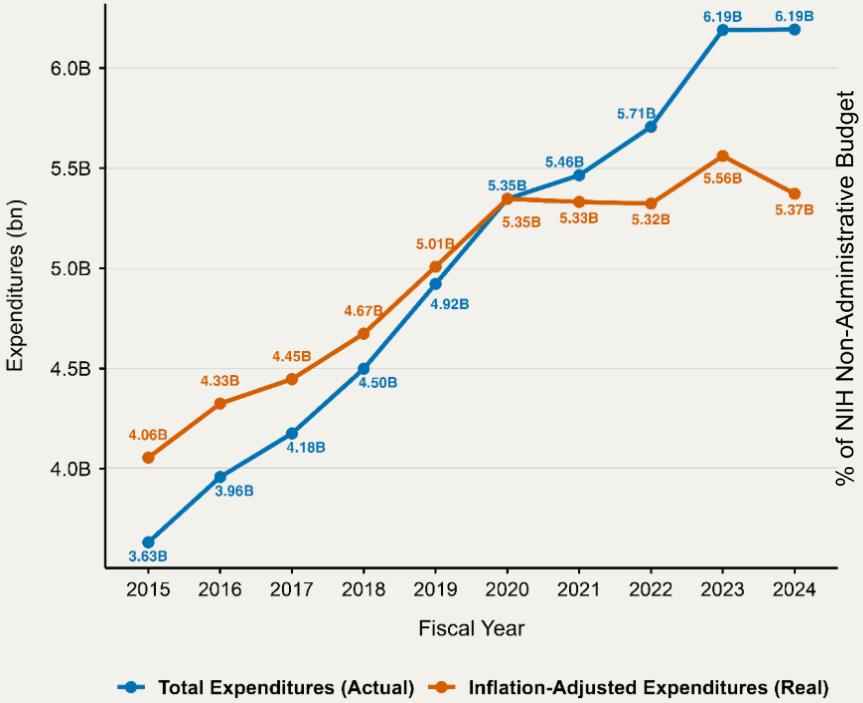
NIH's definition of pediatric research: <21 years (scope includes development + conditions)

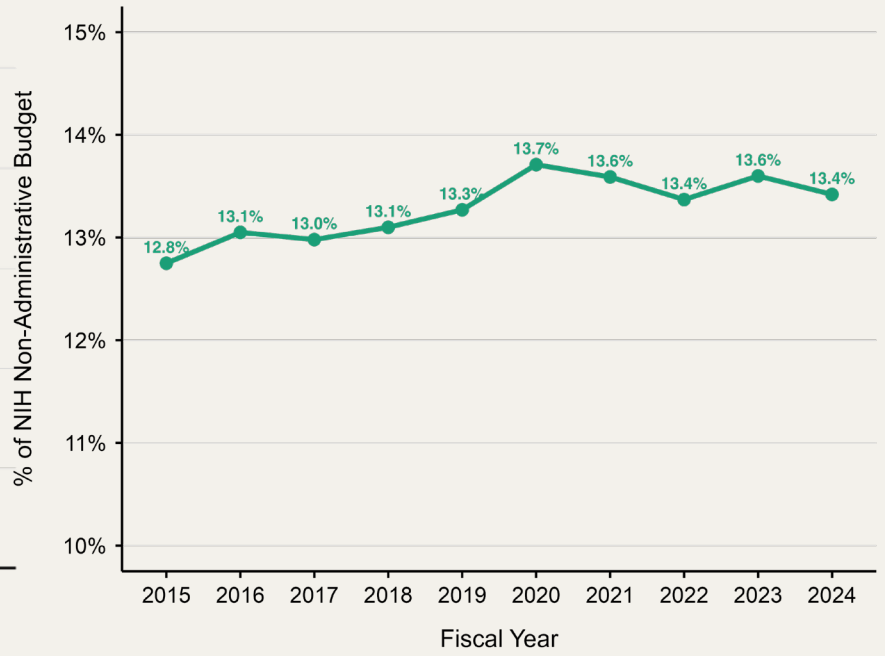
We acknowledged definitional nuance and kept focus on the report's outcomes and impact

