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POSTER ABSTRACTS

906.OUTCOMES RESEARCH-MYELOID MALIGNANCIES

Real-World Retrospective Study of Non-Transfusion Dependent Patients with Myelodysplastic Syndromes Treated with Luspatercept: A Healthcare Claims Database Study

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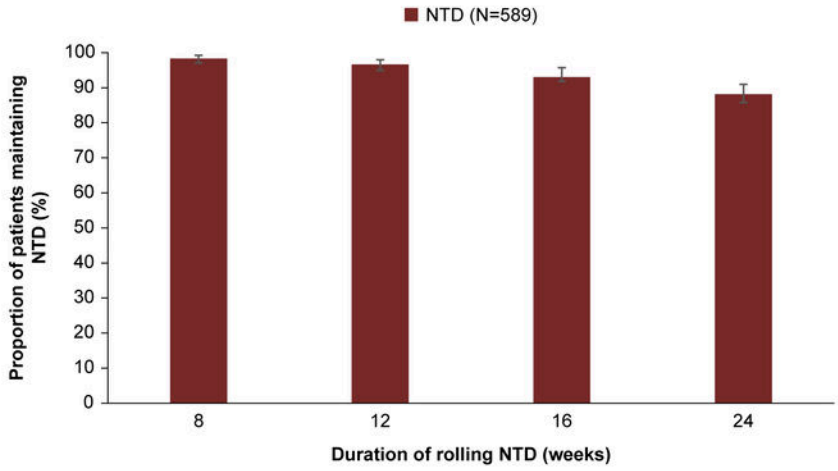
Background: Myelodysplastic syndromes (MDS) are common hematologic malignancies in older adults that are characterized by abnormal bone marrow function leading to reduced blood cell production, persistent cytopenias, with a risk that patients may progress to acute myeloid leukemia (AML). Due to the high rate of anemia, many patients with MDS require red blood cell transfusions (RBCT), which, in turn, are associated with a high clinical and economic burden. In the phase 3 randomized COMMANDS and MEDALIST clinical trials, luspatercept reduced the severity of anemia among transfusion-dependent (TD), erythropoiesis-stimulating agent (ESA)-refractory and ESA-naive patients with lower-risk (LR)-MDS (Platzbecker U, et al. *Lancet* 2023. doi:10.1016/S0140-6736(23)00874-7, Fenaux P, et al. *N Engl J Med* 2020;382:140-151). However, there are limited real-world data on the use of luspatercept in patients who are non-transfusion dependent (NTD) or have a low transfusion burden. This study aims to describe the real-world clinical outcomes and treatment patterns before and after the initiation of luspatercept in patients with LR-MDS who were NTD before luspatercept initiation, in the USA.
*Methods:*In this retrospective claims-based cohort study, data from the Symphony Health database (Jan 1, 2010 to June 30, 2022) were used to identify adults with a diagnosis of MDS (initial diagnosis on or after Jan 1, 2015) before the first claim for luspatercept (index date). Patients were considered NTD if they had no unique dates with a claim for RBCT during the 8-week period before or on the index date. Patients were excluded if they received chemotherapy or had a diagnosis of refractory anemia with excess blasts or AML during the study period, as they were presumed to have higher-risk MDS. Patients were also excluded if they had a diagnosis of β-thalassemia, myelofibrosis, aplastic anemia, or breast cancer in the 6 months pre-index (baseline period) or prior use of hypomethylating agents (HMAs). Patient demographics and clinical characteristics were assessed during the baseline period, and patients' RBCT status (NTD or TD) was measured for 6 months after the index date. A patient was considered 8-week NTD if they had no RBCT in $\geq$ 1 rolling 8-week period; 12-week, 16-week, and 24-week NTD patients were defined similarly, using increasing durations of NTD. Administration of other MDS therapies (ie, chemotherapy, ESA, granulocyte colony stimulating factor [G-CSF], HMA, and immunomodulatory imide drug [IMiD ®] agent) was measured pre-index and post-index until the end of clinical activity or data availability (follow-up).
Results: Of the 871 eligible patients who received luspatercept 589 (67.6%) were NTD before luspatercept initiation. Among this NTD population, the mean (standard deviation [SD]) age was 74.7 (6.3) years and 57.2% were male. Before luspatercept initiation, 15.3% of patients received 2 treatment classes and 0.8% received $\geq$ 3 treatment classes. Almost all patients (98.5%) maintained 8-week NTD status in the 6 months after luspatercept initiation. Similarly, 96.8%, 94.1%, and 88.6% of patients maintained NTD status for at least 12, 16, and 24 weeks in the 6 months after luspatercept initiation, respectively (Figure 1). Most patients (64.7%) did not require any additional MDS-related treatments during the study follow-up period, (median [SD] 15.7 [7.4] months). Overall, 77.1% of patients were treated with luspatercept for > 6 months and 68.4% for > 12 months. After luspatercept initiation, patients most commonly received treatment with ESAs or HMAs (14.9%; Figure 2).
*Conclusion:*This is the first real-world evidence study to address luspatercept treatment in patients who were NTD prior to luspatercept treatment, which resulted in positive clinical outcomes and few RBCTs.Almost all patients with LR-MDS who

