# The National Myelodysplastic Syndromes (MDS) Study

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

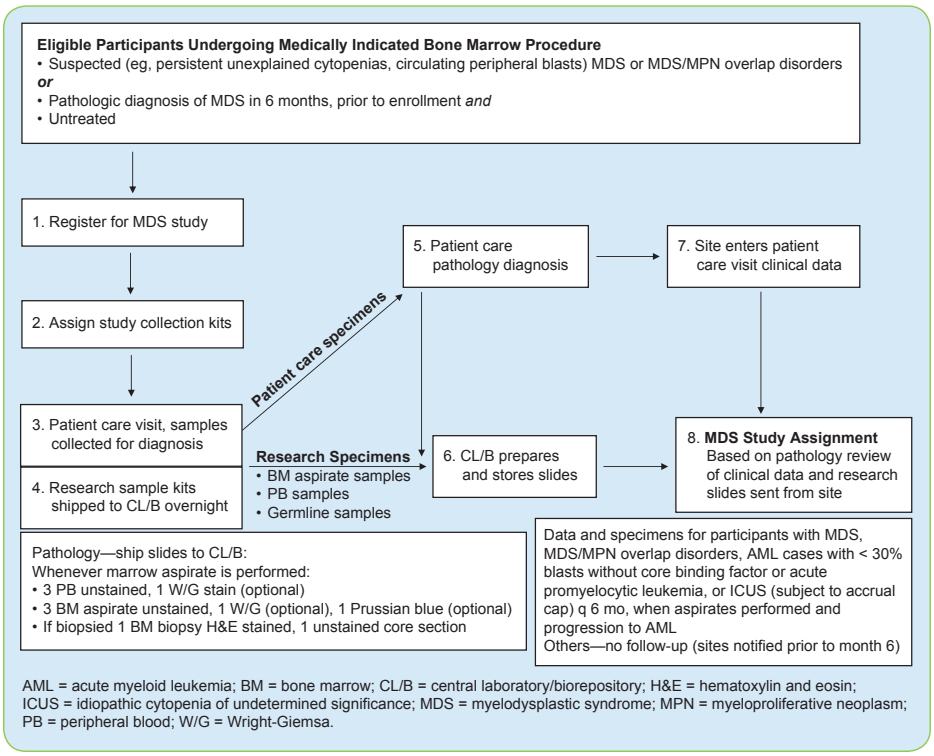
## Overall Study Goal

To establish a publicly available resource to facilitate the study of MDS natural history by creating a multi-institutional, longitudinal biorepository of consistently processed and clinically well-annotated blood and tissue specimens, collected prospectively from up to 2000 participants with MDS and up to 500 participants with idiopathic cytopenia of undetermined significance (ICUS), and to support investigator-initiated studies with high impact for MDS patients

## Objectives

- Develop a high-quality clinical database containing clinical history, including environmental exposure history presenting signs and symptoms, diagnostic testing results, coexisting diseases, therapies and response to therapies, disease progression, quality of life, and survival
- Develop a high-quality biorepository linked to clinical data that will facilitate diverse studies, including genetic, epigenetic, immunologic, proteomic, and cell-functional and -phenotypic studies
- Facilitate broad use of these linked data and specimens to support studies focused on
  - Improving diagnostic accuracy, risk stratification and prognostication, and medical decision-making in MDS
  - Understanding quality of life and its relationship to changing disease and treatment status
  - Understanding the pathogenesis of MDS and diverse MDS subtypes, including genetic, epigenetic, and immunologic mechanisms
  - Optimizing treatment strategies for specific MDS subtypes
  - Identifying novel biomarkers for MDS outcomes
  - Identifying novel targets for therapeutic interventions in MDS

## Study Schema




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.

## How Your Site Can Participate

- **Before recruitment,** investigators must be registered members of an NCTN network group
- Investigators must have a National Cancer Institute (NCI) investigator number and maintain "active" investigator registration status by annually submitting a complete investigator registration packet (current CV and signed FDA Form 1572, Supplemental Investigator Data Form, and Financial Disclosure Form) to the Food and Drug Administration (FDA) and NCI. Please refer to the Cancer Therapy Evaluation Program (CTEP) Web site for additional information and to obtain forms
  - CTEP Web site: http://ctep.cancer.gov/investigatorResources/investigator_registration.htm
  - For questions, please contact: pmbregpend@ctep.nci.nih.gov
  - Sites participating on the NCI Cancer Prevention and Control (CPC) CIRB initiative and accepting CPC CIRB approval need not submit separate IRB approval documentation to the CTSU for initial, continuing, or amendment review. For these sites, IRB data automatically load to RSS. However, sites must submit a Study Specific Worksheet for Local Context (SSW) to the CIRB (via IRBManager) to indicate their intention to open the study locally. The CIRB's approval of the SSW is then communicated to the CTSU Regulatory Office for compliance in the RSS. The signatory site may be contacted by the CTSU Regulatory Office or asked to complete information verifying participating institutions on the study. Other site registration requirements (ie, lab or protocol-specific training certifications, modality credentialing) must be submitted to the CTSU Regulatory Office, or compliance communicated per protocol instructions