Recommendation 3-1



NIH Pediatric Research Portfolio





NIH RCDC: How funding is categorized

Research, Condition, and Disease Categorization (RCDC) supports NIH public reporting

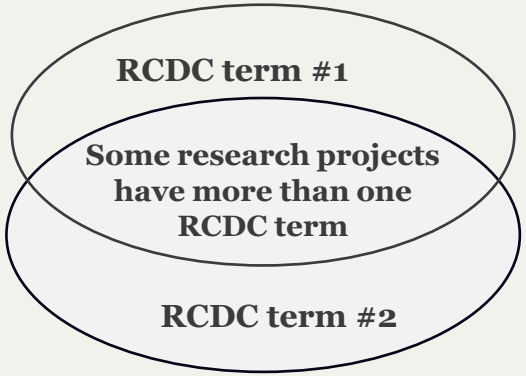
What it does:

- Codes NIH-funded projects for public reporting
- Tags grants + activities so the public can analyze appropriations
- **327** Total RCDC research/condition/ disease categories

Who can request categories:

- NIH leadership
- Advocacy groups
- Congress
- The White House

How RCDC tagging works:

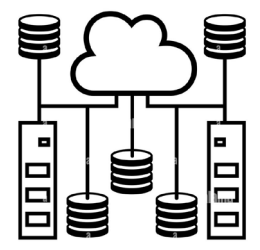


Some health conditions are not assigned to an RCDC term

310 Spending categories assigned to projects also tagged “Pediatric”

Recommendation 3-2

Modernize RCDC to Transparently Measure Pediatric Research Funding



RCDC

Improve Pediatric Representation

- Developmental stages
- Pediatric conditions that represent top categories impacting child health and mortality
- To improve specificity of diagnostic labeling

Recommendation 3-2 *(continued)*

1) Publish (and regularly update) a reference paper describing RCDC's methods

2) Evaluate the validity of terms like "pediatric"

3) Allow export of multiple categories per project

4) Allow download of all NIH projects by year

5) Link each project to the funding announcement

NIH Clinical Center

- Hosts clinical trials and natural history studies for rare and complex pediatric diseases
- Pediatric facilities include:
  - 18-bed inpatient pediatric unit
  - 14-bed pediatric day hospital
  - 4-bed pediatric behavioral health unit with 2-bed day hospital
  - Dedicated outpatient clinics
- Provides access to pediatric subspecialists through NIH institutes and partner hospitals

Recommendation 3-3

NIH Clinical Center should continue to prioritize child health research that is difficult to pursue in extramural environments

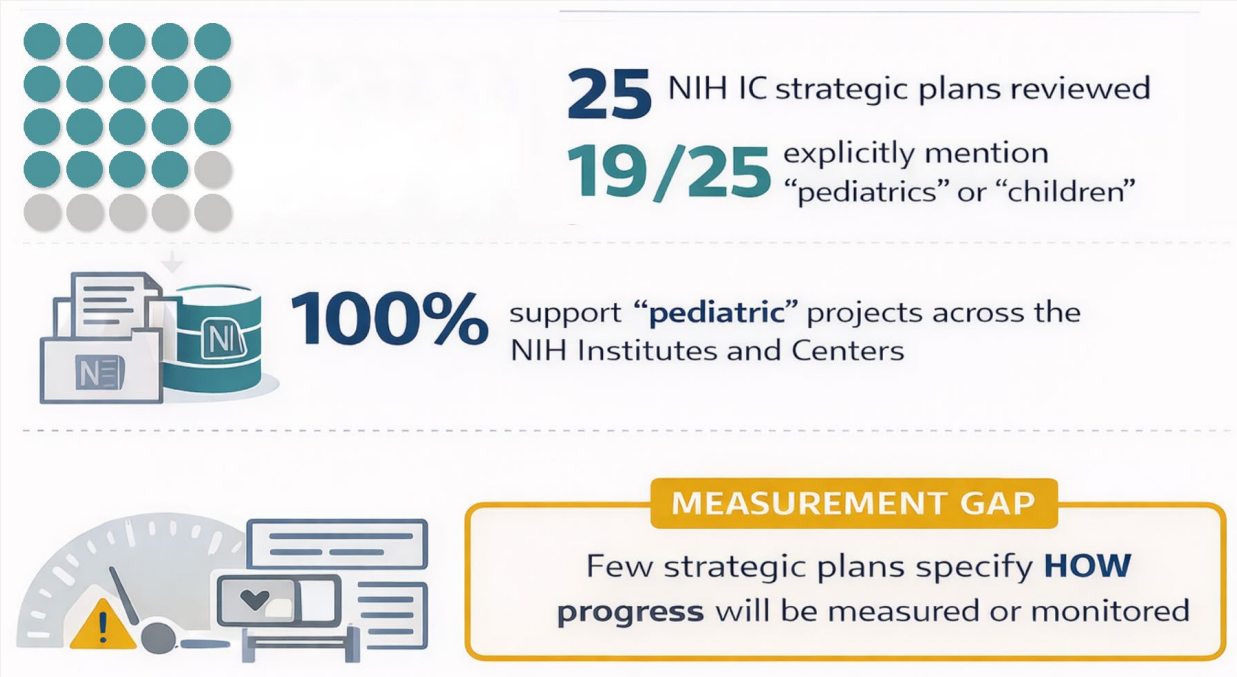
- **Opportunities to study rare diseases**
- **Longitudinal follow-up**
- **Early-stage trials**

