Expanded Analysis from SCA Clinical Trial BHV4157-201 and a Matched Natural History Cohort

Biohaven announced results today of a post-hoc analysis of patients enrolled in the short-term randomization and long-term extension phase of Study BHV4157-201 [NCT02960893], an initial Phase 2b/3 randomized controlled trial of troriluzole in patients with SCA, compared to patients selected from a natural history cohort of SCA patients who were matched on multiple eligibility criteria.

The primary efficacy endpoint for this analysis was the change from baseline in the Scale for the Assessment and Rating of Ataxia (SARA) total score after 48 weeks of follow up. Patients from the natural history cohort were matched to patients from the BHV4157-201 trial on SCA Genotype (SCA1, SCA2, SCA3, SCA6), age at baseline (18 to 75 years of age), gender, SARA Score at baseline (≥ 8 and ≤ 30), and initial score on gait item of the SARA ≥ 2.

Based on analysis of covariance (ANCOVA) least square mean changes after one year were -0.34 points (representing numerical improvement with a 95% confidence interval of -0.94 to 0.26) for 81 troriluzole-treated patients versus +1.07 points (representing numerical decline with a 95% confidence interval of 0.56 to 1.58) for 112 natural history cohort patients (increasing score indicates worsening disease status). The LS Mean difference between cohorts was -1.41 points (95% confidence interval of -2.22 to -0.60) suggesting therapeutic benefits of troriluzole (p=0.0007).

Figure 1: SCA patients treated for 1 year with troriluzole exhibit no disease progression versus Ashizawa natural history cohort**

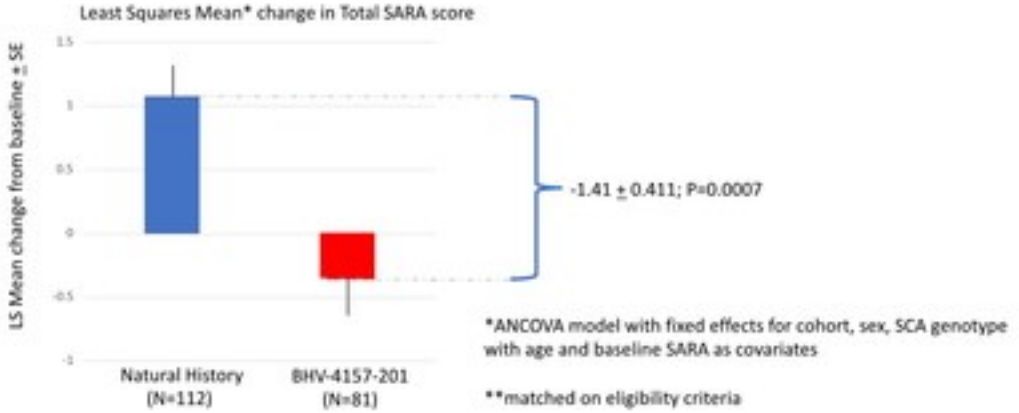

Figure 1 shows an attenuation of disease progression at one year among patients treated with troriluzole versus the natural history cohort in this post-hoc analysis. The difference in progression rates (-1.41) exceeds the minimum clinically important difference of 1.0 on the total SARA score at one year.

The natural history cohort was derived from a prospective study, conducted by the Clinical Research Consortium for Spinocerebellar Ataxias (Ashizawa, et al. 2013) and

recruited from 12 ataxia clinics throughout the United States. Patients in Study BHV4157-201 were treated with 140 mg of troriluzole administered daily for one year.

Gil L'Italien, Ph.D., Head of Global Health Economics and Outcomes Research at Biohaven stated, "The findings from the post-hoc extension phase analysis of troriluzole compared to matched untreated patients from the natural history cohort are encouraging and provide further support for the potential long-term therapeutic benefit of troriluzole in patients with SCA. We are thrilled to have now enrolled the first patient in the Phase 3 study, which will more fully test the therapeutic potential of troriluzole in treating SCA over the course of one year."