**Background**

MSA is a rare, adult-onset, rapidly progressive, and fatal neurodegenerative disease with no disease modifying treatment available. Verdiperstat (previously BHV-3241/AZD3241) is a first-in-class, oral, potent, selective, brain-permeable, irreversible myeloperoxidase (MPO) inhibitor. In phase 2 studies in Parkinson’s disease (PD) and MSA, treatment with verdiperstat was generally safe and well tolerated. Verdiperstat decreased MPO activity in plasma, providing evidence of target engagement: reduced translocator protein binding on brain PET imaging in PD, providing proof of mechanism (decreased microglial activation/neuroinflammation) and demonstrated favorable trends on clinical efficacy measures (Unified MS Rating Scale [UMSARS]) at 12 weeks.

**Methods**

M–STAR is a randomized, double-blind, placebo-controlled, parallel group study. Ambulatory participants, 40–80 years of age, with possible or probable MSA, including MSA–P or MSA–C, are randomized to 48 weeks of treatment with verdiperstat 600 mg twice daily or placebo. The primary efficacy endpoint is change from baseline to Week 48 in verdiperstat– vs. placebo–treated participants on a score derived from the UMSARS, optimized (based on health authority guidance) to assess clinically meaningful change in ability to function.

**Results**

Between July 2019 and July 2020, M–STAR enrolled 336 participants at 48 study sites across 6 countries. Baseline characteristics are presented (Figs. 1–7, Tables 1–5). The sex breakdown of participants is almost even (Fig. 3), 97% are not Hispanic or Latino (Fig. 4), and most are white (Fig. 5). Participants are close to evenly split between Parkinsonian and Cerebellar subtypes of MSA, with slightly more MSA–C, and the breakdown is similar to that in peer trials (Fig. 6). The sex breakdown of participants is almost even (Fig. 3), 97% are not Hispanic or Latino (Fig. 4), and most are white (Fig. 5). Participants are close to evenly split between Parkinsonian and Cerebellar subtypes of MSA, with slightly more MSA–C, and the breakdown is similar to that in peer trials (Fig. 6).

**Conclusion**

Baseline characteristics of M–STAR participants provide key information about the enrolled population, including MSA diagnostic classifications and clinician- and patient-reported disease severities, which is significant for MSA clinical trials and therapeutic development.

**Objective**

To describe baseline characteristics of participants randomized in the ongoing M–STAR phase 3 study evaluating the disease modifying effect of verdiperstat on multiple system atrophy (MSA).