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ORAL ABSTRACTS

906.OUTCOMES RESEARCH-MYELOID MALIGNANCIES

Real-World Impact of Luspatercept on Patients with Myelodysplastic Syndromes Requiring Red Blood Cell Transfusions and with Prior Exposure to Erythropoietin-Stimulating Agents: A United States Healthcare Claims Database Study

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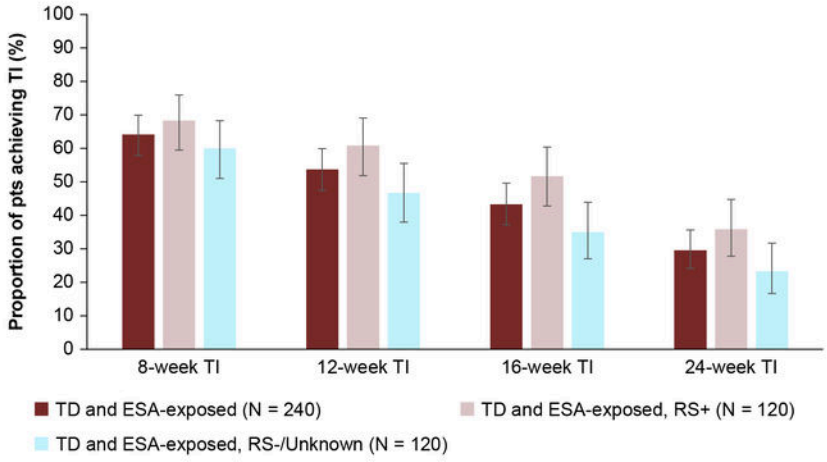
Background: Myelodysplastic syndromes (MDS) are among the most common hematologic cancers in older US adults. MDS are characterized by abnormal bone marrow function leading to impaired production of blood cells, persistent cytopenia, and variable risk of progression to acute myeloid leukemia (AML). Since anemia is a common complication of MDS, many patients (pts) become dependent on red blood cell transfusions (RBCTs) and erythropoiesis-stimulating agents (ESAs) are the standard of care for patients with lower-risk MDS. In a randomized, double-blind, placebo-controlled phase 3 MEDALIST clinical trial, luspatercept, a first-in-class erythroid maturation agent, was shown to reduce the severity of anemia among transfusion-dependent (TD) pts with lower-risk MDS with ring sideroblasts (RS) status and who were refractory to prior ESA treatment (Fenaux P, et al. *N Engl J Med* 2020; 382:140-151). However, real-world evidence regarding the effect of luspatercept on transfusion burden among pts with MDS requiring RBCTs and with prior ESA exposure is currently limited.
Aim: To describe clinical outcomes and MDS-related treatment patterns before and after the initiation of luspatercept among real-world pts with lower-risk MDS in the USA who required RBCTs before luspatercept initiation and were previously exposed to ESAs.
*Methods:*In this retrospective claims-based cohort study, data from Symphony Health, a nationally representative database, (Jan 1, 2010 to June 30, 2022) were used to identify adults with ≥1 claim with a diagnosis of MDS (initial diagnosis on or after Jan 1, 2015) before the first claim for luspatercept (index date). Pts were considered TD at luspatercept initiation if they had ≥1 claim for RBCT in the 8 weeks before index date, and ESA-exposed if they had ≥1 ESA claim at any point before or at luspatercept initiation. Pts with chemotherapy treatments, with a diagnosis for refractory anemia with excess blasts, or with AML during the study period were excluded as they were presumed to have higher-risk MDS. Pts were also excluded if they had prior use of hypomethylating agents (HMAs) or a diagnosis for β-thalassemia, myelofibrosis, aplastic anemia, or breast cancer in the 6 months prior to index date (baseline period). Pt demographics and clinical characteristics were assessed during the baseline period. Pts' RBCT status (transfusion independent [TI] or TD) was measured in the 6 months after index date. A pt was considered 8-week TI if they had 0 RBCT in ≥1 rolling 8-week period in the 6 months after index date. 12-week, 16-week, and 24-week TI pts were defined similarly, using increasingly longer periods without RBCT. Pts' use of MDS-related treatment classes (ie, chemotherapy, ESAs, granulocyte-colony stimulating factor [G-CSF], HMA, and immunomodulatory imide drug [IMiD] agent) was measured before and after index date until end of data availability or clinical activity (follow-up).
*Results:*The population comprisedof240 pts with MDS who were TD and had ESA exposure before luspatercept initiation. The mean (standard deviation [SD]) age was 75.4 (5.4) years and 56.7% were male. About one fifth of pts received ≥2 treatment classes before luspatercept initiation (2: 17.5%; 3+: 1.7%). Most pts (64.2%) achieved 8-week TI in the 6 months after luspatercept initiation. Similarly, 53.8%, 43.3%, and 29.6% of pts achieved 12-, 16-, and 24-week TI in the 6 months after luspatercept initiation, respectively (Figure 1). The percentage of pts achieving 8-, 12-, 16-, and 24-week TI was numerically higher among pts with positive RS status than those with negative/unknown RS status (Figure 1). Most pts (64.2%) did not require any addi-

tional MDS-related treatments during the study follow-up period (mean (SD) 15.6 (7.3) months). The most common treatment classes used after luspatercept initiation were ESA (22.5%) and HMA (16.3%; Figure 2).
*Conclusions:*The findings of this real-world study strongly corroborate clinical trial results, showing that luspatercept is highly effective in reducing transfusion dependence among ESA-exposed pts with lower-risk MDS who were TD before luspatercept initiation. In this study, over half of pts achieved 8-week and 12-week TI status during the follow-up period, and more than a quarter did not require any RBCT over a 24-week period. Further, most pts treated with luspatercept did not require any additional MDS-related treatments during the follow-up period.

