Public Title	Efficacy and Safety of Oral Rigosertib in Transfusion-dependent, Low or Int-1 or Trisomy 8 Int-2 MDS
Scientific Title	A Phase II, Multicenter, Single-arm Study to Assess the Efficacy and Safety of Oral Rigosertib in Transfusion-dependent Low or Intermediate-1 (Any Cytogenetics) or Trisomy 8 Intermediate-2 Myelodysplastic Syndrome Patients Based on IPSS Classification
Short Title	ONTARGET
Id KN/ELN	LN_DEUTSC_2012_528
Trialgoup	Deutsche MDS
Type of Trial	single-group, open-label
Phase	Phase II
Disease	Myelodysplastic Syndrome(MDS) Low risk and intermedia I Myelodysplastic Syndrome(MDS) Intermedia II and high risk
Stage of Disease	.
Aim	- The primary objectives of this study are to determine if rigosertib sodium, given orally in the form of soft gel capsules, is safe and is associated with a reduction in the number of blood transfusion units that are needed in patients with myelodysplastic syndrome (MDS) classified as Low or Intermediate-1 (Int-1) (any cytogenetics) or trisomy 8 Intermediate 2 (Int-2) in the International Prognostic Scoring System (IPSS) who are transfusion-dependent. Rigosertib will be taken only on days 1 to 14 of the 21-day cycle.
Outcomes	- Number of units of red blood cell transfusions 8 weeks (Primary Outcome) - Number of Adverse Events (AEs) From date of randomization until 30 days after last dose of study drug - Bone marrow blasts 4 weeks - Complete blood count 4 weeks
Inclusion Criteria	- Diagnosis of MDS confirmed by bone marrow aspirate and/or biopsy within 6 weeks prior to first dose of study drug according to World Health Organization (WHO) or French-American-British (FAB) classification - MDS classified as Low risk or Int-1 risk (any cytogenetics) or Trisomy 8 Int-2 risk, according to IPSS classification - Transfusion dependency defined by at least 4 units of RBC administered within 8 weeks before baseline - Off all other treatments for MDS (azacitidine, decitabine, lenalidomide, chemotherapy, immunosuppressive agents) for at least 4 weeks - ECOG performance status of 0, 1 or 2
Exclusion Criteria	- Ongoing clinically significant anemia due to factors such as iron, B12, or folate deficiencies, auto-immune or hereditary hemolysis, or gastrointestinal (GI) bleeding, unless stabilized for 1 week after RBC transfusion - Serum ferritin < 50 ng/mL - Hypoplastic MDS (cellularity < 10%) - Any active malignancy within the past year, except basal cell or squamous cell skin cancer or carcinoma in situ of the cervix or breast - Uncontrolled intercurrent illness including, but not limited to, symptomatic congestive heart failure, unstable angina pectoris, or cardiac arrhythmia - Active infection not adequately responding to appropriate therapy - Total bilirubin >= 1.5 mg/dL not related to hemolysis or Gilbert's disease - ALT/AST >= 2.5 x upper limit of normal (ULN)

- Serum creatinine >= 2.0 mg/dL
- Ascites requiring active medical management including paracentesis
- Hyponatremia (defined as serum sodium value of <130 mEq/L)
- Female patients who are pregnant or lactating
- Patients who are unwilling to follow strict contraception requirements
- Female patients with reproductive potential who do not have a negative urine beta-human chorionic gonadotropin (bHCG) pregnancy test at Screening
- Major surgery without full recovery or major surgery within 3 weeks of rigosertib treatment start
- Uncontrolled hypertension (defined as a systolic pressure >=160 mmHg and/or a diastolic pressure >=110 mmHg)
- New onset seizures (within 3 months prior to the first dose of rigosertib) or poorly controlled seizures
- Any other concurrent investigational agent or chemotherapy, radiotherapy, or immunotherapy
- Chronic use (>2 weeks) of corticosteroids (>10 mg/24 hr equivalent prednisone) within 4 weeks of starting rigosertib
- Investigational therapy within 4 weeks of starting rigosertib
- Psychiatric illness or social situation that would limit the patient's ability to tolerate and/or comply with study requirements

Age	>= 18 years
Status	Closed
start of Recruitment	01.05.2012
Target Sample Size	60
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Supporters	Onconova Therapeutics, Inc.
Other Registers	ClinicalTrials.govNCT01584531