Gabriella Miller Kids First Pediatric Research Program (Kids First)
NIH Common Fund Program

National Institutes of Health
Office of Strategic Coordination - The Common Fund

National Institutes of Health

Program Vision

Alleviate suffering from childhood cancer and structural birth defects by fostering collaborative research to uncover the etiology of these diseases and support data sharing within the pediatric research community.

Background

Childhood cancers and structural birth defects have profound, life-long effects on patients and their families. The role of genetics in these areas is not fully understood, and this lack of understanding is impeding researchers from developing preventive, early-detection, and therapeutic interventions.

Prior to her death from cancer in 2013, 10-year-old Gabriella Miller called on Congress to increase support for pediatric research. In 2014, the Gabriella Miller Kids First Research Act was signed into law, and in early 2015, the NIH Common Fund launched the Gabriella Miller Kids First Pediatric Research Program (Kids First).

Gabriella Miller Kids First PEDIATRIC RESEARCH PROGRAM

Kids First will help researchers better understand the genetic contributions to childhood cancer and structural birth defects by whole genome sequencing cohorts of children with these disorders and depositing the sequence and clinical data into the forthcoming Gabriella Miller Kids First Pediatric Data Resource. These activities will allow researchers to search and analyze the data for new causal genetic variants.

Kids First Working Group

Kids First is a trans-NIH effort supported by the NIH Common Fund.
Institutes that chair the Kids First Working Group:

- *Eunice Kennedy Shriver* National Institute of Child Health and Human Development — NICHD
- National Cancer Institute — NCI
- National Heart, Lung and Blood Institute — NHLBI
- National Human Genome Research Institute — NHGRI

Other NIH Institutes and Centers, and HHS agencies, that are a part of the Working Group:
NCATS, NIDCR, NIDCD, NIDA, NIAAA, NIDDK, NIEHS, NEI, NIAMS, NIAID, ORIP and the CDC

NIH Common Fund Programs

A DECADE OF DISCOVERY — NIH COMMON FUND 2004-2014 — 10 Years

The Common Fund is a unique resource at NIH, functioning as a "venture capital" space where high-risk, innovative endeavors with the potential for extraordinary impact can be supported. The CF programs, such as the Kids First Program, are short-term, goal-driven strategic investments, with deliverables intended to catalyze research across multiple biomedical research disciplines.

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Sign up for the Kids First list-serv:
https://commonfund.nih.gov/kidsfirst/register

Kids First Program: https://commonfund.nih.gov/KidsFirst

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Major Initiatives

1. Cohort Identification & DNA Sequencing

Cohorts of children with childhood cancer and/or structural birth defects, and their families, were selected for whole genome sequencing provided by the Kids First Sequencing Centers after undergoing a peer-reviewed process.

Disease Areas

- Adolescent Idiopathic Scoliosis
- Cancer Susceptibility
- Congenital Diaphragmatic Hernia
- Craniofacial Microsomia
- Disorders of Sex Development
- Enchondromatoses
- Ewing Sarcoma
- Familial Leukemia
- Hearing Loss
- Infantile Hemangiomas
- Neuroblastomas
- Nonsyndromic Craniosynostosis
- Orofacial Clefts
- Osteosarcoma
- Patients with both childhood cancer and birth defects
- Structural Heart & Other Defects
- Syndromic Cranial Dysinnervation Disorders

Kids First Sequencing Centers

- HudsonAlpha Institute for Biotechnology & St. Jude Children's Research Hospital
- The Broad Institute of MIT & Harvard
- Baylor College of Medicine Human Genome Sequencing Center
- McDonnell Genome Institute at Washington University

2. Kids First Data Resource

The Kids First Data Resource Center (DRC) will create a resource which will serve as a centralized database to store and integrate genomic data from childhood cancer and structural birth defects patients and their families. Additionally, the Data Resource Portal will allow researchers to instantly search large genomic datasets using new data visualization tools and cloud-based data-sharing platforms. Researchers everywhere will be able to identify genetic pathways that underlie the biological causes of childhood cancer and structural birth defects. These new pathways may help researchers discover novel and improved treatments for children diagnosed with childhood cancer or structural birth defects.

The DRC is charged with re-processing and "harmonizing" data generated by the sequencing centers, as well as clinical and phenotypic data to facilitate analyses across all Kids First datasets.

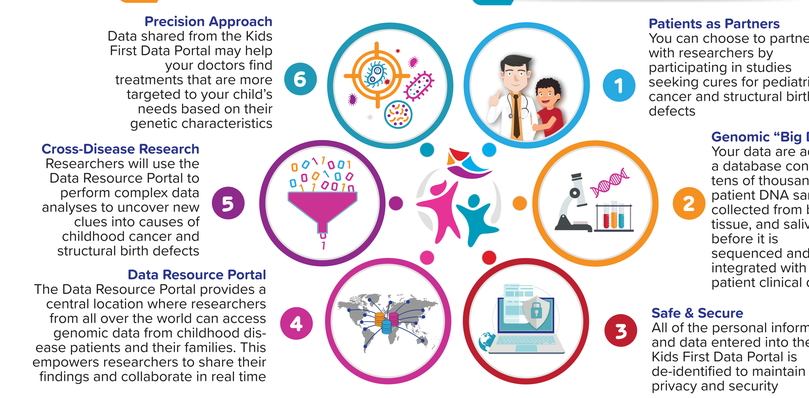
Kids First DRC Member Institutions

- Center for Data Driven Discovery in Biomedicine at Children's Hospital of Philadelphia
- Oregon Health and Science University
- Children's National Health System
- University of Chicago
- Ontario Institute for Cancer Research
- Seven Bridges Genomics, Inc.

Data Resource Portal
- Web-based, public-facing platform
- Designed to house, organize, index,

Data Coordinating Center
- Facilitate the deposition of sequence and phenotype data into relevant repositories
- Harmonize phenotypes

Administrative & Outreach Core
- Develop policies & procedures
- Facilitate meetings & communication
- Educate and seek feedback from users
- Reach out to advocacy groups



3. Data Analysis: Data Mining & Demonstration Projects

In the future, Kids First intends to support Data Mining & Demonstration Projects for analysis of Kids First-generated and other pediatric data to uncover new insights into the biology of childhood cancer and structural birth defects, including the discovery of shared genetic pathways between childhood cancer and structural birth defects.