Recommendation 3-3 *(continued)*

And Sustain and Grow Pediatric Research at the NIH Clinical Center

- Increase capacity safely
- Include younger/ higher-acuity patients
- Develop external partners
- Expand training opportunities
- Present data about pediatric research at CC annually

Strategic Plans: pediatric research is ubiquitous across the NIH but planning and measurement is not



Recommendation 4-1

Include Pediatric Health In Every NIH Strategic Plan

- Childhood health shapes lifelong outcomes
- All NIH Institutes & Centers should explicitly include pediatric health and life-course impact in strategic plans

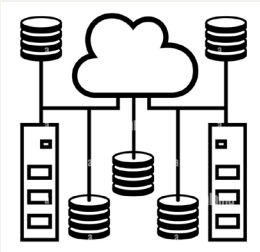
Each plan should include pediatric planning around	Suggested metrics
Workforce & Investigators	Funding rates, career advancement
Science & Innovation	Publications, discoveries
Child Health Outcomes	Morbidity, mortality
Life-Course Impact	Adult disease risk

NIH Pediatric Research Consortium (*NPeRC)*

NPeRC coordinates pediatric research across NIH Institutes and Centers

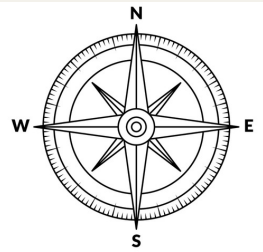
Find and close pediatric research gaps

Respond quickly to threats



Harmonize data

Build partnerships



Guide inter-IC efforts; provide consultation for ICs

NIH Pediatric Research Consortium (NPeRC) *(continued)*

$0
dedicated funding
Operates via volunteer representation

37
participants
Cross-NIH representatives

25
NIH Institutes & Centers

- No dedicated resources → *limited capacity for sustained work*
- No formal leadership structure → *inconsistent prioritization and follow-through*
- Limited enforcement mechanisms → *variable adoption across ICs*

High engagement and reach, but limited ability to execute at scale without a defined structure and resources.

Inter-IC Initiatives

- Inter-IC pediatric initiatives are supported by ≥2 NIH ICs and/or the Common Fund (OD).
- They may be congressionally mandated or IC-led with cross-IC support.
- The committee reviewed ABCD, ECHO, All of Us, and PROMIS for facilitators and barriers to advancing pediatric health research.

