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# NIH-funded Pediatric Myelodysplastic Syndrome (MDS) and Bone Marrow Failure (BMF) Disorder Patient Registry at Children's Hospital Boston

## Data Sharing Agreement

**NIH funding information:**
FOA: OD09-003 entitled, "Recovery Act Limited Competition: NIH Challenge Grants in Health and Science Research"
Funding Institution: NIH/NIDDK
Grant # 1RCDK086861-01
Multiple PI: David Williams
Mark Fleming

**Investigators:** David Williams, MD
Mark Fleming, DPhil, MD
Inga Hofmann, MD

**Research Specimen and Patient Registry Data Sharing Agreement**

**Goals of the registry:**

The overall goal of the pediatric MDS and BMF disorder patient registry and tissue bank is to systematically evaluate pediatric patients with MDS, inherited and acquired bone marrow failure disorders in collaboration with other Pediatric Hematology centers.

The ultimate goal is to define the genetics and underlying biology of childhood MDS and BMF disorders, identify pathways for therapy, and ultimately translate this knowledge to improve the outcome for these children.

Specifically we seek to:

1. To improve the accuracy of the diagnosis for children and young adults with MDS and BMF disorders by a standardized review of morphology and standardized cytogenetics and molecular analysis.
2. To evaluate the frequency of the different subtypes of MDS and BMF disorders by using a standardized diagnostic approach.
3. To assess disease progression or evolution, survival, relapse rate, morbidity and mortality in children with MDS and BMF disorders.
4. Evaluate new diagnostic tools such as flow cytometry and molecular testing in the utility of improving the diagnosis of pediatric MDS and BMF disorders.
5. Build a large patient repository and tissue bank of patients with these rare disorders to allow us to systematically analyze these patients in the future.

The NIH-funded Pediatric MDS and BMF disorder registry will be overseen by a Steering Committee composed of the following members:

- David A. Williams, MD (Pediatric Hematology/Oncology)
- Mark D. Fleming, DPhil, MD (Pediatric Hematopathology)
- Inga Hofmann, MD (Pediatric Hematology/Oncology and Hematopathology)
- Kevin Shannon, MD (external advisor-University of California San Francisco)
- Charlotte Niemeyer, MD (external consultant-Albert-Ludwigs University, Freiburg, Germany)
- William Woods, MD (external advisor-Children's Healthcare of Atlanta at Egleston, Emory University School of Medicine)

**Collaborations:**

Meaningful scientific research on rare disorders can only advance with the collaboration of multiple centers. The goal of the Pediatric MDS and BMF disorder registry is to collaborate with other centers to develop NIH funded disease-specific registry of rare pediatric MDS and BMF (pre-MDS) patients and to ultimately advance the scientific knowledge in the field with the hope to discover new therapeutic targets for therapy.

CHB will serve as the coordinating study center for these efforts and provide both infrastructure and technical support. Data collected at CHB and at participating centers will be entered into a web-based Pediatric MDS and BMF disorder database.

Each participating institution will obtain local IRB approval to participate in the NIH-funded Pediatric MDS and BMF disorder registry at CHB. The IRB protocol developed and approved at CHB will be made available to any other institutions interested in participating for expedited IRB review at the local institution.

In the future, as more centers participate in this multi-center study, we will expand the Steering Committee to include other disease specific investigators from each region of the country where possible.

Benefits of participating in the NIH-funded Pediatric MDS and BMF disorder registry at CHB:

1. Second opinion pathology review by an expert pediatric hematopathologist of any diagnostic and follow up material for patients with known or suspected/potential MDS or BMF disorder (without charge).
2. Expert flow cytometry analysis of patients with suspected or known MDS (without charge).
3. Ability to store patient data in state of the art database maintained by CHB Clinical Research Program staff at no cost.
4. Storage of patient research samples in a state of the art storage facility with computerized specimen inventory tracking at CHB at no cost.
5. Free marketing and patient recruitment materials.

**Collaborators Rights:**

Research data collected or maintained by the NIH-funded Pediatric MDS and BMF disorder registry at CHB will be made available as shared data for research purposes.

Investigators will have access to data and samples from the registry after completing a research request. The research data and sample request will be reviewed by the Steering Committee of the Pediatric MDS and BMF disorder registry. Investigators participating in the registry will have preferred rights to any outside investigators.

**Publication of study results and authorship:**

Any formal presentation or publication of data collected as a direct or indirect result of this trial will be considered as a joint publication by the investigators. The Steering Committee will utilize the International Committee of Medical Journal Editors ("ICMJE") customary standards of authorship applicable to publishing journals to determine final inclusion, contributorship and authorship. The contributions could be based on the number of patients enrolled in the study as well as scientific contributions or analysis of data.

**Financial Compensation**

Participating centers will be paid $250 per eligible patient enrolled in the study and entered into the research database. The provided funding support is intended to help offset cost such as time and percent effort of clinical research coordinators and staff to enter data into the database or case report forms. We will reimburse each institution on a quarterly basis. All other potential costs such as collection tubes, shipping material and FedEx costs will be provided and covered by the coordinating study center at Children's Hospital Boston.

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Date Signature of Investigator at Collaborating Institution