Bristol Myers Squibb™

Investigational Trials of Luspatercept in Myeloproliferative Neoplasm–Associated Myelofibrosis

INDEPENDENCE

A Phase 3, Double-Blind, Randomized Study to Compare the Efficacy and Safety of Luspatercept (ACE-536) Versus Placebo in Subjects With Myeloproliferative Neoplasm–Associated Myelofibrosis on Concomitant JAK2 Inhibitor Therapy and Who Require Red Blood Cell Transfusions[1,2]

Enrolling globally ***NCT04717414 PHASE 3***

PRIMARY ENDPOINT
Proportion of patients who become red blood cell–transfusion-free over any consecutive 12-week period

SELECT SECONDARY AND EXPLORATORY ENDPOINTS
Proportion of patients who become red blood cell–transfusion-free over any consecutive 16-week period, safety, OS

KEY ELIGIBILITY CRITERIA

- Age ≥18 years
- Diagnosis of PMF or post-ET or post-PV MF
- Require RBC transfusions (4–12 RBC U/12 weeks)
- Require continuous JAK2 inhibitor therapy for SOC
- ECOG PS ≤2

Stratification factors:

- *RBC transfusion burden at baseline (4–5 vs 6–12 U/12 weeks)*
- *DIPSS score at baseline (intermediate-1/-2 vs high risk)*

TREATMENT

Blinded core treatment period (Weeks 1–24)

Blinded extension treatment period (Week 25+)

Luspatercept + BSC
1.33 mg/kg every 3 weeks with titration up to 1.75 mg/kg

Luspatercept + BSC

R 2:1
Estimated N=309

Response assessment

Placebo + BSC

Placebo + BSC

FOLLOW-UP

Post-treatment follow-up period
(5 years post first dose or 3 years post last dose, whichever occurs later)

42-day follow-up period:
AEs and hematology

Long-term follow-up period:
AEs of special interest

Transformation to blast phase and other malignancies/premalignancies

Survival and other post-treatment myelofibrosis therapies

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Enrolling ***NCT03194542 PHASE 2***

Study to Evaluate Luspatercept in Subjects With Myeloproliferative Neoplasm–Associated Myelofibrosis Who Have Anemia With and Without Red Blood Cell–Transfusion Dependence[3]

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The safety and efficacy of luspatercept for the indications under investigation have not been established. There is no guarantee that luspatercept will receive health authority approval or become commercially available in any country for the uses being investigated.

ACCELERON in collaboration with Celgene Corporation, a Bristol Myers Squibb company

AE, adverse event; **BSC**, best supportive care; **DIPSS**, Dynamic International Prognostic Scoring System; **ECOG PS**, Eastern Cooperative Oncology Group performance status; **ET**, essential thrombocythemia; **JAK2**, Janus kinase 2; **MF**, myelofibrosis; **OS**, overall survival; **PMF**, primary myelofibrosis; **PV**, polycythemia vera; **RBC**, red blood cell; **SOC,** standard of care; **U,** units.

1. Bristol-Myers Squibb Company. Data on file (protocol number ACE-536-MF-002). **2.** ClinicalTrials.gov. NCT04717414. Accessed May 7, 2021. **3.** ClinicalTrials.gov. NCT03194542. Accessed May 7, 2021.