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Efficacy and Safety of Eltrombopag In Patients With Severe and Very Severe Aplastic Anemia

This study is currently recruiting participants.

Verified March 2013 by University of Utah

Sponsor:
George Rodgers

Collaborator:
GlaxoSmithKline

Information provided by (Responsible Party):
George Rodgers, University of Utah

ClinicalTrials.gov Identifier:
NCT01703169

First received: September 27, 2012
Last updated: March 7, 2013
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History of Changes

Full Text View | Tabular View | No Study Results Posted | Disclaimer | How to Read a Study Record

Purpose

The investigators hypothesis is that eltrombopag given to patients with moderate to very severe aplastic anemia will result in an increase in platelet counts. The investigators hypothesize that in patients with moderate to very severe aplastic anemia, treatment with eltrombopag will lead to fewer platelet transfusions, red blood cell transfusions, and fewer bleeding events. The investigators hypothesize that in patients with moderate to very severe aplastic anemia, eltrombopag will have an acceptable toxicity rate <3%, at doses that result in increased platelet counts. Finally the investigators hypothesize that plasma eltrombopag levels in peripheral blood will correlate with improved platelet counts.

Condition	Intervention	Phase
Severe Aplastic Anemia Very Severe Aplastic Anemia Moderate Aplastic Anemia	Drug: Eltrombopag	Phase 2

Study Type: Interventional
Study Design: Endpoint Classification: Safety/Efficacy Study
Intervention Model: Single Group Assignment
Masking: Open Label
Primary Purpose: Treatment

Official Title: Efficacy and Safety of Eltrombopag In Patients With Severe and Very Severe Aplastic Anemia

Resource links provided by NLM:

MedlinePlus related topics: Anemia Aplastic Anemia

Drug Information available for: Eltrombopag

U.S. FDA Resources

Further study details as provided by University of Utah:

Primary Outcome Measures:

- Platelet Count Response [Time Frame: up to 12 weeks] [Designated as safety issue: No]

  Defined as a stable platelet count of 50,000/µl or more during any 4 week period within the possible 12 weeks while on study,and including maximal platelet counts achieved in patients with moderate to very severe aplastic anemia.

Secondary Outcome Measures:

- Platelet count twice baseline. [Time Frame: Between weeks 1-12.] [Designated as safety issue: Yes]

  Proportion of subjects who achieve platelet counts at least twice their baseline value at any point while on study medication, in patients with moderate to very severe aplastic anemia.

- Hematology labs [Time Frame: 12 weeks] [Designated as safety issue: Yes]

  Association between eltrombopag use and response in hemoglobin, hematocrit, total white blood cell count, and absolute neutrophil count to be evaluate by maximal hemoglobin, hematocrit, total white blood cell count, and absolute neutrophil counts achieved in patients with moderate to

very severe aplastic anemia

- Number of patients with AE to measure toxicity, using NCI CTCAE [Time Frame: 12 weeks] [Designated as safety issue: Yes]

  Evaluated weekly, up to 12 weeks. Association between eltrombopag use, dose, and tolerability in patients with moderate to very severe aplastic anemia

- Characterization of the PK profile of eltrombopag in patients with moderate to very severe aplastic anemia. Evaluated with AUC, Cmax, Cmin, tmax. [Time Frame: Weeks 2, 6 and 12] [Designated as safety issue: No]

  Samples will for PK analysis will collected as a trough level weeks 2, 6 and 12, prior to dose of eltrombopag. Additional PK level drawn at 2, 4 and 6 hours post-dose at the scheduled week 2 visit.

Estimated Enrollment:	30
Study Start Date:	November 2012
Estimated Study Completion Date:	November 2015
Estimated Primary Completion Date:	November 2014 (Final data collection date for primary outcome measure)

Arms	Assigned Interventions
Experimental: Eltrombopag Single arm study. Dose Escalation.	Drug: Eltrombopag Oral eltrombopag 150mg/day by mouth starting on Day 1 with dose modification over 12 weeks to a maximum of 300mg/day determined by platelet count

Eligibility

Ages Eligible for Study:	18 Years and older
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria

Inclusion Criteria:

- Able to provide written informed consent and any other authorizations required by local law (e.g., Protected Health Information [PHI])
- Have severe or very severe aplastic anemia, or moderate aplastic anemia with platelet counts that have dropped below 20,000/µl
- Have moderate, severe, or very severe aplastic anemia with moderate bleeding during or after a surgical procedure, (including bone marrow biopsy, lumbar puncture, thoracentesis, paracentesis, port placement, dermal biopsy) or minimal mucocutaneous bleeding otherwise noted
- Subjects with current or previous exposure to approved medications for the treatment of aplastic anemia will not be excluded; these include but may not be limited to, anti-thymocyte globulin (ATG), cyclosporine, corticosteroids, and G-CSF.

Exclusion Criteria:

- Have diagnosis of Fanconi anemia
- Have infection not adequately responding to appropriate therapy
- Have Paroxysmal Nocturnal Hemoglobinuria (PNH) clone size in neutrophils of greater than or equal to 50%
- Have known HIV positivity
- Have creatinine and/or blood urea nitrogen (BUN) ≥2 times the upper limit of normal
- Have serum bilirubin ≥ 1.5 times the upper limit of normal
- Have AST and/or ALT ≥ 3 times the upper limit of normal
- Have hypersensitivity to eltrombopag or its components
- Have chemotherapy given less than or equal to 14 days prior to initiating the study medication. This does not include immunosuppressive agents and growth factor as described above
- Are female and are nursing or pregnant or are unwilling to take oral contraceptives or refrain from pregnancy if of childbearing potential
- Are unable to understand the investigational nature of the study or give informed consent
- Have a history of arterial or venous thrombosis within the last 1 year (excluding those due to indwelling lines)
- Have an ECOG performance status of 3 or greater
- Have had treatment with Campath within 6 months of entry into the study
- Have pre-existing cardiovascular disease (congestive heart failure with New York Heart Association [NYHA] grade III/IV), arrhythmia known to increase the risk of thromboembolic events (e.g. atrial fibrillation), unstable angina, or QTc > 450 msec (QTc 480 msec for subjects with bundle branch block), or myocardial infarction within the preceding 6 months) at study entry
- Have had other TPO-R agonists medication in the previous 4 weeks.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01703169

Contacts

Contact: Renee Rinaldi, BS 801-585-5341 renee.rinaldi@hsc.utah.edu

Contact: Marc Nuttall, MD marc.nuttall@hsc.utah.edu

Locations

United States, Utah

University of Utah **Recruiting**
Salt Lake City, Utah, United States, 84112
Contact: Renee Rinaldi 801-585-5341 renee.rinaldi@hsc.utah.edu
Contact: Marc Nuttall, MD marc.nuttall@hsc.utah.edu

Sponsors and Collaborators

George Rodgers

GlaxoSmithKline

Investigators

Principal Investigator: George M Rodgers, M.D. University of Utah

More Information

No publications provided

Responsible Party:	George Rodgers, Professor of Medicine, University of Utah, University of Utah
ClinicalTrials.gov Identifier:	NCT01703169 History of Changes
Other Study ID Numbers:	ELT115895
Study First Received:	September 27, 2012
Last Updated:	March 7, 2013
Health Authority:	United States: Food and Drug Administration United States: Institutional Review Board

Keywords provided by University of Utah:
severe aplastic anemia
very severe aplastic anemia
moderate aplastic anemia
bleeding

Additional relevant MeSH terms:
Anemia
Anemia, Aplastic
Hematologic Diseases
Bone Marrow Diseases

ClinicalTrials.gov processed this record on October 21, 2013