European LeukemiaNet
SHORTPROTOCOL
AZA-MDS-003

Public Title	Azacitidine Plus BSC vs Placebo Plus BSC in Red Blood Cell Transfusion-dependent Anemia and Thrombocytopenia Due to Lower-risk MDS
Scientific Title	A Phase 3, Multicenter, Randomized, Double-blind Study to Compare the Efficacy and Safety of Oral Azacitidine Plus Best Supportive Care Versus Placebo Plus Best Supportive Care in Subjects With Red Blood Cell Transfusion-dependent Anemia and Thrombocytopenia Due to IPSS Lower-risk Myelodysplastic Syndromes
Short Title	AZA-MDS-003
Id KN/ELN	LN_DEUTSC_2013_513
Trialgoup	Deutsche MDS
Type of Trial	multicentric, randomized, prospective, double-blind, double-group
Phase	Phase III
Disease	Myelodysplastic Syndrome(MDS) Low risk and intermedia I
Stage of Disease	.
Outcomes	- Red blood cell (RBC) transfusion independence Up to 60 months (Primary Outcome) - Number of patients alive Up to 60 months - Hematological improvement-platelet response (HI-P) Up to 60 months - Duration of RBC transfusion independence Up to 60 months - Time to RBC transfusion independence Up to 60 months - Progression to acute myeloid leukemia (AML) Up to 60 months - Time to AML progression Up to 60 months - Hematological improvement-erythroid response (HI-E) Up to 60 months - Platelet-transfusion independence Up to 60 months - Duration of platelet transfusion independence Up to 60 months - Time to platelet transfusion independence Up to 60 months - Hematologic response Up to 60 months - Clinically significant bleeding events Up to 60 months - Number of subjects with adverse events Up to 60 months - Health-related quality-of-life Up to 60 months - Healthcare resource utilization Up to 60 months
Inclusion Criteria	- 18 years or older - Have a documented diagnosis of MDS - Anemia that requires red blood cell transfusions - Thrombocytopenia (sustained for at least 21 days) within 14 days prior to randomization - Have an Eastern Cooperative Oncology Group (ECOG) performance status of 0, 1, or 2 - Must agree to follow pregnancy precautions as required by protocol - Must be willing to consent to two or more bone marrow aspirate procedures to be completed during study
Exclusion Criteria	- Secondary or hypoplastic MDS or other subtype with eligibility for treatment with immunotherapy - Prior treatment with azacitidine, decitabine, other hypomethylating agents and lenalidomide - Prior allogeneic or autologous stem cell transplant - Eligible for allogenic or autologous stem cell transplant

- History of inflammatory bowel disease (eg, Crohn's disease, ulcerative colitis), celiac disease (ie, sprue), prior gastrectomy or upper bowel removal, or any other gastrointestinal disorder or defect
- Thrombocytopenia secondary to other possible causes, including medication(s), congenital disorder(s), immune disorder(s), or microvascular disorder(s)
- Use of cytotoxic, chemotherapeutic, targeted or investigational agents/therapies, thrombopoiesis-stimulating agents (TSAs), erythropoiesis-stimulating agents (ESAs) and other red blood cell hematopoietic growth factors, and within 28 days prior to randomization
- Ongoing adverse events from previous treatment, regardless of the time period
- Concurrent use of iron-chelating agents, (except for subjects on a stable dose for at least 8 weeks (56 days) prior to randomization), corticosteroid (except for subjects on a stable or decreasing dose for >= 1 week prior to randomization for medical conditions other than MDS)
- Prior history of cancer, other than MDS, unless the subject has been free of the disease for >= 3 years. (Basal or squamous cell carcinoma of the skin, carcinoma in situ of the cervix, carcinoma in situ of the breast, and incidental histologic finding of prostate cancer) (T1a or T1b using the tumor, nodes, metastasis [TNM] clinical staging system is allowed)
- Significant active cardiac disease within the previous 6 months
- Uncontrolled systemic fungal, bacterial, or viral infection
- Known Human Immunodeficiency Virus (HIV) or Hepatitis C (HCV) infection, or evidence of active Hepatitis B Virus (HBV) infection
- Known clinically significant anemia due to iron, vitamin B12, or folate deficiencies, or autoimmune or hereditary hemolytic anemia, or gastrointestinal bleeding
- Abnormal coagulation parameters
- Abnormal liver function test results
- Abnormal kidney function test results
- Known or suspected hypersensitivity to azacitidine or mannitol
- Any significant medical condition, laboratory abnormality, or psychiatric illness

Age	>= 18 years
Status	Active
start of Recruitment	01.02.2013
Recruiting countries	Germany
	U.K.
	Finland
	Czech Republic
	Denmark
	Spain
	Portugal
Target Sample Size	386

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Other Registers ClinicalTrials.govNCT01566695
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