

Myelodysplastic Syndromes (MDS) and Anemia: Potential New Treatments Through Clinical Research

EPO-ANE-3018 Study

Anemia (a drop in the body's red-blood-cell count) is the most common blood abnormality in the early stages of MDS. Treatments that can reduce or delay the need for blood transfusions may improve and extend better quality of life for persons with early stage MDS. More research is needed to evaluate such treatments and to obtain FDA approval for use in patients with early disease who are not yet transfusion dependent.

In the EPO-ANE-3018 study, epoetin alfa will be evaluated in patients with early stage MDS, who are not yet treatment dependent, to see if it can delay the need for transfusion. Transfusion dependence is defined as the requirement of an average of two units of adult sized red blood cell units per month. Patients with early stage MDS who have no or low red blood cell transfusion requirements are included in this study because there currently are limited treatment options for MDS patients who have anemia but are not requiring red blood cell transfusions on a regular basis.

Research to date suggests that epoetin alfa is effective in reducing the need for transfusions in patients with early stages of MDS. Epoetin alfa is a manufactured form of the human hormone erythropoietin, which stimulates the production of red blood cells.

Epoetin alfa is distributed in the United States, the European Union, and other countries under several brand names including PROCIT®, EPREX®, and ERYPO® for the treatment of other related disease conditions.

If you are a patient with early stage MDS and anemia who is not yet transfusion dependent or a health professional caring

for a patient, and would like to receive more information about this study, please refer to the contact information at the end of this article.

What is the purpose of this study?

The purpose of the EPO-ANE-3018 clinical research study is to explore the use of epoetin alfa, to see if it will decrease the need for blood transfusions and increase the hemoglobin level in patients with early stage MDS and anemia.

Who qualifies for this study?

To qualify for this study you must:

- Be at least 18 years of age
- Have been diagnosed with MDS
- Have an International Prognostic Scoring Systems (IPSS) score of Low- to Intermediate-1 Risk Disease
- Have anemia (a hemoglobin count of 10 g/dL or below)
- Not transfusion dependent (<4 red blood cell units during a consecutive 8-week period) in the past 6 months

What can you expect if you are eligible and enroll in this study?

- Before any study related procedures are performed, the study doctor will discuss the study in detail with you, including any potential risks or benefits.
- If you participate, you will be randomly assigned (by chance, like flipping a coin) to one of three investigational treatment schedules:
 - Epoetin alfa 40,000 IU (1 mL) given once a week by subcutaneous (under the skin) injection
 - Epoetin alfa 80,000 IU (2 mL) given once a week by subcutaneous injection
 - Placebo given once a week by subcutaneous injection. Half of this group will be assigned to 1 mL dosing and the other half will be assigned to 2 mL dosing.
- You will visit the study center each week during a 48-week Study Treatment Phase for blood tests, assessment of disease progression, to receive study drug and periodic measurement of iron stores.

- You may continue to receive the investigational study drug beyond the 48-weeks if you do not require transfusions and your doctor feels that you are benefiting from the treatments.
- All patients will receive current standard of care for anemia management.
- You will continue to have safety evaluations for 4 and 1/2 years following study participation. These visits for the most part should coincide with routine scheduled visits to your doctor for your condition.

For doctors caring for a patient(s) with early stage MDS who may be a candidate(s) for this study:

- Approximately 450 subjects will be randomly assigned to one of the study drug schedules
- The Study Phases Include:
 - Pre-randomization (Screening) Phase: Day –1 to –14
 - Study Treatment Phase: Day 1/Week 1 to Week 48
 - Safety Assessment Phase, consisting of:
 - Short Term Safety (Week 52) or Early Withdrawal from treatment visit
 - Long Term Safety Assessments—until progression to AML, death, or the clinical cutoff is reached, whichever occurs first
- An Independent Data Monitoring Committee (IDMC) will periodically review overall safety data throughout the study.
- An Independent Central Pathology Reviewer will review bone marrow samples and peripheral blood counts for assessment of disease progression.

To learn more about participating in the EPO-ANE-3018 study or to refer a patient to this study, please contact the MDS Foundation by E-mailing us at: CTC@mds-foundation.org or by calling our toll free EPO-ANE-3018 study number: 1-888-813-1260 (within the US) or 609-298-7741 (outside of the US).

We look forward to talking with you and working together to find new and better treatments for patients with early stage MDS.