MDS The National Myelodysplastic Syndromes Natural History Study

Sponsored by the National Heart, Lung, and Blood Institute in collaboration with the National Cancer Institute

The National Myelodysplastic Syndromes (MDS) Study

Recruiting patients with low blood counts undergoing a bone marrow assessment for evaluation of MDS

Who Is Eligible?

The NHLBI-MDS study is open to patients with a cytopenia that does not have another explainable cause and for whom you are planning to do a bone marrow assessment to evaluate for myelodysplastic syndromes (MDS) or to confirm a diagnosis received within the 12 months prior to treatment.

Why Participate?

The study will help investigators design high-impact studies to benefit MDS patients. Here's how:

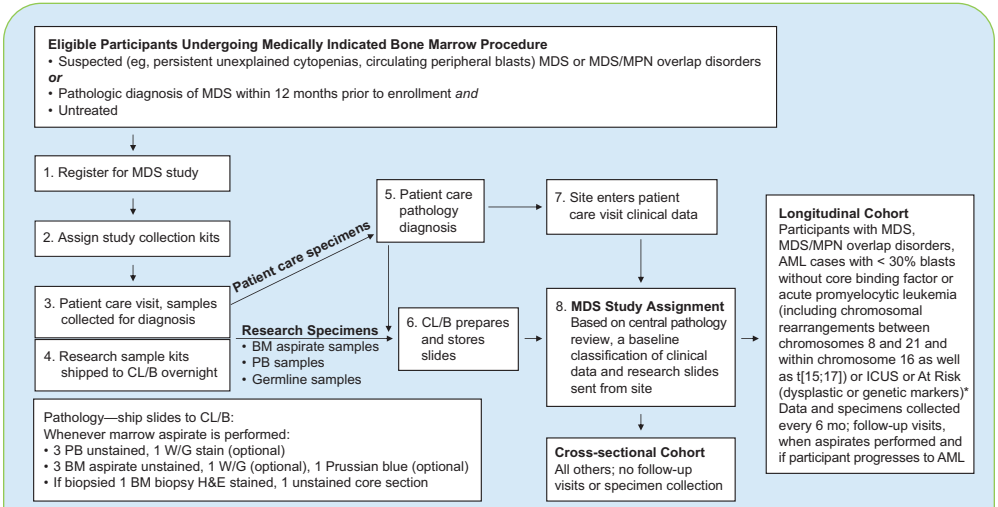
- Blood and tissue samples will be collected prospectively from patients with suspected MDS and those with idiopathic cytopenia of undetermined significance (ICUS) or at risk (dysplastic or with selected genetic markers)
- This biorepository will be linked to a database capturing critical clinical information, such as patient history (including environmental exposures), presenting symptoms, and test results

What type of studies will this research support?

This high-quality, publicly available resource will enable studies to:

- Improve diagnostic accuracy and medical decision-making in MDS
- Understand quality of life and its relationship to changing disease and treatment status
- Illuminate the pathogenesis of MDS and its genetic, epigenetic, and immunologic mechanisms
- Optimize treatment strategies for specific MDS subtypes
- Identify novel biomarkers for MDS outcomes
- Discover novel targets for therapeutic interventions

Study Schema



*Cases with local or central pathology assessments of dysplasia in baseline bone marrow aspirate; select karyotype abnormalities; locally or centrally detected genetic mutations meeting minimally acceptable criteria for allelic variant presence.
AML = acute myeloid leukemia; BM = bone marrow; CL/B = central laboratory/biorepository; H&E = hematoxylin and eosin; ICUS = idiopathic cytopenia of undetermined significance; MDS = myelodysplastic syndrome; MPN = myeloproliferative neoplasm; PB = peripheral blood; W/G = Wright-Giemsa.

Eligibility Criteria*

Main Inclusion Criteria

- ≥ 18 years of age
- Suspected MDS or MDS/myeloproliferative neoplasm (MPN) overlap disorders and undergoing diagnostic work-up with planned bone marrow assessments ***or***
- Diagnosed with de novo or therapy-related MDS within 12 months of enrollment per the World Health Organization (WHO) criteria and undergoing clinical evaluation and planned bone marrow assessment to confirm MDS or to evaluate disease status
- Bone marrow aspirate expected to be performed within 1 week of registration and, in all cases, must be performed no later than 4 weeks after enrollment
- If anemic without prior MDS diagnosis, the following tests must be completed within the prior 6 months: B_{12} level, serum folate, mean corpuscular volume (MCV), red cell distribution width (RDW), ferritin, and iron studies. Values significantly outside normal range do not exclude participation but should prompt investigation of alternative etiologies for anemia

Main Exclusion Criteria

- Prior treatment for MDS at entry and through time of entry bone marrow aspirate
- Treatment with hematopoietic growth factors in prior 6 months
- Diagnosis of a solid tumor or hematologic malignancy within 2 years prior to enrollment, except for in situ cancer of the skin (basal or squamous cell), cervix, bladder, breast, or prostate

*When evaluating patients for this study, please refer to the full protocol for the complete list of eligibility criteria. Additional eligibility criteria will apply to patients entering the intensification or randomization steps.

Main Exclusion Criteria (Cont)

- Treatment with radiation therapy 2 years prior to registration
- Nonhormonal treatment for malignancy 2 years prior to registration
- Established hereditary bone marrow failure syndrome
- Known primary diagnosis of aplastic anemia, classical paroxysmal nocturnal hemoglobinuria, amegakaryocytic thrombocytopenic purpura, or large granular lymphocytic leukemia
- Not enrolled in the Connect® MDS/AML Disease Registry

This NHLBI-sponsored protocol is OPEN across the Adult Network Groups.

It is the largest-ever prospective study of MDS in the US, with a goal of up to 2000 patients.

Additional Notes

The NHLBI-MDS protocol is NCI CIRB approved, and funding is available to participating sites for each enrollment, via either DCP (for NCORP sites) or capitation (for non-NCORP sites).

NIH National Heart, Lung, and Blood Institute

NCI Community Oncology Research Program

ECOG-ACRIN cancer research group