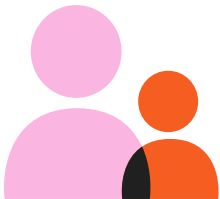


AMERICAN MADE CURES

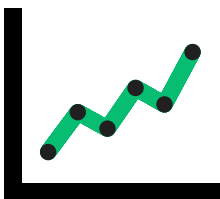
Medical Research at Children's Hospitals Improves Child Health

As global leaders in pediatric medical training and research, children's hospitals are committed to advancing scientific innovation that focuses on pediatric patients and medical conditions that emerge in childhood. Federal research funding and direct investments from children's hospitals allow pediatric clinical research teams to identify the underlying causes of pediatric chronic diseases, determine how best to promote optimal child development, and develop safe treatments for complex pediatric medical conditions that improve long-term health outcomes.



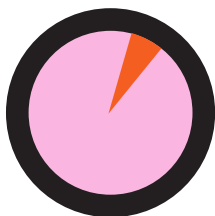
PEDIATRIC-FOCUSED RESEARCH

must be designed with children's unique developmental and physiological characteristics in mind.



ADDRESSING CHILDHOOD HEALTH

issues through research can help prevent or mitigate chronic diseases that may develop later in life.



MANY PEDIATRIC CONDITIONS

are rare and impact very few children. Smaller sample sizes and a limited market for new treatments offer fewer incentives for private funding of pediatric research, hindering the development of effective treatments



THE CHILDREN'S HOSPITAL ASSOCIATION URGES CONGRESS TO:

1. Make sustained and robust investments in pediatric medical research across the National Institutes of Health (NIH).
2. Work with children's hospitals to strengthen the biomedical research infrastructure and prioritize children's health.

INNOVATIVE RESEARCH AT CHILDREN'S HOSPITALS

Children's hospitals are leaders in pediatric medical research and major grantees of the NIH, including the National Institute of Child Health and Development, which is the sole NIH institute focused on infant and child health. **Federally funded research partnerships at children's hospitals:**

- Facilitate the development of curative and life-enhancing therapies for children with chronic and life-threatening illnesses.
- Advance research in areas that include infectious diseases, infant and child safety, nutrition, and the effects of environmental factors on children's health.



A child diagnosed with a rare genetic disorder was successfully treated with a customized gene editing therapy by a team at **Children's Hospital of Philadelphia** and Penn Medicine. The infant spent the first several months of his life in the hospital on a very restrictive diet. He is now growing well and thriving after receiving the personalized therapy.



Boston Children's is conducting research on how abnormal fetal brain development may contribute to disabilities in pediatric patients with congenital heart disease. The findings will inform clinical care and therapy advancements.



Children's Mercy Kansas City developed a therapeutic medication to treat neurological movement disorders in children. The product is also intended to manage Parkinson's disease in adults.



The **Child Health Research Institute**, a partnership between **Children's Nebraska** and the University of Nebraska Medical Center, is studying a novel strategy for treating the most aggressive form of medulloblastoma, a childhood brain cancer. The research targets identifying FDA-approved non-cancer medications that can be used to treat brain tumors, with less long-term health impacts than traditional forms of chemotherapy.

UNIQUE CONSIDERATIONS FOR CHILDREN

Consistent and strong federal funding is crucial to support children's hospitals in addressing the unique needs of children through pediatric medical research.

1. **Ensuring research protocols are developmentally appropriate is crucial.** All procedures, tools, and materials must be designed to align with children's cognitive, physical, and emotional abilities while incorporating the role of parents and caregivers.
2. **Children's developing bodies process medications differently than adults.** Additional safeguards must be taken to determine pediatric dosing methods and other interventions to prevent adverse effects from new treatments.
3. **Limited funding to support training pediatric physician-scientists early in their careers has contributed to a shortage of pediatric researchers.**¹ Instability and significant cuts to federal research funding will worsen this workforce shortage and disproportionately impact students and early career researchers.

1. "The Future Pediatric Subspecialty Physician Workforce: Meeting the Needs of Infants, Children, and Adolescents," National Academies of Sciences, Engineering, and Medicine, 2023.

2. "Population Distribution by Age," Kaiser Family Foundation, 2023.

3. Children's Hospital Association's Analysis of Estimates of Funding for Various Research, Condition, and Disease Categories (RCDC), Office of Extramural Research, National Institutes of Health, U.S. Department of Health and Human Services, May 2024.

4. Children's Hospital Association's Analysis of NIH Awards by Location & Organization, Office of Extramural Research, National Institutes of Health, U.S. Department of Health and Human Services, January 2025.

5. "Cancer in Children and Adolescents," National Cancer Institute, National Institutes of Health, August 2024.

6. "The landscape for rare diseases in 2024," The Lancet Global Health: Vol. 12, No. 3, March 2024.

KEY FACTS

While 23% of the U.S. population is age 18 or under,² less than

4%

of total NIH funding was awarded to pediatric-related research/disease areas.³

>25%

of NIH estimated funds designated for pediatric research/disease areas was awarded to children's hospitals and their pediatric affiliates at medical schools.^{3,4}

Thanks to advances in effective therapies, the five-year survival rate for children with cancer has

DOUBLED

over the last 50 years, reaching 85%.⁵

70%

of rare genetic conditions present in childhood, and 95% do not have approved treatments. About 30% of children with a rare disease will die before the age of 5.⁶