Gabriella Miller Kids First Pediatric Research Program

Council of Councils Update

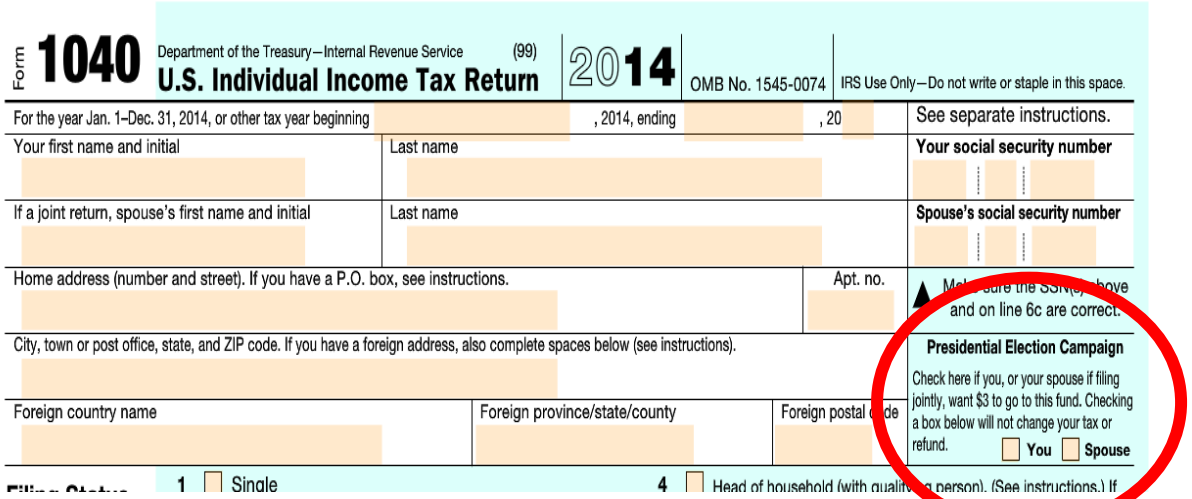
September 1, 2017

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



Form **1040** Department of the Treasury—Internal Revenue Service (99) **U.S. Individual Income Tax Return** 2014 OMB No. 1545-0074 IRS Use Only—Do not write or staple in this space.

For the year Jan. 1–Dec. 31, 2014, or other tax year beginning , 2014, ending , 20 See separate instructions.

Your first name and initial | Last name | **Your social security number**

If a joint return, spouse's first name and initial | Last name | **Spouse's social security number**

Home address (number and street). If you have a P.O. box, see instructions. | Apt. no.

▲ Make sure the SSN(s) above and on line 6c are correct.

City, town or post office, state, and ZIP code. If you have a foreign address, also complete spaces below (see instructions).

Foreign country name | Foreign province/state/county | Foreign postal code

Presidential Election Campaign

Check here if you, or your spouse if filing jointly, want $3 to go to this fund. Checking a box below will not change your tax or refund. ☐ You ☐ Spouse

Filing Status 1 ☐ Single 4 ☐ Head of household (with qualifying person). (See instructions.) If

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

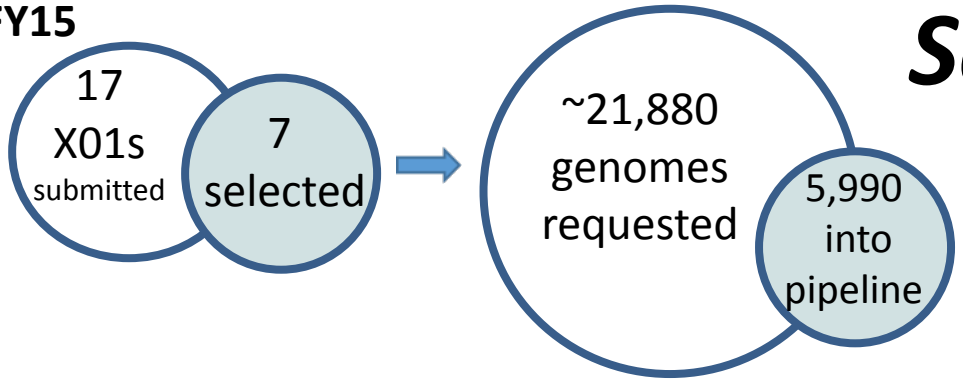
Broad Institute of MIT & Harvard

- Stacey Gabriel
- Michael Talkowski
- Daniel MacArthur

History of Sequencing Activity

HG Admin Supplements
Baylor and Washington University

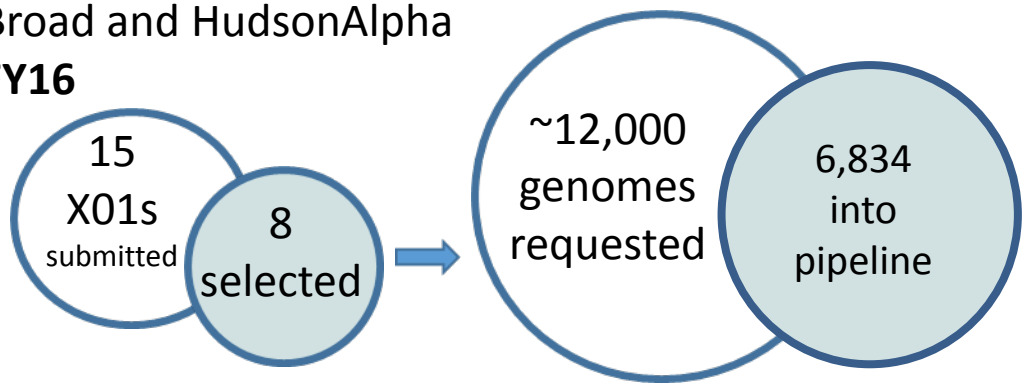
FY15



$12.6M

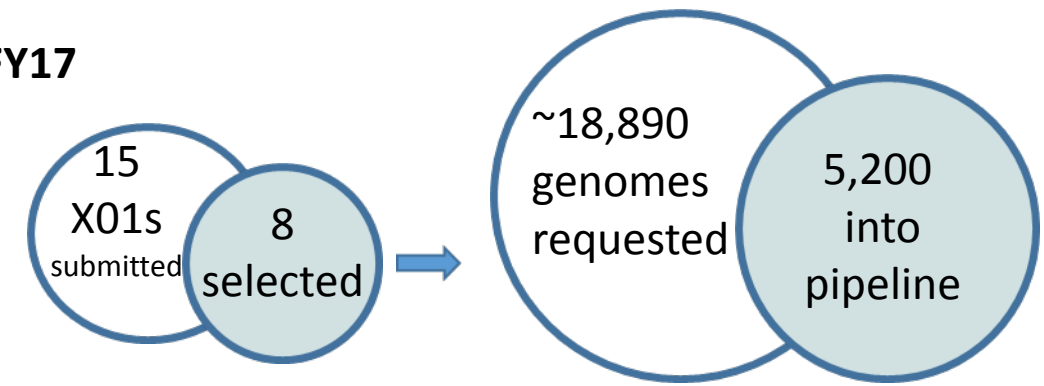
RFA-RM-16-001 Kids First Sequencing Centers
Broad and HudsonAlpha

FY16



$12.6M

FY17



$~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)
Enchondromatoses (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

Seven Bridges Genomics, Inc.

Ontario Institute for Cancer Research

University of Chicago

regon Health and Science University

Children's National Health System

Commitment Partners

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