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Chugai Obtains Regulatory Approval for Evrysdi for the Treatment of Spinal Muscular Atrophy

- Evrysdi has been approved as the first oral drug to be taken at home for spinal muscular atrophy (SMA) in Japan with proven efficacy in adults, children and babies
- The approval is based on the results from two positive pivotal studies evaluating Evrysdi in Types 1, 2 and 3 spinal muscular atrophy (SMA) across infants and adults

TOKYO, June 23, 2021 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it obtained regulatory approval from the Ministry of Health, Labour and Welfare (MHLW) for Evrysdi® Dry Syrup 60 mg (generic name: risdiplam) (hereafter, Evrysdi) for the treatment of spinal muscular atrophy (SMA). Evrysdi had been granted an orphan drug designation in March 2019, and Chugai filed a regulatory application in October 2020.

"We are very pleased that Evrysdi will offer at-home dosing for a wide range of patients from infants to adults as the first oral drug for the treatment of SMA," said Dr. Osamu Okuda, Chugai's President and CEO. "Evrysdi is expected to reduce the treatment burden on people with SMA. We will work diligently for the launch of Evrysdi to make this new therapeutic option for spinal muscular atrophy available as soon as possible."

This approval is based on the results from the FIREFISH study in infants with symptomatic SMA Type 1 and SUNFISH study in children and young adults with SMA Type 2 or 3.

*The description in the Japanese package insert
Product name: Evrysdi® Dry Syrup 60 mg
Generic name: risdiplam
Indications: spinal muscular atrophy
Dosage and administrations: The usual dosage for patients 2 months to less than 2 years of age is 0.2 mg/kg risdiplam administered orally once a day after a meal. The usual dosage for patients weighing 20 kg or more is 5 mg risdiplam administered orally once a day after a meal.

<Reference>
Chugai Receives Orphan Drug Designation for Risdiplam in Spinal Muscular Atrophy (Mar 27, 2019)
https://www.chugai-pharm.co.jp/english/news/detail/20190327150001_602.html

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・SUNFISH study
New two-year data show Roche's Evrysdi (risdiplam) continues to demonstrate improvement or maintenance of motor function in people aged 2-25 with Type 2 or Type 3 Spinal Muscular Atrophy (SMA) (Press release by Roche issued on March 16, 2021)
https://www.roche.com/media/releases/med-cor-2021-03-16.htm

・FIREFISH study
Roche's Evrysdi continues to improve motor function and survival in babies with Type 1 Spinal Muscular Atrophy (SMA) (Press release by Roche issued on April 15, 2021)
https://www.roche.com/media/releases/med-cor-2021-04-15.htm

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 was approved in the U.S. in August 2020 and in Europe in March 2021.

About spinal muscular atrophy (SMA)
Spinal muscular atrophy (SMA) is a genetic neuromuscular disease that causes muscule atrophy and muscle weakness due to degeneration of the motor neuron.[1)] It is the most frequently observed life-threatening genetic disease in infants.[2)] The incidence of SMA from infancy to childhood is one to two in 100,000 individuals.[3)] 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 genes alone, in addition to the dysfunction of the SMN1 gene.[4)]

Sources

1) Farrar MA and Kiernan MC. The genetics of spinal muscular atrophy: progress and challenges. Neurotherapeutics. 2015;12:290-302.
2) Cure SMA. About SMA. 2018. Available from: http://www.curesma.org/sma/about-sma/. Accessed June 2021.
3) Japan Intractable Diseases Information Center. Available from: https://www.nanbyou.or.jp/. Accessed June 2021. (Japanese only)
4) Kolb SJ and Kissel JT. Spinal muscular atrophy. Neurol Clin. 2015;33:831-46.

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