DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Office of the Director

Report of the Director’s Discretionary Fund
Second Quarter of FY 2017

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Director, NIH
Report of the Director’s Discretionary Fund

Introduction
The report accompanying the fiscal year (FY) 2017 Labor, Health and Human Services, Education and Related Agencies House bill stated the following:

“The Committee continues the bill language for specific funds authorized by the Gabriella Miller Kids First Research Act within the CF to support the third year of the 10-year Pediatric Research Initiative. The Committee urges the Director to use a portion of the $10,000,000 made available to the Director’s Discretionary Fund (DDF) to support additional pediatric research. The Committee requests, within 30 days after the end of each fiscal year quarter, a quarterly report on DDF obligations for each activity supported. The report should include a description of the program, which ICs are to provide continuation costs, and how this research serves a high priority for pediatric diseases. The quarterly reports shall be posted on-line via the NIH web-site within 30 days after being released to the Committee.”

The following report of obligations for the second quarter of FY 2017 has been prepared by the National Institutes of Health (NIH), part of the Department of Health and Human Services, in response to this request. As of the end of the second quarter, obligations totaling $394,000 have been applied to the FY 2017 Director’s Discretionary Fund (DDF). The balance of the FY 2017 DDF funding will be obligated during the third and fourth quarters.

Background
The Director’s Discretionary Fund (DDF) is used annually to enable NIH to address high-priority research opportunities and respond to new scientific issues, including through the development of improved management, planning, and analytical tools.

## Director’s Discretionary Fund

**FY 2017**

**Second Quarter Obligations**

(Dollars in Thousands)

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**Total Approvals and Obligations as of 3-31-17:** $394

**Projects Supported by FY 2017 DDF as of the Second Quarter**

**Bench-to-Bedside Award**
The Bench-to-Bedside (BtB) Program funds research teams seeking to translate basic scientific findings into therapeutic interventions for patients and to increase understanding of important disease processes. Funds were used to support the following five one-year Bench-to-Bedside awards: (1) Study of Combination HIV-Specific Antibodies in Infected Individuals; (2) Microbiome and Uveitis; (3) Therapeutic Targets in African Americans with Primary
Aldosteronism, (4) Role of Mutant STAT3 in Airway Epithelium Mucus and Host Defense; and (5) Investigations of Juvenile Neuronal Ceroid Lipofuscinosis (CLN3).

The latter two awards listed above include a pediatric research focus as follows: The Role of Mutant STAT3 in Airway Epithelium Mucus and Host Defense project focuses on the rare primary immunodeficiency disease called Hyper IgE Syndrome (HIES) which is associated with heterozygous mutations in the transcription factor STAT3. HIES occurs in both males and females of all ethnic groups, and symptoms can start within the first month of life with poor prognosis for most affected individuals who may not reach adulthood if untreated. Often deaths occur in the second and third decades of life due to severe pulmonary infections; this is the focus of this study.

The Investigations of Juvenile Neuronal Ceroid Lipofuscinosis (CLN3) project supports pediatric research because CLN3 is a recessive, fatal, lysosomal storage disease that results in progressive neurodegeneration. Neurological symptoms typically manifest between 4 and 7 years of age. The goal of the project is to establish a biorepository of samples from CLN3 pediatric patients in order to identify and characterize biomarkers, and to evaluate clinical aspects of CLN3 in order to provide tools for future therapeutic trials.

Chief Officer for Scientific Workforce Diversity (COSWD) Intramural Laboratory One-Time Costs
Dr. Hannah A. Valantine is the Chief Officer for Scientific Workforce Diversity in the Office of the Director, NIH. Dr. Valantine’s Intramural Research Program is housed within a laboratory at the National Heart, Lung, and Blood Institute (NHLBI). Dr. Valantine utilizes various genomics tools to study outcomes related to transplant rejection. These studies include large scale next generations sequencing assays and clinical studies. The clinical studies involved patient recruitment and enrollment at five transplant centers supported by the NIH intramural program. These centers engage subjects and collect bio specimens needed to study transplant outcomes.

Accelerating Cures for Inherited Hemoglobinopathies Working Group Meeting
In March 2017, NHLBI convened a working group comprised of experts in the field of basic and translational science focused on sickle cell disease and thalassemia to discuss a possible “moonshot” for curing hemoglobinopathies utilizing recent advances in genetic and molecular technology. These genetic hemoglobinopathies are prime candidates for such a concerted research effort, particularly in light of the profound global mortality and morbidity that sickle cell disease and thalassemia engender. Funds were obligated to support the travel costs for 15 experts to participate in this meeting to discuss the feasibility of inaugurating a targeted program to potentially jump-start such an effort.

In addition to the projects described above, the NIH Director and Principal Deputy Director are currently in discussions of how best to strategically support pediatric research programs, and aim to finalize the plans in the third quarter of FY 2017.