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Extended Dosing With Eltrombopag for Severe Aplastic Anemia

This study is currently recruiting participants.

Verified April 2013 by National Institutes of Health Clinical Center (CC)

Sponsor:
National Heart, Lung, and Blood Institute (NHLBI)

Information provided by (Responsible Party):
National Institutes of Health Clinical Center (CC) (National Heart, Lung, and Blood Institute (NHLBI))

ClinicalTrials.gov Identifier:
NCT01891994

First received: June 28, 2013
Last updated: August 20, 2013
Last verified: April 2013
History of Changes

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

Purpose

Background:

- Eltrombopag is a drug being tested for treating severe aplastic anemia. It can help improve blood counts in these patients. However, researchers do not know how long the drug can and should be taken for this type of anemia..

Objectives:

- To look at whether 6 months of treatment with eltrombopag can improve patient's blood counts.

Eligibility:

- Individuals at least 2 years of age who are taking eltrombopag for severe aplastic anemia.

Design:

- Participants will take eltrombopag by mouth once a day for 6 months.
- Blood samples will be collected every 2 weeks for the first 6 months. Bone marrow samples will be collected at 3 and 6 months. These samples will look at the effects of the study drug on the marrow.
- Participants will continue to take the study drug for as long as it is effective and if the side effects are not severe.

Condition	Intervention	Phase
Severe Aplastic Anemia (SAA)	Drug: Eltrombopag (promacta)	Phase 2

Study Type: Interventional
Study Design: Allocation: Non-Randomized
Endpoint Classification: Efficacy Study
Intervention Model: Single Group Assignment
Masking: Open Label
Primary Purpose: Treatment

Official Title: Extended Dosing With Eltrombopag in Refractory Severe Aplastic Anemia

Resource links provided by NLM:

Genetics Home Reference related topics: cyclic neutropenia

MedlinePlus related topics: Anemia Aplastic Anemia

Drug Information available for: Eltrombopag

U.S. FDA Resources

Further study details as provided by National Institutes of Health Clinical Center (CC):

Primary Outcome Measures:

- The proportion of drug responders as defined by changes in the platelet count and/or platelet transfusion requirements, hemoglobin levels, number of red blood cell transfusions, or neutrophil counts as measured by IWG criteria. [Time Frame: 6 months]
  [Designated as safety issue: No]

Secondary Outcome Measures:

- Hematological response at 3 & 12 months and yearly thereafter; (b) relapse (c) clonal evolution to PNH, clonal chromosomal population in bone marrow, myelodysplasia by morphology, or acute leukemia; (d) survival & (e) health-related qu... [Time Frame: Indefinite]
  [Designated as safety issue: Yes]

Estimated Enrollment:	60
Study Start Date:	June 2013
Estimated Study Completion Date:	March 2017
Estimated Primary Completion Date:	March 2017 (Final data collection date for primary outcome measure)

Intervention Details:

Drug: Eltrombopag (promacta)

N/A

Show Detailed Description

Eligibility

Ages Eligible for Study:	2 Years and older
Genders Eligible for Study:	Both
Accepts Healthy Volunteers:	No

Criteria

- INCLUSION CRITERIA:
- Previous diagnosis of refractory severe aplastic anemia and following at least one treatment course of immunosuppression with a regimen consisting of either antithymocyte globulin, alemtuzumab or cyclophosphamide.
- One or more of the following three clinically-significant cytopenias: platelet count less than or equal to 30,000/micro L or platelet-transfusion-dependence (requiring at least 4 platelet transfusions in the 8 weeks prior to study entry); neutrophil count less than 500/micro L; hemoglobin less than 9.0 g/dL or red cell transfusion-dependence (requiring at least 4 units of PRBCs in the eight weeks prior to study entry)
- Age greater than or equal to 2 years old
- Weight > 12 kg

EXCLUSION CRITERIA:

- Infection not adequately responding to appropriate therapy
- Evidence of a clonal disorder on cytogenetics performed within 12 weeks of study entry.
- Creatinine > 2.5 mg/dl
- Bilirubin > 2.0 mg/dl
- SGOT or SGPT > 5 times the upper limit of normal
- Hypersensitivity to eltrombopag or its components
- Female subjects who are nursing or pregnant or are unwilling to take oral contraceptives or refrain from pregnancy if of childbearing potential
- Unable to understand the investigational nature of the study or give informed consent
- Moribund status or concurrent hepatic, renal, cardiac, neurologic, pulmonary, infectious, or metabolic disease of such severity that it would preclude the patient's ability to tolerate protocol therapy, or that death within 7-10 days is likely
- Treatment with ATG, cyclophophamide or alemtuzamab within 6 months of study entry.

Contacts and Locations

Please refer to this study by its ClinicalTrials.gov identifier: NCT01891994

Contacts

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Locations

United States, Maryland

National Institutes of Health Clinical Center, 9000 Rockville Pike
Bethesda, Maryland, United States, 20892
Contact: For more information at the NIH Clinical Center contact Patient Recruitment and Public Liaison Office (PRPL) 800-411-1222 ext TTY

Sponsors and Collaborators

National Heart, Lung, and Blood Institute (NHLBI)

Investigators

Principal Investigator: Ronan G Desmond, M.D. National Heart, Lung, and Blood Institute (NHLBI)

More Information

Additional Information:

NIH Clinical Center Detailed Web Page

Publications:

Young NS, Calado RT, Scheinberg P. Current concepts in the pathophysiology and treatment of aplastic anemia. Blood. 2006 Oct 15;108(8):2509-19. Epub 2006 Jun 15. Review.

Risitano AM, Maciejewski JP, Green S, Plasilova M, Zeng W, Young NS. In-vivo dominant immune responses in aplastic anaemia: molecular tracking of putatively pathogenetic T-cell clones by TCR beta-CDR3 sequencing. Lancet. 2004 Jul 24-30;364(9431):355-64.

Chen J. Animal models for acquired bone marrow failure syndromes. Clin Med Res. 2005 May;3(2):102-8. Review.

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Keywords provided by National Institutes of Health Clinical Center (CC):
Cytokine
Hematopoiesis
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Neutropenia

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

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