

The Gabriella Miller Kids First Pediatric Research Program



*National Advisory Child Health and Human Development Council
June 7th, 2023*

James Coulombe Ph.D., Chief DBCAB, Kids First Working Group Coordinator

Kids First Started with Gabriella Miller's Pediatric Cancer Advocacy Empowering Research Across Pediatric Conditions



Oct 2013

Gabriella Miller childhood cancer advocate, died at age 10 from an aggressive brain cancer

April 2014

Gabriella Miller Kids First Research Act authorizes \$12.6 million/year for 10 years to NIH for pediatric research

Sept 2015


NIH Kids First Program first annual appropriation. The program is funded through FY 2024




The Kids First Working Group - a Collaboration to Accelerate Discoveries in Pediatric Research


KIDS FIRST WORKING GROUP

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

 Eunice Kennedy Shriver National Institute of Child Health and Human Development

 National Heart, Lung and Blood Institute

 NATIONAL CANCER INSTITUTE

 National Human Genome Research Institute

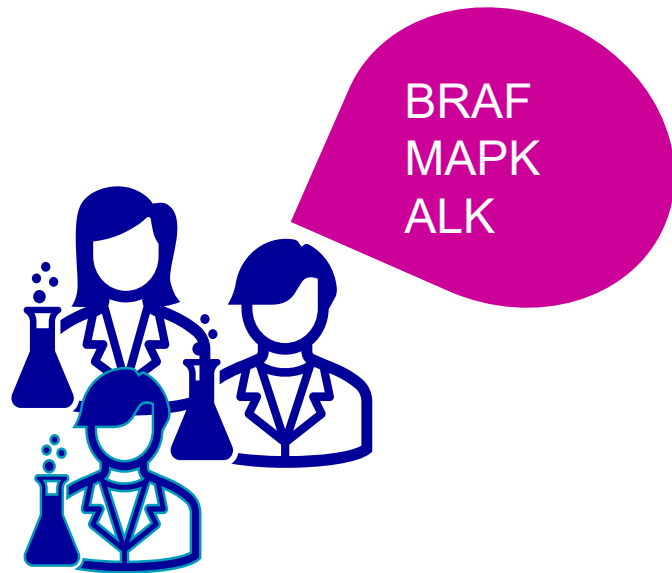
NIDCR	NINDS	NEI	NCATS
NIDA	NIDDK	NIAMS	ORIP
NIAAA	NIEHS	NIAID	CDC

The Kids First Working Group is charged with building a cloud-based genomic data resource to share data and accelerate collaborative research leading better prevention, diagnosis, and treatments for patients and families with pediatric cancer and structural birth defects



Association Between Structural Birth Defects and Childhood Cancer

Shared mutations and drug targets across pediatric conditions



Cancer risk increased among children with birth defects:

Original Investigation

FREE

June 20, 2019

Association Between Birth Defects and Cancer Risk Among Children and Adolescents in a Population-Based Assessment of 10 Million Live Births

Philip J. Lupo, PhD^{1,2}; Jeremy

[» Author Affiliations](#) | [Article](#)

JAMA Oncol. 2019;5(8):1150-

RESEARCH

Cancer risk in individuals with major birth defects: large Nordic population based case-control study among children, adolescents, and adults

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Gabriella Miller
INUS First
PEDIATRIC RESEARCH PROGRAM

