BioSpectrum
the business of Bio & Health Sciences
ASIA EDITION

Japan grants Orphan Drug Designation to Lundbeck's potential treatment for Multiple System Atrophy

March 11, 2025 | News

Lundbeck has recently initiated MASCOT trial, open for enrollment in North America, Europe, Asia and Australia



The Ministry of Health, Labour, and Welfare (MHLW) in Japan has granted Orphan Drug Designation (ODD) to Lundbeck's investigational drug, amlenetug, a potential new treatment option targeting Multiple System Atrophy (MSA).

MSA is a rapidly progressing rare condition of the nervous system that causes damage to nerve cells in the brain. MSA is seriously debilitating and places a high disease burden on patients.

The ODD in Japan adds to other important designations: the SAKIGAKE designation by Japan's MHLW in March 2023, the Orphan Drug Designation (ODD) by the US FDA in April 2024, and by EMA in May 2021.

Lundbeck has recently initiated, MASCOT, a phase III trial to assess efficacy and safety of amlenetug in the treatment of MSA. The MASCOT trial will open for enrollment in North America, Europe, Asia and Australia.

Amlenetug is a human monoclonal antibody (mAb) that recognises and binds to all major forms of extracellular ?-synuclein and thereby intended to prevent uptake and inhibit seeding of aggregation. Amlenetug has an active Fc region, which may increase immune-mediated clearance of ?- synuclein /mAb complexes through microglia mediated uptake. Amlenetug is being developed by Lundbeck under a joint research and licensing agreement between Lundbeck and Genmab A/S.