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CHUGAI

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Chugai Launches Evrysdi Dry Syrup 60 mg for the Treatment of Spinal Muscular Atrophy

- Evrysdi has been launched as the first oral drug for spinal muscular atrophy (SMA) with proven efficacy in adults, children and babies, enabling treatment at home

TOKYO, August 12, 2021 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that it launched Evrysdi® Dry Syrup 60 mg (generic name: risdiplam) for the treatment of spinal muscular atrophy (SMA). Evrysdi had been approved on June 23, 2021, and was listed on the national health insurance (NIH) reimbursement price list today.

“We are very pleased to launch Evrysdi as the first at home drug for the treatment of SMA,” said Dr. Osamu Okuda, Chugai’s President and CEO. “Evrysdi has shown efficacy in a wide age range from babies to adults, and will offer at-home dosing. We are committed to promoting appropriate use of Evrysdi so that we may deliver unprecedented value to people with SMA and their families through this new therapeutic option.”

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

<Reference>
Chugai Obtains Regulatory Approval for Evrysdi for the Treatment of Spinal Muscular Atrophy (Jun 23, 2021)
https://www.chugai-pharm.co.jp/english/news/detail/20210623170000_832.html

・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

*The description in the Japanese package insert
Product name: EVRYSDI® Dry Syrup 60 mg

CHUGAI PHARMACEUTICAL CO., LTD. Corporate Communications Dept.
1-1, Nihonbashi-Muromachi 2-chome, Chuo-ku Tokyo, 103-8324 Japan
Media Relations Group TEL:+81-(0)3-3273-0881 E-mail:pr@chugai-pharm.co.jp
Investor Relations Group TEL:+81-(0)3-3273-0554 E-mail:ir@chugai-pharm.co.jp

Generic name: risdiplam

Indications: spinal muscular atrophy

Dosage and administrations: The usual dosage for patients aged 2 months to less than 2 years is 0.2 mg/kg risdiplam administered orally once a day after a meal. The usual dosage for patients aged 2 years or more is 0.25mg/kg for those weighing less than 20 kg, and 5mg for those weighing 20 kg or more, both administered orally once a day after a meal.

Date of approval: June 23, 2021

Date of NHI reimbursement price listing: August 12, 2021

Date of launch: August 12, 2021

Drug price: Evrysdi® Dry Syrup 60 mg JPY 974,463.70/bottle

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 muscular 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 August 2021.
3) Japan Intractable Diseases Information Center. Available from: https://www.nanbyou.or.jp/. Accessed August 2021. (Japanese only)
4) Kolb SJ and Kissel JT. Spinal muscular atrophy. Neurol Clin. 2015;33:831-46.

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