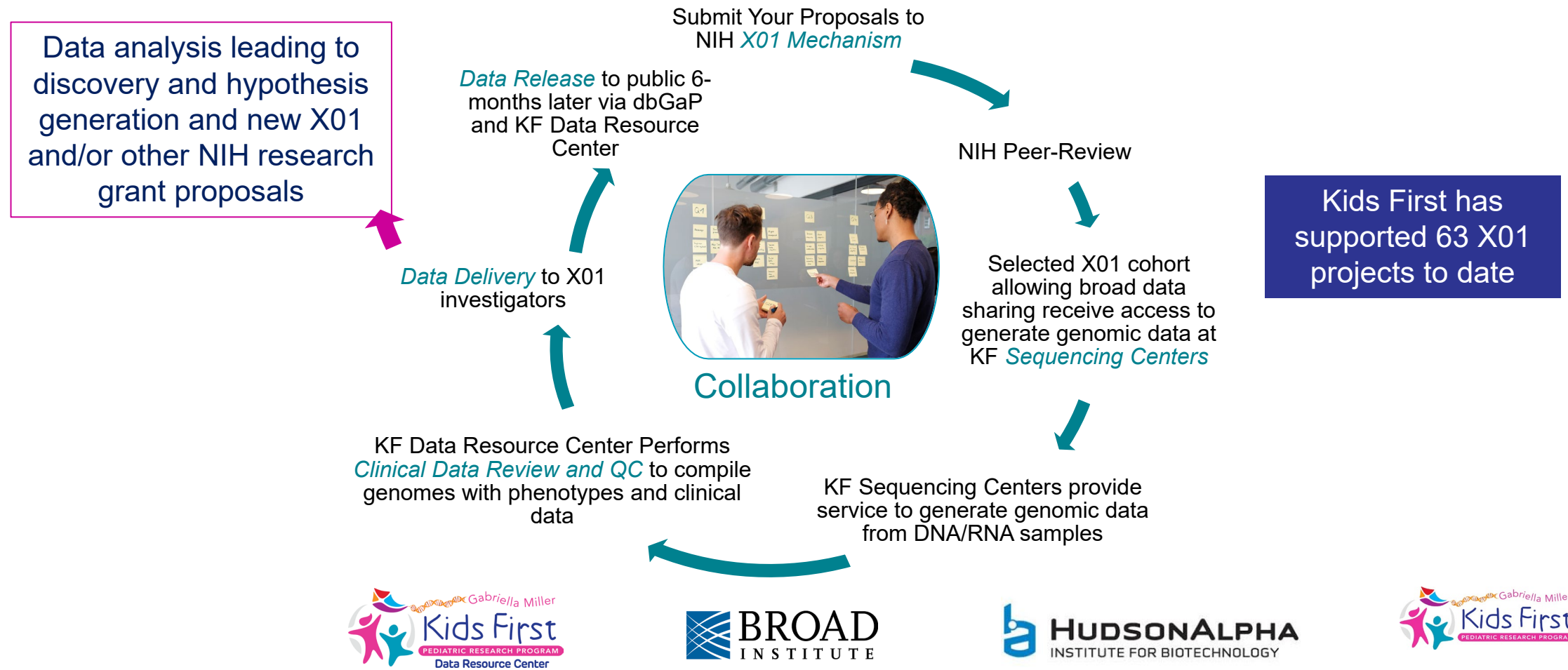


Kids First Vision

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



Kids First Empowered by X01 Mechanism, Genome Sequencing Centers, and Data Resource Center. Starts with Patients Cohorts and Investigator Proposals



