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Promising treatment results with Imetelstat, a novel telomerase inhibitor, in patients with lower risk myelodysplastic syndromes

IMerge is a Phase 2/3 clinical trial evaluating imetelstat as a treatment for patients with lower risk myelodysplastic syndromes (MDS)that are non-del(5q), dependent on red blood cell transfusion, and are relapsed after or refractory to treatment with erythropoiesis stimulating agents.

The primary efficacy endpoint of IMerge is 8-week red blood cell transfusion independence (RBC-TI) rate, defined as the proportion of patients not receiving any RBC transfusion during any consecutive eight weeks since entry into the trial. This presentation reports long-term efficacy and safety data from 38 patients in the IMerge Phase 2 clinical trial, based on a February 4, 2020 cut-off date and a median follow-up of 24 months:

- 16 patients (42%) achieved 8-week RBC-TI, and 12 of these responders (75%) showed a hemoglobin rise of > 3 g/dL compared to pretreatment during the transfusion-free interval.
- 12 patients (32%) achieved a 24-week RBC-TI.
- 11 patients (29%) were transfusion-free for more than one year; the longest transfusion-free interval was 2.7 years.
- Median RBC-TI duration was 88 weeks, the longest reported to date in non-del(5q) LR MDS.
- Hematologic improvement-erythroid was achieved by 26 patients (68%) with a median duration of 93 weeks.
- Durability of TI, cytogenetic and mutational malignant clone reduction in some patients indicates potential disease-modifying activity of Imetelstat.
- Most frequently reported adverse events were manageable and reversible grade > 3 cytopenias.

The Phase 3 double-blind, placebo-controlled stage of IMerge is currently recruiting patients and is ongoing.

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Abstract: **#S183** TREATMENT WITH IMETELSTAT PROVIDES DURABLE TRANSFUSION INDEPENDENCE (TI) IN HEAVILY TRANSFUSED NON-DEL(5Q) LOWER RISK MDS (LR-MDS) RELAPSED/REFRACTORY (R/R) TO ERYTHROPOIESIS STIMULATING AGENTS (ESA)

About the EHA Annual Congress: Every year in June, EHA organizes its Annual Congress in a major European city. Due to the COVID19 pandemic, the physical meeting was transformed into a Virtual Congress this year. Please note that our embargo policy applies to all selected abstracts in the Press Briefings. For more information, see our EHA Media and Embargo policy here.

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