SOM Biotech receives positive EMA COMP opinion on European Orphan Drug Designation for SOM3355 for treatment of Huntington's Disease

Barcelona, Spain, September 15, 2025 - SOM Biotech, a clinical-stage company dedicated to the discovery and development of innovative therapies for rare central nervous system disorders, today announces that the European Medicines Agency (EMA) Committee for Orphan Medical Products (COMP) has issued a positive opinion on granting Orphan Drug Designation (ODD) for SOM3355 for the treatment of Huntington's disease (HD).

SOM3355 is a molecule with a unique multimodal mechanism of action being a mild beta-blocker and a VMAT1 and VMAT2 inhibitor, that has already an ODD designation in the US. It has achieved positive results in both proof-of-concept and phase 2b clinical studies in HD.

The EU orphan designation for SOM3355 is important as it is granted when there is an ongoing unmet medical need for new and improved treatment options for those living with HD and their families. One of the criteria for orphan designation by the EMA is that 'the medicinal product will be of significant benefit to those affected by the condition' as in Article 3(1)(b) of Regulation (EC) No 141/2000. The benefit remains to be confirmed at the time of marketing authorization. The concept of significant benefit is unique to the EU orphan legislation.

"Such promise, which must be substantiated in the phase 3 clinical study, will resonate not only with patients in Europe but also worldwide. This recognition supports SOM Biotech's mission to explore the full potential of SOM3355 and to bring a novel first-line treatment option to the HD patients in the EU and beyond," said Silvia Panigone, CEO of SOM Biotech.

HD has a complex symptomatology, varying in nature at different stages of the disease. This arises from progressive neurodegeneration and the associated imbalance of neurotransmitters.

Current therapeutic interventions, as in the case of dopamine depleting agents, focus on individual symptoms of HD's complex clinical presentation that include motor, behavioral or psychiatric disturbances. This leads to polypharmacy and associated poor adherence, increased risk of adverse events, exacerbation of HD symptoms, drug–drug interactions and hospitalization. Side effects induced by dopamine depleting agents might not be distinguished from HD progression.

"The dynamic nature of HD requires that more medications are prescribed based on disease progression. The need to improve quality of care by treating HD in its complexity is now well recognized. If the clinical profile of SOM3355 is confirmed, we hope to offer a streamlined medicinal intervention addressing multiple symptoms with good tolerability that is accepted well by individuals suffering from HD," said Rossella Medori, MD, CMO of SOM Biotech.

SOM Biotech is fully committed to progressing globally with the last stage of development of SOM3355.

About Huntington's Disease (HD)
HD is a rare, hereditary, progressive neurodegenerative disorder, caused by a trinucleotide CAG-repeat expansion ≥36 in the Huntington gene. The disease is clinically characterized by a triad of symptoms, including motor abnormalities, neuropsychiatric disturbances and cognitive decline. At present, there is no cure for HD.

About SOM Biotech
SOM Biotech is a clinical-stage company dedicated to the discovery and development of innovative therapies for patients with rare central nervous system disorders. SOM developed a unique proprietary artificial intelligence (AI) platform (*SOM^{AI}PRO*) for the identification of a drug's new mechanism of action that has demonstrated a high clinical predictivity. SOM can rely on an extensive pipeline of molecules, including products for the treatment of TTR Amyloidosis (product licensed out upon positive phase 2a data), Huntington's Disease, Tardive Dyskinesia, Phenylketonuria and Duchenne Muscular Dystrophy.
www.sombiotech.com

Media and analyst contact
Andrew Lloyd & Associates
Juliette Schmitt – Saffiyah Khalique
US: +1 203 724 5950
UK: +44 1273 952 481