LUSPATERCEPT FOR ANEMIA IN MYELOFIBROSIS

A Systematic Evidence Synthesis and Random-Effects Meta-Analysis

ABSTRACT 1675

Arafat Shabbir, MD[1]; Anas Al-Sadi[2]; Muhammad Zubair[3]; Amna Bint I Munir[4]; Priyanka Barad[5]; Khubaib Gul[6]; Zeeshan Sattar[7]; Juhi Chovatiya[8]; Muhammad Kashif Amin[7]; Muhammad Umair Mushtaq[7:] Moazzam Shahzad[9]

[1]Jefferson Regional Medical Center, Pine Bluff, AR, USA., [2]University of Missouri-Kansas City, Kansas City, MO, USA.,[3]Harvard Medical School, Beth Israel Deaconess Medical Center, Center for Resuscitation Science, Boston, MA, USA.,[4]North Alabama Medical Center, Florence, AL, USA., [5]Harnett Health Internal Medicine Residency, Dunn, NC, USA.,[6]Inspira Health Network, Vineland, NJ, USA.,[7]University of Kansas Medical Center, Kansas City, KS, USA.,[8]University of California, Riverside School of Medicine, Riverside, CA, USA.,[9]H. Lee Moffitt Cancer Center, University of South Florida, Tampa, FL, USA.

INTRODUCTION

Anemia and RBC transfusion dependence are major drivers of morbidity in myelofibrosis and may limit continuation of JAK inhibitor therapy.

Luspatercept is a TGF-β superfamily ligand trap that enhances late-stage erythropoiesis and addresses refractory anemia while preserving disease-directed treatment.

OBJECTIVE

Quantify pooled efficacy and safety outcomes of luspatercept in myelofibrosis-associated anemia.

METHODS

- Structured synthesis of phase II, retrospective, real-world, and conference data.
- Random-effects meta-analysis of proportions using logit transformation and DerSimonian–Laird estimation.
- Heterogeneity assessed using Cochran Q and I².
- Sensitivity and leave-one-out analyses evaluated robustness.

TREATMENT PROFILE

LUSPATERCEPT DOSE

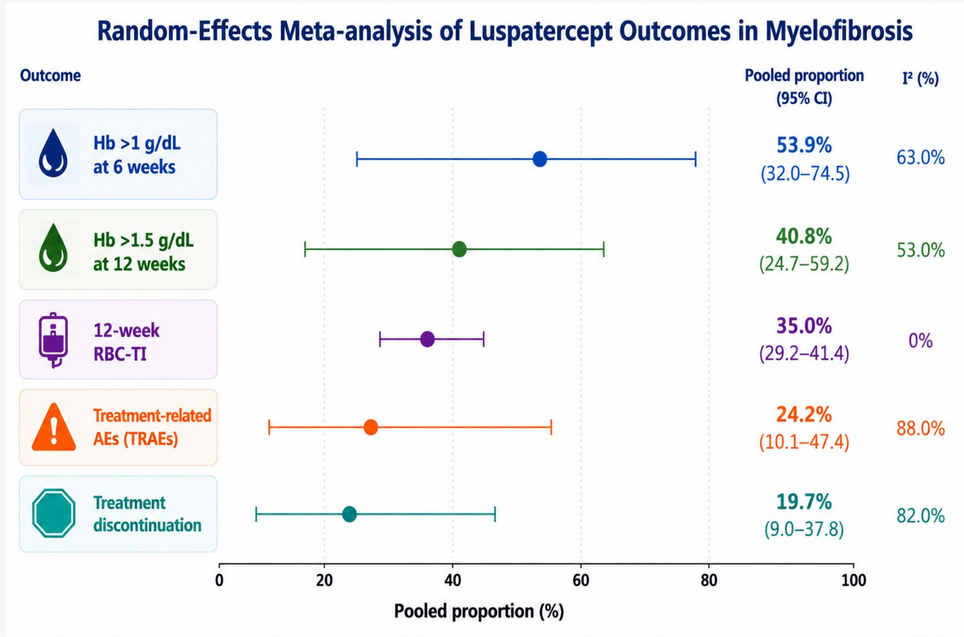
1.0–1.75 mg/kg SC every 3 weeks

MEDIAN FOLLOW-UP

7–24 months reported; 12.3 months in the largest real-world cohort

RESULTS

11 study entries • Random-effects synthesis • 12-week RBC-TI robust to sensitivity and leave-one-out analysis



PRIMARY FINDING

12-week RBC transfusion independence: 35.0% (95% CI 29.2–41.4); I² = 0%

KEY FINDINGS

35.0%
12-week RBC-TI

53.9%
Hb >1 g/dL at 6 weeks

40.8%
Hb >1.5 g/dL at 12 weeks

SAFETY

24.2%
Treatment-related AEs

19.7%
Treatment discontinuation

CONCLUSION

- Luspatercept achieved clinically meaningful 12-week transfusion independence in approximately one-third of evaluable patients.
- These findings support luspatercept as an effective anemia-directed therapy that complements JAK inhibitor treatment.
- Hemoglobin responses were favorable but heterogeneous.
- Safety and discontinuation varied substantially across studies.

Abbreviations: AE, adverse event; CI, confidence interval; Hb, hemoglobin; RBC-TI, red blood cell transfusion independence; SC, subcutaneous. Meta-analysis based on extractable aggregate study-level data.