were NTD at luspatercept initiation maintained 8-week NTD status during the 6 months after luspatercept initiation. The majority of patients did not receive a RBCT for a 24-week period after luspatercept initiation. Finally, over three-quarters of the patients remained on luspatercept and two-thirds of patients did not receive additional MDS-related treatments after a median follow-up of 15.7 months.

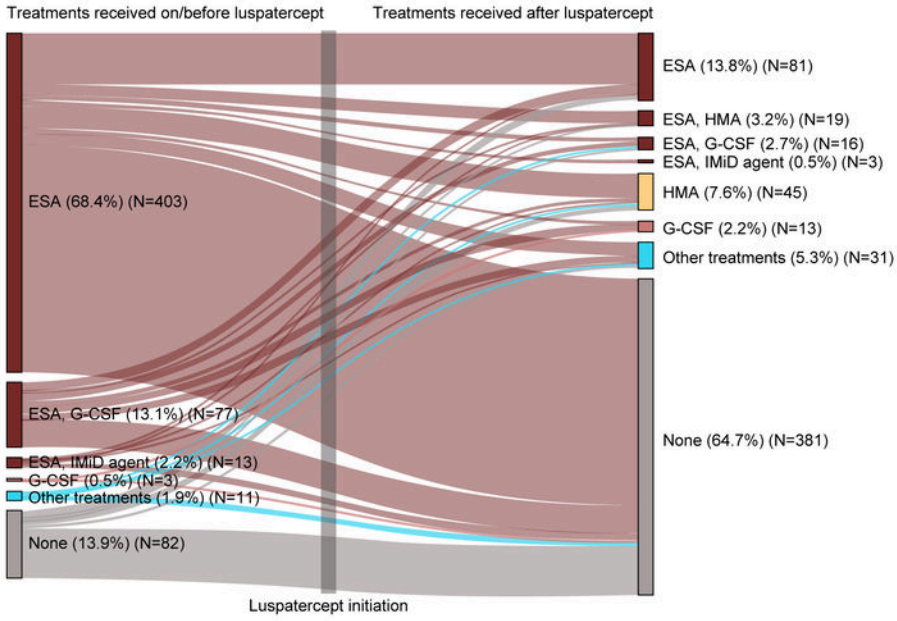
Disclosures Hanna: *M Health Fairview:* Ended employment in the past 24 months; *BeiGene:* Consultancy, Speakers Bureau; *Seagen:* Consultancy, Speakers Bureau; *Bristol Myers Squibb:* Speakers Bureau; *Exelixis:* Speakers Bureau; *Minnesota Oncology:* Current Employment; *AbbVie Inc:* Speakers Bureau;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

Figure 1. Baseline NTD patients achieving rolling NTD within 6 months after luspatercept initiation[a,b]



[a]A patient was NTD during follow-up if they had no 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. RBCT, red blood cell transfusion; NTD, non-transfusion dependence.

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



[a]Use of MDS-related treatments prior to luspatercept initiation was assessed using the total data availability on or prior to the 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; [c]Treatment groups in the "Other treatments" category include those with prevalence < 2% in both the pre- and post-luspatercept periods.These include: ESA, HMA, and G-CSF; HMA and G-CSF; IMiD agent; HMA and IMiD agent; ESA, G-CSF, and IMiD agent; ESA, HMA, G-CSF, and IMiD agent; HMA, G-CSF, and IMiD agent. ESA, erythropoiesis-stimulating agent; G-CSF, granulocyte colony-stimulating factor; HMA, hypomethylating agent; IMiD, immunomodulatory imide drug; MDS, myelodysplastic syndromes.

Figure 1

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