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Press Release

Ono Pharma Announces Poster Presentation of New Clinical Data from Phase 2 Study of ONO-2808 (S1P5 Receptor Agonist) in Multiple System Atrophy at the 7th World Parkinson Congress (WPC)

- In the Phase 2 study, the incidence of treatment-emergent adverse events was similar to placebo, and no unexpected safety signals were identified
- In mUMSARS scores and MRI-based brain volume assessment (vMRI), a tendency toward suppressed disease progression was observed compared to the placebo group
- Based on the results of the Phase 2 study, a pivotal Phase 3 study will be initiated

Osaka, Japan, May 28, 2026 - Ono Pharmaceutical Co., Ltd. (Headquarters: Osaka, Japan; President and COO: Toichi Takino; "Ono") announced the presentation of data from the randomized, double-blind, placebo-controlled, Phase 2 study of ONO-2808 （ONO-2808-03 study） for the treatment of multiple system atrophy (MSA) at the 7th World Parkinson Congress, held from May 24 to May 27, 2026, in Phoenix, Arizona, USA.

This presentation includes results of the double-blind core part of the ONO-2808-03 study at 24 weeks. In addition to the incidence of treatment-emergent adverse events (TEAEs), which is the primary endpoint, efficacy is evaluated using multiple exploratory indicators, including the modified Unified Multiple System Atrophy Rating Scale* (mUMSARS). TEAEs incidence was similar to placebo (91%, n=21/23) and ONO-2808 (93%, n=64/69), with no unexpected safety signals. As exploratory efficacy endpoints, in the subgroup of parkinsonism-predominant type (MSA-P), the change from baseline (95% CI) of the mUMSARS score at 24 weeks was 3.90 (1.76 to 6.04) in the placebo group, compared to 1.39 (-0.85 to 3.64) and 1.16 (-1.1 to 3.41) in the medium-dose and high-dose groups, respectively. In brain volume assessment using MRI (magnetic resonance imaging), the ONO-2808 group showed a tendency toward a dose-dependent response to the suppression of brain atrophy progression. These data suggest that ONO-2808 has the potential for continued development, and Ono and its affiliate company Deciphera plan to initiate a pivotal Phase 3 study.

* Unified Multiple System Atrophy Rating Scale (UMSARS): Used as an objective indicator for evaluating the severity and progression of MSA, and mUMSARS (modified UMSARS) is an index extracted from UMSARS in a one-year clinical trial, selecting nine items (speech, eating, dressing, hygiene, walking, excretory function, standing, posture, and gait) as indicators that clinically capture disease progression in a meaningful and sensitive manner [1)].

About ONO-2808-03 Study

The ONO-2808-03 study is a multicenter, randomized, double-blind, placebo-controlled Phase 2 clinical study in early MSA patients within 5 years of symptom onset in Japan and the US. This study consisted of 2 parts. In the core part, 92 participants were randomly allocated to ONO-2808 (3 doses) or placebo at a ratio of 1:1:1:1, and orally received the treatment once daily for 24 weeks in a blinded manner. The objective of the core part is to assess the safety, tolerability, pharmacokinetics, and potential efficacy of ONO-2808 in comparison with placebo. After the completion of the core part,

ONO-2808 will be administered for up to 80 weeks in the extension part to assess the safety, tolerability, and potential efficacy of long-term treatment with ONO-2808.

About Multiple System Atrophy (MSA)

MSA is a progressive neurodegenerative disease, which leads to the gradual loss of neurons in the brain due to abnormal accumulation of a protein called α-synuclein. Major symptoms include Parkinson's symptoms such as muscle stiffness, cerebellar ataxia such as difficulty walking, and autonomic dysfunction such as orthostatic dizziness and urinary incontinence. MSA is a rare and aggressive intractable disease with an average life expectancy of 9 to 10 years [2) - 4)]. It is reported that approximately 80% of patients become aid-requiring walking within 5 years of onset and only 20% of patients survive for at least 12 years [2)]. In Japan, MSA has been designated as an intractable disease, and the number of patients is estimated to be approximately 10,000 as of the end of fiscal year 2019 [5)]. The number of patients in the US is estimated to be 15,000 to 50,000 or approximately 40,000 [6), 7)].

At present, no radical treatment has been established for MSA, and symptomatic treatment and rehabilitation are mainly used to maintain patients' quality of life.

About ONO-2808

ONO-2808 is an orally bioavailable selective agonist to a Sphingosine 1-Phosphate (S1P) receptor 5, one of the S1P receptors, discovered by Ono. S1P5 receptors play an important role in the maintenance of normal functions of nerves, such as the stabilization and regeneration of myelin sheath that covers nerve axons, by promoting the differentiation of oligodendrocytes, a type of glial cells present in the central nervous system such as the brain and spinal cord [8), 9)]. ONO-2808, a selective S1P5 receptor agonist, is expected to alleviate the progression of MSA by promoting remyelination and inhibiting the accumulation of α-synuclein in the central nervous system, which is the cause of MSA.

References:

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