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## Chugai Files a New Drug Application for Risdiplam as the First Oral Drug for Spinal Muscular Atrophy in Japan

- The application is based on the results from two positive studies evaluating risdiplam in Types 1, 2 and 3 spinal muscular atrophy across infants and adults.
- The application is subject to a priority review in Japan.

TOKYO, Oct 15, 2020 -- [Chugai Pharmaceutical Co., Ltd.](#) (TOKYO: 4519) announced that it filed a new drug application with the Ministry of Health, Labour and Welfare (MHLW) for risdiplam, an oral survival motor neuron-2 (SMN2) splicing modifier, for the treatment of spinal muscular atrophy (SMA). Risdiplam received orphan drug designation from the MHLW on Mar 27, 2019, and the application is subject to a priority review.

“As potentially the first oral drug for SMA, risdiplam demonstrated clinically meaningful treatment effects across infants and adults in two clinical studies for Types 1, 2 and 3 SMA,” said Chugai’s President and COO, Dr. Osamu Okuda. “I believe that risdiplam will make a significant contribution to the treatment of SMA with its proven efficacy. We will work to obtain the regulatory approval for risdiplam to provide this new oral therapy for people with SMA as soon as possible.”

This application 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. SUNFISH is the only placebo-controlled trial to include children, teenagers and adults with SMA.

<Reference>

• FIREFISH study

Roche’s risdiplam shows significant improvement in survival and motor milestones in infants with Type 1 spinal muscular atrophy (SMA) (Press release by Roche issued on April 28, 2020)

<https://www.roche.com/media/releases/med-cor-2020-04-28.htm>

• SUNFISH study

Roche’s risdiplam showed significant improvement in motor function in people aged 2-25 with type 2 or 3 spinal muscular atrophy (Press release by Roche issued on February 6, 2020)

<https://www.roche.com/media/releases/med-cor-2020-02-06.htm>

Roche announces 2-year risdiplam data from SUNFISH and new data from JEWELFISH in infants, children and adults with spinal muscular atrophy (SMA) (Press release by Roche issued on June 12, 2020)

<https://www.roche.com/media/releases/med-cor-2020-06-12.htm>

•Orphan drug designation

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](https://www.chugai-pharm.co.jp/english/news/detail/20190327150001_602.html)

### **About risdiplam**

Risdiplam is an investigational, oral medicine designed to increase and sustain survival motor neuron (SMN) protein levels in the central nervous system and throughout the body. It is designed to help the SMN2 gene produce more functional SMN protein to better support motor neurons and muscle function. Risdiplam was approved in the U.S. in August 2020. Orphan Drug status has been granted in the EU, and the European Medicines Agency (EMA) granted PRIME (PRiority MEdicines) designation for risdiplam in December 2018 for the treatment of SMA. In Japan, risdiplam has been designated as an orphan drug for the treatment of SMA in March 2019.

### **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 spinal motor nerve cells.<sup>1)</sup> It is the most frequently observed life-threatening genetic disease in infants.<sup>2)</sup> The incidence of SMA from infancy to childhood is one to two in 100,000 individuals.<sup>3)</sup> 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.<sup>4)</sup>

### 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 October 2020.
- 3) Japan Intractable Diseases Information Center. Available from: <https://www.nanbyou.or.jp/>. Accessed October 2020. (Japanese only)
- 4) Kolb SJ and Kissel JT. Spinal muscular atrophy. *Neurol Clin*. 2015;33:831-46.

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