ECHO
Environmental influences on Child Health Outcomes
A program supported by the NIH

All of Us RESEARCH PROGRAM

Patient-Reported Outcomes Measurement Information System (PROMIS)

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

Recommendation 4-2

Elevate Pediatric Leadership at NIH

- **Elevate NPeRC to the Office of the Director**
- **Include Senior IC leadership membership within NPeRC**
  Pediatric + life-course experts from every IC
- **Annual review of strategic plans**
  Review progress and impact across IC plans

Recommendation 4-2 *(continued)*

Give NPeRC the Tools to Continue to:

- Identify research gaps
- Rapid response to emerging threats
- Harmonize data across initiatives
- Build new partnerships
- Support inter-IC initiatives
- Increase visibility

Resource NPeRC with:

1) Dedicated funding
2) Personnel/admin support
3) Ethical expertise
4) Patient advocacy expertise for community engagement

Recommendation 4-2 *(continued)*

Develop a Communication Plan

NPeRC should share findings broadly and coordinate NIH-wide response to emerging pediatric needs:

- Public facing website
- Reports
- Transparent data sharing
- Shared learning to support NIH-funded efforts

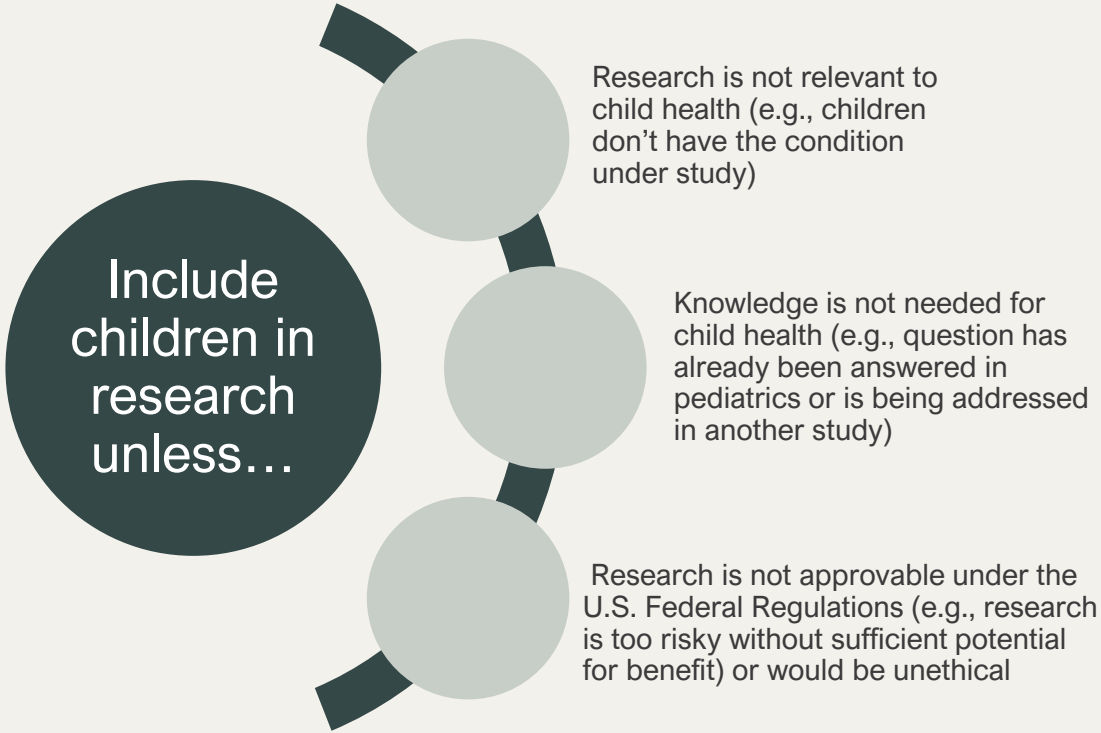
Recommendation 4-3

Initiatives across NIH should coordinate with NPeRC to include children in research in a strategic and deliberate way

NPeRC should support enforcement of existing policies requiring that studies do not exclude children without clear and adequate justification

Inclusion of Children in Research

- Congress has recognized the urgent need to correct the default of “adults first" in research
- Inclusion of Children & Across the Lifespan policies mandate including people of all ages, unless scientific or ethical reasons not to do so
  - Enforcement limited



Recommendation 5-1

The NIH should work to create a culture of ethical inclusion of children in research, focused on protecting children **through** research rather than **from** research.