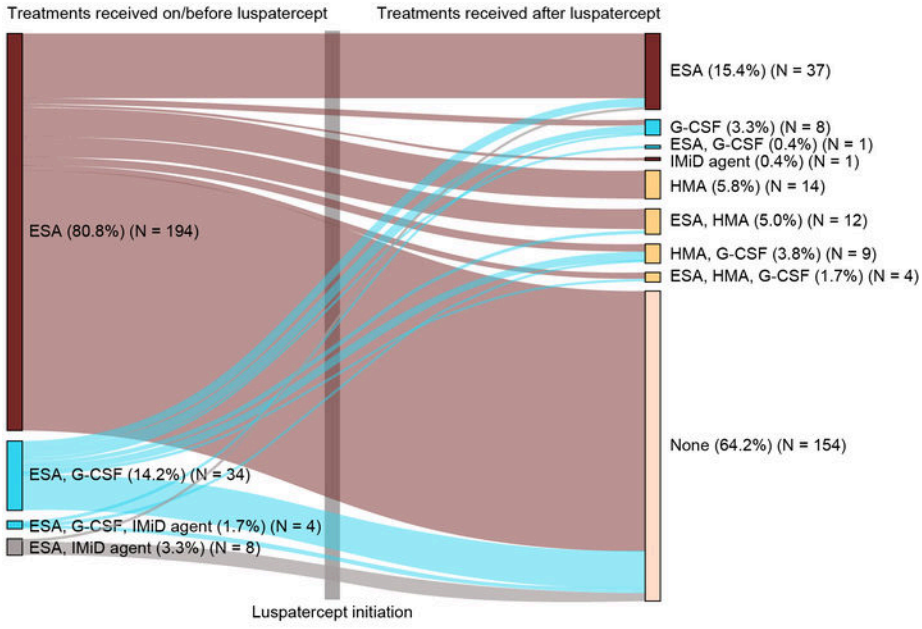
Disclosures Patel: *Carolina Blood and Cancer:* Current Employment. **Hanna:** *Janssen:* Speakers Bureau; *Pharmacyclics:* Speakers Bureau; *AbbVie Inc:* Speakers Bureau; *Rigel Inc:* Speakers Bureau; *G1 Therapeutics:* Speakers Bureau; *Exelixis:* Speakers Bureau; *Bristol Myers Squibb:* Speakers Bureau; *Seagen:* Consultancy, Speakers Bureau; *BeiGene:* Consultancy, Speakers Bureau; *M Health Fairview:* Ended employment in the past 24 months; *NCODA:* Membership on an entity's Board of Directors or advisory committees; *Minnesota Oncology:* Current Employment. **Zanardo:** *United Therapeutics Co.:* Research Funding; *Sun Pharmaceuticals Ltd.:* Research Funding; *Novartis AG:* Research Funding; *Pfizer Inc:* Research Funding; *Takeda Pharmaceutical Company:* Research Funding; *Merck & Co Inc:* Research Funding; *AbbVie Inc:* Research Funding; *Bristol Myers Squibb:* Research Funding. **McBride:** *Bristol Myers Squibb:* Current Employment. **Barghout:** *Regeneron:* Consultancy; *Sun Pharma:* Consultancy; *Novartis:* Consultancy; *Taiho:* Consultancy; *Bristol Myers Squibb:* Consultancy. **Song:** *Analysis Group:* Current Employment; *Novartis:* Research Funding; *Alexion:* Research Funding; *Bristol Myers Squibb:* Research Funding; *GlaxoSmithKline Pharmaceuticals Ltd:* Research Funding; *Pfizer:* Research Funding; *Merck:* Research Funding. **Huynh:** *Takeda Oncology:* Research Funding; *Novartis:* Research Funding; *Merck & Co Inc:* Research Funding; *Apellis Pharmaceuticals:* Research Funding; *Genmab:* Research Funding. **Tang:** *Bristol Myers Squibb:* Current Employment, Current equity holder in publicly-traded company. **Yenikomshian:** *Cleerly Health:* Research Funding; *Procept Biorobotics:* Research Funding; *Sun Pharmaceuticals Inc:* Research Funding; *Bristol Myers Squibb:* Research Funding; *Enanta Pharmaceuticals Inc:* Current equity holder in publicly-traded company; *Analysis Group:* Current Employment.

Figure 1. Baseline TD and ESA-exposed pts achieving rolling TI within 6 months after luspatercept initiation[a,b]



[a]A pt was TI during follow-up if they had 0 dates of RBCT in ≥ 1 rolling 8-, 12-, 16-, or 24-week period after the index date; [b]Error bars indicate lower and upper bounds of the 95% confidence interval, calculated using the modified Wald method. ESA, erythropoiesis-stimulating agent; pt, patient; RBCT, red blood cell transfusion; RS, ring sideroblast; TD, transfusion dependent; TI, transfusion independent.

Figure 2. Sankey diagram of MDS-related treatments received before and after luspatercept initiation[a,b]



[a]Use of MDS-related treatments prior to luspatercept initiation was assessed using the total data availability on or prior to the initiation date. Use of MDS-related treatments after luspatercept initiation was assessed using the total data availability after initiation date; [b]Treatment groups represent presence of ≥1 claim for each treatment in the group in the respective period. Treatments received after initiation of luspatercept may be used concomitantly with luspatercept. ESA, erythropoiesis-stimulating agent; pt, patient; RBCT, red blood cell transfusion; RS, ring sideroblast; TD, transfusion dependent; TI, transfusion independent.

Figure 1

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