QUALITY OF LIFE OF HEMOPHILIA PATIENTS WITH INHIBITORS – OPPORTUNITIES FOR IMPROVEMENT

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Introduction

- Morbidity of hemophilia A and B increases with age
- Patients who develop neutralizing antibodies (inhibitors) (HPWI) to replacement clotting factor typically receive bypassing agents for treatment of bleeds and are not provided prophylaxis
- The very short half-life of available intravenous agents for HPWI means episodic treatment for bleeds that results in:
  - Subjectively poorer Quality of Life (QOL)
  - Significantly premature mortality
  - Worse musculoskeletal outcomes when compared with patients without inhibitors
- QOL in hemophilia may be evaluated by Haem-A-QOL and impaired physical activity with Haemophilia Activities List (HAL)

Objectives

- Document the baseline quality of life status of HPWI subjects entering the MAA-201 trial using two validated QOL tools
- Compare the QOL of MAA-201 subjects with published values for a similar population without inhibitors
- Compare before and after treatment Haem-A-QOL and HAL scores in the MAA-201 trial

Methods

- MarzAA was administered in an open-label safety and efficacy trial (MAA-201) in HPWI (Minimum ABR 12 and documented inhibitor) and the efficacy results were presented at ISTH 2019
- Baseline QOL for the 17 subjects screened for this trial were compared with published values for subjects with severe hemophilia using Haem-A-QOL and HAL
- For Haem-A-QOL reference data were used from subjects without inhibitors recruited into a long term prophylaxis trial (The A-LONG trial). For HAL we used the scores of van Genderen's HAL validation set
- Missing values were imputed or using last-value-carried-forward method
- Evidence of interval change in QOL for subjects who completed the MAA-201 trial were assessed

Results

- Seventeen subjects were screened; 6 were screen failures; 1 subject withdrew consent after a single SQ dose of MarzAA; 1 subject died from an unrelated SAE 12 days into the study
- Baseline QOL data was available (Haem-A-QOL and HAL) for 16 screened subjects
- Baseline QOL scores in MAA-201 were worse across the majority of domains compared with the published reference groups
- Eight subjects had interval reassessment of QOL (6 subjects 50 days; 1 subject 42 days and 1 subject 28 days)
- There were 8 QOL interval pairs for Haem-A-QOL and 7 pairs for HAL

Baseline comparisons of screened subjects with published scores

Trend toward improvement in HAL Scores in almost all domains

Consistent trend toward improvement in Mean Haem-A-QOL Scores in treated subjects

References