Recommendation 5-1

Strategies to ensure inclusion of children in projects and proposals are:

- Update review framework by explicitly considering the inclusion of children as a component of evaluating a proposal during review
- Require and monitor pediatric representation consistent with the number of applications on all relevant standing study sections
- Ensure provision and documentation of adequate justification for exclusion of children during grant review
- Elevate prioritization of implications of research on the life course during scientific review by training reviewers to identify inclusion of children as an important part of a proposal's significance

Recommendation 5-1 *(continued)*

Make Inclusion Measurable and Transparent

Track exclusions

Annual reporting of child exclusion justifications

Publish pediatric inclusion metrics via NIH + NPeRC

Make measurable progress (or lack thereof) visible

Recommendation 5-1 *(continued)*

Provide clearer institutional guidance about the ethical inclusion of children in research by developing a toolkit on issues like:

- **Assent & consent**
- **Age de-escalation**
- **Adolescent independent consent**
- **Participant compensation**

Greater clarity will prevent unnecessary barriers to pediatric research

Structural barriers specific to pediatric research

- **Particular scientific challenges for pediatric research**
  - Rapidly developing physiological and psychological functions, sometimes in critical windows
  - Limitations of existing research models to capture pediatric developmental stages, diseases, and conditions
- **Pediatric research processes often require more time**
  - Need to enroll across more sites to obtain sufficient sample sizes
  - Assent/consent processes involve more people (parents/guardians) and can take longer as a result

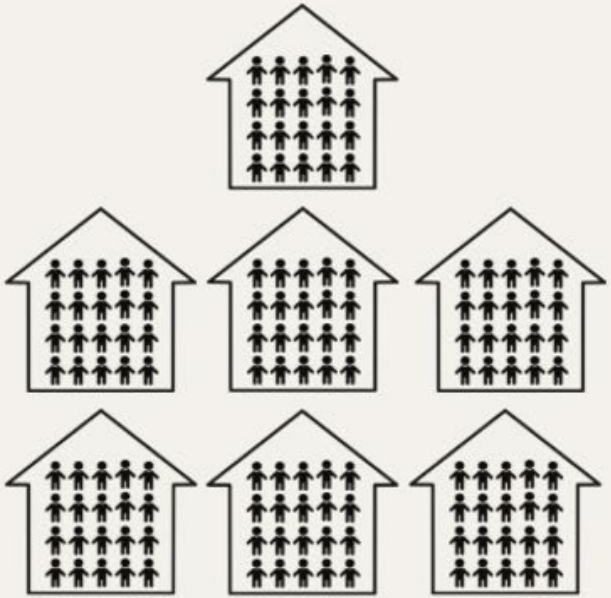
Pediatric Research

50 sites were required to enroll 1,000 children

1 stick figure = 20 participants
1 house = 2 sites

Adult Research

14 sites were required to enroll 5,625 adults



Recommendation 5-2

Pediatric research grants and budgets should support the specific needs of pediatric studies. To address the mismatch between study timelines and funding cycles, NIH should support:

Long-term studies aligned with development

Extendable pediatric grant timelines and/or renewable grant mechanisms

Bigger budgets when justified

Higher budget caps for multi-site recruitment

Longitudinal opportunities

Follow up funding for enhanced life-course learning

Recommendation 5-2 *(continued)*

NIH should also provide:

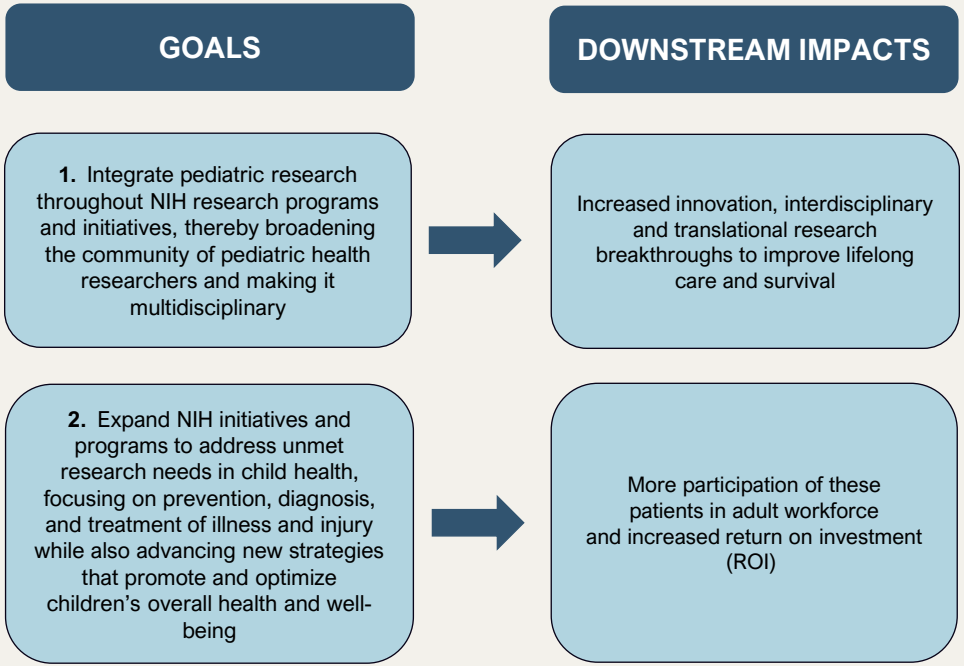
Supplements for Wider Recruitment to Enhance Generalizability

Multilingual + community engagement funding

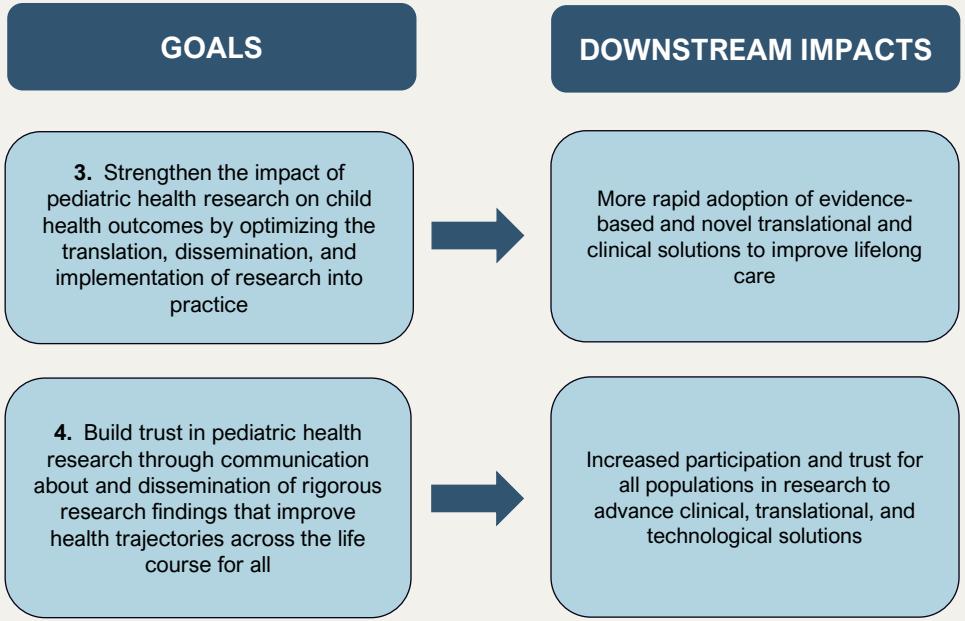
Greater Access and Use of Translational Infrastructure

Use Clinical Translational Science Award hubs for pediatrics

Committee's Goals and Downstream Impacts



Committee's Goals and Downstream Impacts *(continued)*



Committee's Vision for the Future:
Invest early. Prevent disease. Strengthen society.

- **Reorientation:** prevention-first, child-centered priorities focused on future health, well-being, and resilience

- **Alignment:** decisions and priorities grounded in data, supporting researchers, addressing public concerns, showing impact

- **Why it matters:** children are America's greatest lever towards a productive and healthier society

NIH-led pediatric research →
prevention + targeted therapeutics + cures

lower disease burden, healthier communities, productivity

*America's **children** are our most profound opportunity to shape a better and healthier future*

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https://www.nationalacademies.org/our-work/strategies-to-enhance-pediatric-health-research-funded-by-nih