Kids First's Cloud-Based Platforms



Kids First Data Resource Portal - portal.kidsfirstdrc.org

EXPLORE datasets and build cohorts of participants

DISCOVER harmonized genomic data files for further research

CONNECT data from multiple Kids First studies



CAVATICA - cavatica.sbgenomics.com

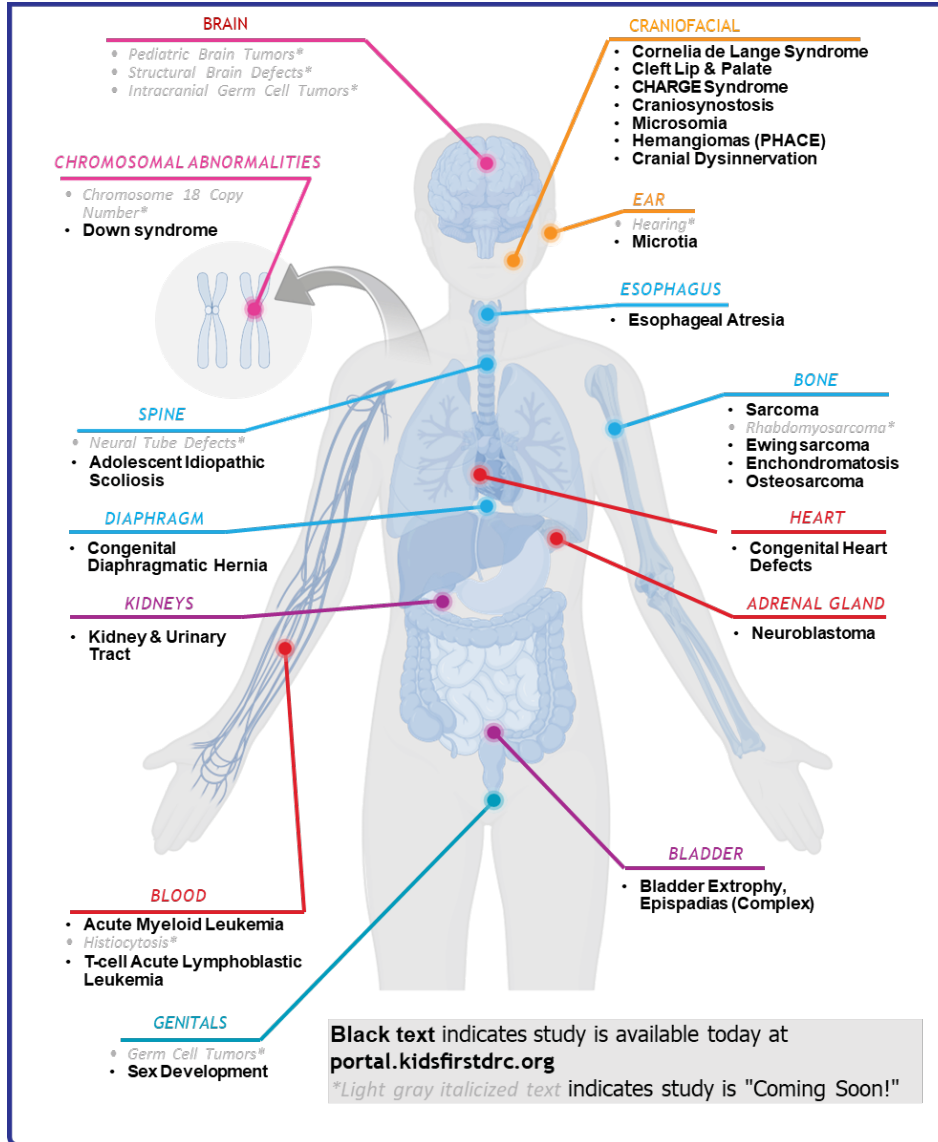
COMPUTE large scale workflows on genomic data files

