

The Pediatric Myelodysplastic Syndrome and Bone Marrow Failure Disorder Patient Registry

About the Registry

Funded by the National Institutes of Health, this centralized patient registry and tissue repository will improve the understanding and treatment of all pediatric myelodysplastic syndromes (MDS) and bone marrow failure (BMF) disorders. Our objectives are to:

- Improve diagnostic accuracy of these rare conditions by using a standardized diagnostic approach
- Build a tissue repository and database that allows for meaningful compilation of clinical outcomes to facilitate research
- Use new knowledge gained from these observations to develop and recommend new treatments
- Gain new insights into the causes of MDS and BMF

Who is Eligible?

Patients must be <35 years old and have a suspected or known diagnosis of primary MDS or one of the following diagnoses:

- Suspected or known diagnosis of primary or secondary MDS
- Suspected or known diagnosis of Myeloproliferative Disorders (MPD)
- Suspected or known diagnosis of Myelodysplastic/Myeloproliferative Neoplasms
- Suspected or known inherited Bone Marrow Failure Disorders (i.e. Fanconi Anemia, Dyskeratosis Congenita, Diamond-Blackfan Anemia, amongst others)
- Acquired Severe Aplastic Anemia and Very Severe Aplastic Anemia

What Information is Needed to Join?

As part of their work up, every patient will undergo a routine clinical evaluation and diagnostic procedures, including lab tests and a bone marrow aspirate and biopsy. No additional procedures will be performed for the study. The following information will be obtained:

- Patient demographics
- Clinical history
- Peripheral blood, bone marrow, and germ line tissue (buccal mucosa swabs or skin biopsies for fibroblasts)
- Ancillary information such as cytogenetics and molecular testing

In regular time intervals (6–12 months), follow up information will be requested.

How to Refer a Patient

Contact our coordinating research nurse Grace Yoon, NNP, MSN, and the principal investigator Inga Hofmann, MD, PhD, by emailing mds@childrens.harvard.edu or calling 617-355-9148.



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