Gabriella Miller Kids First
Pediatric Research Program

NICHD Council
September 14, 2017
Bethesda, MD
Background

Initiated in response to the 2014 Gabriella Miller Kids First Research Act

- Signed into law on April 3, 2014
- Ended taxpayer contribution to presidential nominating conventions
- Transferred $126 million into a Pediatric Research Initiative Fund
- Authorized appropriation of $12.6 million per year for 10 years to the NIH Common Fund for pediatric research; first appropriation was for FY2015
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 Working Group
Institute Director Co-chairs

Diana W. Bianchi, M.D.,
Director, Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD)

Lawrence Brody, Ph.D.,
Division Director, Division of Genomics and Society, National Human Genome Research Institute (NHGRI)

Gary H. Gibbons, M.D.,
Director, National Heart, Lung, and Blood Institute (NHLBI)

Douglas Lowy, M.D.,
Acting Director, National Cancer Institute (NCI)
Kids First Leadership Team

Program Officers

- Lorette Javois (NICHD)
  Working Group Coordinator
  Sequencing Centers Project Officer

- Jonathan Kaltman (NHLBI)

- Malcolm Smith (NCI)

- Adam Felsenfeld (NHGRI)

- Jaime Guidry Auvil (NCI)
  Sequencing Center Project Scientist

- James Coulombe (NICHD)
  Sequencing Center Project Scientist

- Maarten Leerkes (NHLBI)
  Data Resource Project Scientist

- Charlene Schramm (NHLBI)
  Data Resource Program Officer

Admin Point-of-Contact

- Valerie Cotton (NICHD)

Common Fund, OD

- Marie Nierras – Program
- Danyelle Winchester – Policy, Planning, Evaluation, and Communications
- Michael Steenstra – Operations and Budget

Grants Management

- Bonnie Jackson (NICHD)
- Tracee Foster (NHLBI)

Other participants: NIDCR, NIDCD, NIDA, NIAAA, NIDDK, NIEHS, NEI, NIAMS, NIAID, ORIP, and the CDC
**Kids First Major Initiatives**

1. Cohort identification and DNA sequencing
   - Identify children with childhood cancer and/or structural birth defects, and their families, [PAR-15-259](#); [PAR-16-150](#); [PAR-17-063](#); [next FY18 cycle]
   - Whole genome sequencing by the Kids First Sequencing Centers, [RFA-RM-16-001](#)

2. Gabriella Miller Kids First Pediatric Data Resource, [RFA-RM-16-010](#)
   - Will greatly aid researchers in identifying DNA changes that cause or contribute to childhood cancer and/or structural birth defects
   - Will provide researchers with necessary computational infrastructure and analysis tools to analyze large and complex data sets

3. Data Analysis: Data Mining & Demonstration Projects [future]
   - Support analysis of Kids First-generated and non-Kids First-generated 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
Kids First Sequencing Centers

HudsonAlpha Institute for Biotechnology
• Shawn Levy

St. Jude Children’s Research Hospital
• Jinghui Zhang
• John Easton

Broad Institute of MIT & Harvard
• Stacey Gabriel
• Michael Talkowski
• Daniel MacArthur
History of Sequencing Activity

HG Admin Supplements
Baylor and Washington University

FY15
- 17 X01s submitted
- 7 selected
- ~21,880 genomes requested
- 5,990 into pipeline
- $12.6M

RFA-RM-16-001 Kids First Sequencing Centers
Broad and HudsonAlpha

FY16
- 15 X01s submitted
- 8 selected
- ~12,000 genomes requested
- 6,834 into pipeline
- $12.6M

FY17
- 15 X01s submitted
- 8 selected
- ~18,890 genomes requested
- 5,200 into pipeline
- $~9.5M
52,770 genomes proposed (47 cohorts)
- 18,024 genomes selected (23 cohorts)
34,746 genomes left unsequenced
Cohorts Selected for Sequencing

Adolescent Idiopathic Scoliosis (FY16)
Cancer Susceptibility (FY16)
Congenital Diaphragmatic Hernia (FY15, 16, 17)
Craniofacial Microsomia (FY17)
Disorders of Sex Development (FY15)
Enchondromatases (FY17)
Ewing Sarcoma (FY15, 17)
Familial Leukemia (FY16)
Hearing Loss (FY16)
Infantile Hemangiomas (FY17)
Neuroblastomas (FY16)
Nonsyndromic Craniosynostosis (FY17)
Orofacial Clefts; Caucasian (FY15), Latin American (FY16), Asian & African (FY17)
Osteosarcoma (FY15)
Patients with both childhood cancer and birth defects (FY17)
Structural Heart & Other Defects (FY15, 16)
Syndromic Cranial Dysinnervation Disorders (FY15)
Data Resource Center

Data Resource Portal
- Web-based, public facing platform
- House, organize, index, and display data and analytic tools

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

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

Children’s Hospital of Philadelphia
Center for Data Driven Discovery in Biomedicine (D3b)
Adam Resnick, PI

Commitment Partners

- Seven Bridges Genomics, Inc.
- Ontario Institute for Cancer Research
- University of Chicago
- Oregon Health and Science University
- Children’s National Health System
Governance

External Scientific Advisors

NIH Working Group

Data Resource Center

Steering Committee

Sequencing Centers

X01 Investigators
Small Research Grants for Analyses of Data for the Gabriella Miller Kids First Data Resource (R03)
PAR-16-348

• NICHD, NCI, NHLBI, NIAAA, NIDCR, and NINDS

• Standard R03 receipt dates

• Combined direct cost budget for the two-year project period may not exceed $200,000
GMKF Major Initiatives & Budget

Activities and Timeline

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<td>Cohort ID (X01) &amp; Sequencing (Admin Suppl &amp; U24s)</td>
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<td>New Insights from Data Mining/Demo Projects</td>
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