Thalassemia (2 Presentations) & MDS (1 Presentation)

S844 IMPROVEMENTS IN HEMOGLOBIN, QUALITY OF LIFE, AND SIX-MINUTE-WALK DISTANCE IN ADULTS WITH B-THALASSEMIA TREATED WITH LUSPATERCEPT: LONG-TERM PHASE 2 STUDY

Author(s): Antonio Piga, et al. ABSTRACT

Conclusion
In this phase 2 open-label study, long-term luspatercept treatment in pts with β-thalassemia was generally safe and well tolerated up to 2 years. Clinically relevant measures of luspatercept efficacy were observed in both NTD pts (increased Hgb levels and improved QoL) and TD pts (decreased transfusion burden).

PF691 IMPACT OF CLINICAL AND SOCIAL FACTORS ON QUALITY OF LIFE (QOL) IN PATIENTS (PTS) WITH TRANSFUSION-DEPENDENT (TDT) AND NON-TRANSFUSION-DEPENDENT (NTDT) BETA-THALASSEMIA: A MULTICENTER STUDY

Author(s): Vip Viprakasit, et al. ABSTRACT

Conclusion
In the routine clinical care setting, there are critical unmet medical needs for pts with NTDT as they reported lower QoL scores compared with pts with TDT, as captured by two health questionnaires (SF-36v2 and FACT-An), across all domains except one. There is a need for new interventions to treat pts with NTDT and reduce their burden of disease. Significant differences between pt populations from different geographical locations were identified, suggesting social factors had an impact on difference in QoL between pts with TDT and NTDT. Pts from Thailand reported higher QoL scores for domains on the FACT-An questionnaire versus pts from centers in Italy, Greece, and Lebanon; half of the pts with TDT were from the Thai center.

PF498 MUTATIONAL AND SUBGROUP ANALYSES OF LOWER-RISK MYELODYSPLASTIC

SYNDROMES (MDS) PATIENTS TREATED WITH LUSPATERCEPT: PHASE 2 PACE-MDS STUDY

Author(s): Uwe Platzbecker, et al. ABSTRACT

Conclusion

Increased response rates in patients with lower M/E ratios suggest that an expanded erythroid population at baseline may be associated with response to luspatercept, supporting the concept that luspatercept is acting as an erythroid-maturation agent (EMA). Patients with and without the SF3B1 mutation with EPO