Efficacy and Safety of Imetelstat in RBC Transfusion-Dependent IPSS Low/Int-1 MDS Relapsed/Refractory to Erythropoiesis-Stimulating Agents (ESA) (IMERGE)

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Background

- MDSD: characterized by clinical myelofibrosis arising from malignant megakaryocytes, with polyclonal genetic abnormalities.
- Telomerase activity: An enzyme that catalyzes the synthesis of telomeric repeat DNA at the ends of chromosomes. It is responsible for maintaining telomere length, which is important for the proper function and survival of cells.

Eligibility

1. IMerge: ongoing 2-part, global, phase 2/3 study of imetelstat in red blood cell (RBC) transfusion-dependent (TD), ESA-relapsed/refractory lower risk MDS. Part 1 consists of an open-label, single-arm design with imetelstat monotherapy.
2. IMerge: ongoing 2-part, global, phase 2/3 study of imetelstat in red blood cell (RBC) transfusion-dependent (TD), ESA-relapsed/refractory lower risk MDS. Part 1 consists of an open-label, single-arm design with imetelstat monotherapy.

Endpoints and Analysis

- Primary endpoint: rate of RBC transfusion-independence (TI) lasting ≥8 weeks
- Key secondary endpoints:
  1. Safety
  2. Rate of ≥24-week TI
  3. Time to and duration of TI
  4. Hematologic improvement (HI)
  5. Rate of complete response (CR) and partial response (PR) per IWG

Results

- A total of 32 patients were included in Part 1 of the study.
- The median age of patients was 64 years, with a range of 31 to 83 years.
- The median number of RBC transfusions received in the prior 8 weeks was 20.
- At baseline, 13 patients (41%) were classified as hypomethylating agent (HMA) naïve and lacked del(5q).

Safety

- No patients had liver function test (LFT) elevations at least one grade.
- No events were generally grade 2 or reversible.
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