## How Your Site Can Participate (cont)

- **Requirements for NHLBI-MDS site registration:**
  - CTSU IRB Certification (for sites not participating via the NCI CIRB)
  - CTSU IRB/Regulatory Approval Transmittal Sheet (for sites not participating via the NCI CIRB)
  - Protocol, biospecimen acquisition, biospecimen shipping, GlobalTrace, and Medidata Rave training
- OPEN blocks enrollment until this training is completed:
  - One investigator per site takes the NHLBI-MDS investigator training course on the protocol and biospecimen acquisition by accessing https://coccg813.mindflash.com/PublicCoursePage.aspx?c=1483326417
  - One coordinator per site takes the NHLBI-MDS coordinator training course on the protocol, CLB shipping, biospecimen acquisition, GlobalTrace, and Medidata Rave by accessing https://coccg813.mindflash.com/PublicCoursePage.aspx?c=1483397179
  - Direct questions to EAClinEd@ecog-acrin.org. ECOG-ACRIN is automatically e-mailed a certificate copy and sends information electronically to the CTSU Regulatory Office
- For all sites, Central Laboratory/Biorepository registration processes are required including the following:
  - Identification of at least one member of the study staff certified with IATA or equivalent training to ship biological substances
  - Completed CL/B Information Checklist
- **Submit all required regulatory documents to:**
  - CTSU Regulatory Office via the Regulatory Submission Portal (www.ctsu.org [members' area] → Regulatory Tab → Regulatory Submission)
  - When applicable, mail original documents to:
    - CTSU Regulatory Office
      1818 Market Street, Suite 1100
      Philadelphia, PA 19103
- **Required regulatory documentation:**
  - Copy of IRB Informed Consent Document
  - CTSU IRB Certification Form ***or*** signed HHS OMB No. 0990-0263 (replaced Form 310) ***or*** IRB Approval Letter

  *Note:* Submission must include all sites approved for the protocol under an assurance number; OHRP assurance number of reviewing IRB; full protocol title and number; version date; type of review (full board vs expedited); date of review; signature of IRB official.
- Check registration status at https://www.ctsu.org
- **Once documentation has been submitted and approved:**
  - Study samples should not be collected prior to registration
  - Patient enrollment is via OPEN, accessed at https://open.ctsu.org. Data collection is exclusively through Medidata Rave. Address OPEN and Medidata Rave questions to the CTSU Help Desk at 1-888-823-5923 or ctsucontact@westat.com

## NHLBI-MDS Funding and NCORP Credit Reimbursement

- All participating sites (non-NCORP and NCORP [Standard and High Performance]) will receive a base payment of $1250.00 per enrollment
- For enrollment of patients with MDS or MDS/MPN overlap disorders (up to 2000 participants), or ICUS (up to 500 participants), who proceed to the longitudinal study cohort as determined by the Central Pathology Review, institutions will receive an additional $1250.00 per patient
- Total potential federal funds are $2500.00 for all participating sites
- Funds are provided via NCORP funding or capitation payments
- In addition to capitation payments for enrollments, these groups give credit toward membership requirements:
  - Alliance: 1.0 credit for initial protocol enrollment for base intervention
  - ECOG-ACRIN: 0.5 credit for initial protocol enrollment for base intervention; 0.5 credit for any patient assigned to longitudinal cohort
  - NRG: 1.0 credit for initial protocol enrollment for base intervention
  - SWOG: 0.5 credit for initial protocol enrollment for base intervention; 0.5 credit for any patient assigned to longitudinal cohort
- For more information or any questions, please contact ea.fundingsheet@jimmy.harvard.edu

## Contact Information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## For Further Study Information

- For more information about the NHLBI-MDS study, please visit the following:
  - The National MDS Study Web site, https://thenationalmdsstudy.net
  - Cancer.gov; search NHLBI-MDS
  - Clinicaltrials.gov; search NCT02775383

NIH National Heart, Lung, and Blood Institute

NCI Community Oncology Research Program

ECOG-ACRIN cancer research group
Reshaping the future of patient care