ANALYZE data in the cloud via R Studio and Python Notebooks

SHARE tasks and findings with collaborators around the world



Kids First Data Available Today



500+ users approved for 2nd use of data

29 studies at dbGaP

Get started at <https://portal.kidsfirstdrc.org>



Harmonized data/ 22 birth defect and 15 cancer cohorts/ >28,500 participants

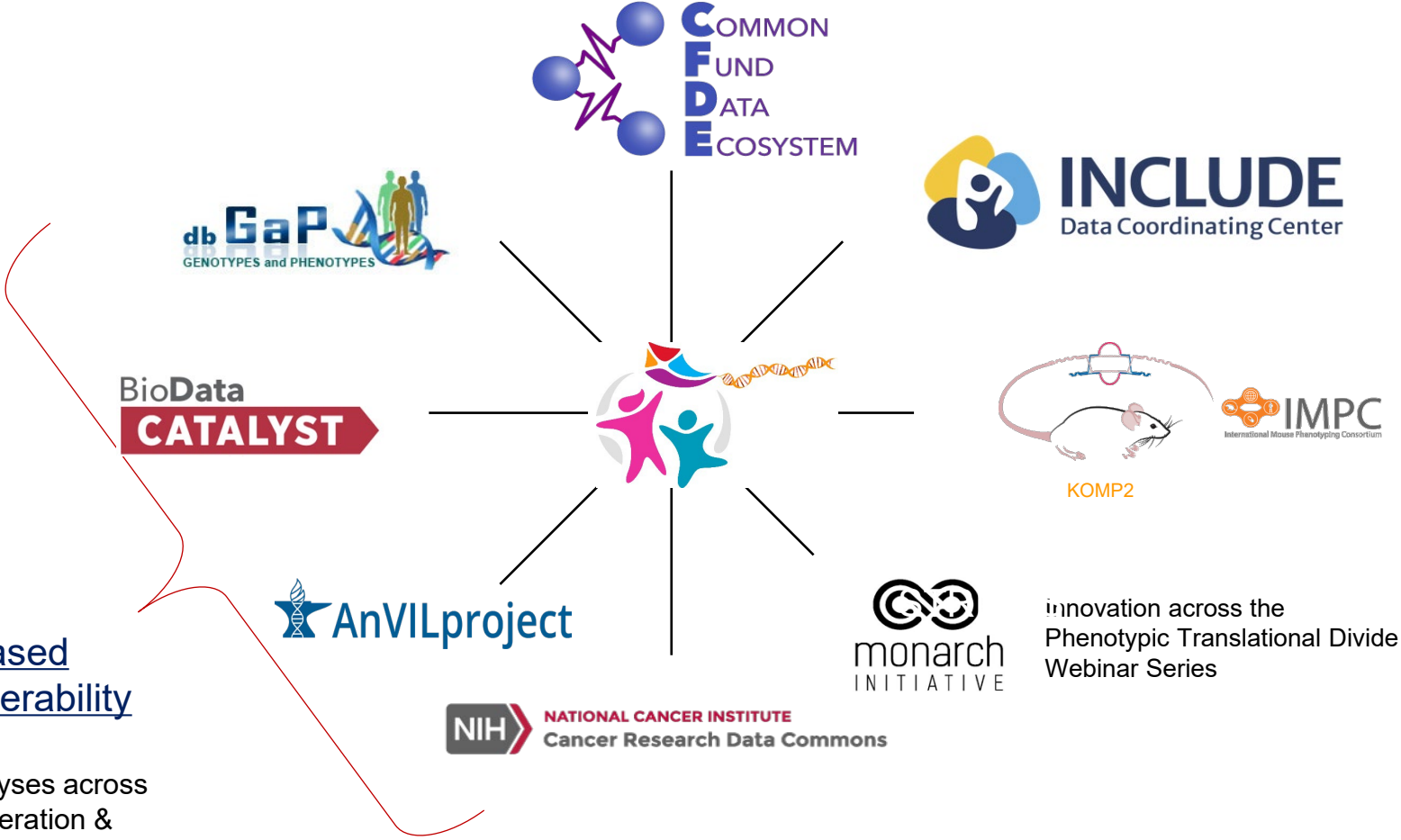


Kids First Data Resource Shares Multiple Data Types Across Pediatric Conditions

- Kids First Data Resource Center allows for multiple data types and cross disease associations
 - better view of the condition's underlying biology
 - knowledge of common biology across pediatric conditions can speed up the development of diagnostics, treatments, and prevention tools
- Kids First commitment to sharing genomic data associated with clinical data empowers genetic variant discovery
 - within a single disease area or dataset
 - across diagnoses and cohorts
 - allow users to explore similar underlying causes of cancers, structural birth defects, and other rare disorders
- Individual level genomic data are available through dbGaP data access request.



Kids First is Part of a Larger Data Ecosystem



NIH Cloud-Based Platforms Interoperability (NCPI):

Empower end-user analyses across platforms through federation & interoperability



Kids First Data & the Data Resource Portal

ABOUT THE DATA



22
Birth defect
cohorts



15
Cancer
cohorts



32,000+
Study
participants



192,000+
Data files
available

ABOUT THE PORTAL



50
Unique
countries
represented by
portal users



500+
Approved access
requests for
secondary data
use



3,389+
Total portal
users



6,500+
Total portal
logins in
2022



Acknowledgements

Kids First Working Group:

Director co-Chairs: NICHD, NCI, NHLBI, NHGRI

Other Working Group Representation:

NIDCR	NIAAA	NIDDK	NEI	NIAID	ORIP
NIDA	NINDS	NIEHS	NIAMS	NCATS	CDC



Data Resource Center:



VELSERA



Sequencing Centers:

