News Release

Chugai Obtains Regulatory Approval for First Tablet for SMA, Evrysdi

- Evrysdi is the only orally available Spinal Muscular Atrophy (SMA) treatment and is approved in over 100 countries
- Approval based on bioequivalence study confirming comparable exposure to currently available dry syrup formulation
- New tablet formulation is expected to provide greater freedom in daily life for people with SMA thanks to simpler administration process and handling

TOKYO, March 27, 2025 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today that it has obtained regulatory approval from the Ministry of Health, Labour and Welfare for "Evrysdi® Tablets 5mg," a new formulation of the spinal muscular atrophy (SMA) treatment Evrysdi [generic name: risdiplam]. Evrysdi is currently the only orally administered treatment for SMA. While the currently available dry syrup can be taken regardless of age and weight, the newly approved tablet formulation provides an additional option for people with SMA who are 2 years of age or older and weigh 20 kg or more.

The currently available dry syrup formulation requires refrigerated storage in a bottle and is administered as a liquid form using measuring tools. As SMA is a disease that causes decreased motor function, some patients may find the currently available administration method challenging. Therefore, there has been a need for a simpler medication option for patients and caregivers. The newly approved "Evrysdi® Tablets 5mg" can be stored at room temperature, making it easier to handle and carry. By offering two formulation choices, patients can select the most appropriate option based on their individual lifestyles, potentially leading to greater freedom in daily life for people with SMA.

"Since its launch, Evrysdi has been chosen by many as the only oral medication for SMA. We are very pleased that a new formulation enabling simpler administration has now been approved," said Dr. Osamu Okuda, Chugai's President and CEO. "Evrysdi tablets not only simplify the administration process but are also more convenient for storage compared to the dry syrup, increasing convenience for long-term outings. We will continue our efforts to deliver this medication, which enables treatment that fits to each individual's lifestyle, to as many people as possible."

This approval is based on a bioequivalence study conducted by Roche. The study confirmed that the 5 mg tablet, when swallowed whole, provides comparable exposure to the existing dry syrup formulation. This means patients taking the tablet can expect

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similar efficacy and safety as the currently available dry syrup. The Evrysdi dry syrup will continue to be available for those on other doses of Evrysdi and for those who prefer the oral solution.

Roche leads the clinical development of Evrysdi as part of a collaboration with the SMA Foundation and PTC Therapeutics.

About Evrysdi
Evrysdi is a survival motor neuron 2 (*SMN2*) splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi is designed to treat SMA by increasing and sustaining the production of the survival motor neuron (SMN) protein. SMN protein is found throughout the body and is critical for maintaining healthy motor neurons and movement. Evrysdi dry syrup was approved in the U.S. in August 2020, in Europe in March 2021. In Japan, it was launched in June 2021, and in September 2024, it received approval for expanded indication for pre-symptomatic treatment and additional dosage for infants under 2 months of age. The new tablet formulation was approved in the United States in February 2025.

About Spinal Muscular Atrophy (SMA)[1]
Spinal muscular atrophy (SMA) is a genetic neuromuscular disease that causes muscle atrophy and muscle weakness due to degeneration of the motor neuron.[2] The causative gene for SMA is the survival motor neuron (*SMN*) gene. The disease develops because of insufficient production of functional SMN protein from *SMN2* gene alone, in addition to the dysfunction of the *SMN1* gene.[3]

Sources:

1. With your SMA. Walking with everyone involved in spinal muscular atrophy (SMA). Available from: https://with-your-sma.jp/. Accessed March 2025. (Japanese only)
2. Farrar MA and Kiernan MC. The genetics of spinal muscular atrophy: progress and challenges. Neurotherapeutics. 2015;12:290-302.
3. Kolb SJ and Kissel JT. Spinal muscular atrophy. Neurol Clin. 2015;33:831-46

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