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Chugai Receives Orphan Drug Designation for Risdiplam in Spinal Muscular Atrophy

- Risdiplam is expected to be the first oral medicine for the treatment of spinal muscular atrophy, a genetic disease causing debilitation due to muscle weakness.
- Global phase II/III clinical studies are currently ongoing. Chugai will file a regulatory application in Japan in 2020.

TOKYO, March 27, 2019 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced that risdiplam (development code: RG7916), an oral survival motor neuron-2 (SMN2) splicing modifier under development for an expected indication of spinal muscular atrophy (SMA), received orphan drug designation by the Ministry of Health, Labour and Welfare. Risdiplam is systemically distributed and designed to durably increase SMN protein levels in the central nervous system and throughout the body. Global phase II/III clinical studies (FIREFISH, SUNFISH) with risdiplam are currently ongoing in Japan.

“SMA is the leading cause of death in infants with genetic diseases for which therapeutic options are limited,” said Dr. Yasushi Ito, Chugai’s Executive Vice President, Co-Head of Project & Lifecycle Management Unit. “We intend to proceed with the ongoing clinical studies in cooperation with Roche so that we can deliver risdiplam to patients with SMA as the first oral systemic medicine.”

<Reference>
Roche announces new data for risdiplam in Spinal Muscular Atrophy (SMA) at the World Muscle Society Congress (Roche media release dated October 3, 2018)
https://www.roche.com/media/releases/med-cor-2018-10-03.htm

About risdiplam
Risdiplam is an investigational, oral medicine designed to increase 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. The European Medicines Agency (EMA) granted PRIME (PRIority MEdicines) designation for risdiplam in December 2018 for the treatment of SMA. Orphan Drug status has also been granted in the EU, US and Switzerland as well as Fast Track Designation by the US Food and Drug Administration (FDA). Roche is leading the clinical development of risdiplam in collaboration with the SMA Foundation and PTC Therapeutics.

About SMA
Spinal muscular atrophy (SMA) is a genetic neuromuscular disease which causes muscular atrophy and muscle weakness due to degeneration of the spinal motor nerve cells.[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

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in 100,000 individuals.[3] The causative gene for SMA is the SMN gene. The disease develops because of insufficient production of functional SMN protein from SMN2 genes alone, in addition to dysfunction of the SMN1 gene.[4]

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 March 2019.
3) Japan Intractable Diseases Information Center. Available from: http://www.nanbyou.or.jp/entry/135. Accessed March 2019. (Japanese only)
4) Kolb SJ and Kissel JT. Spinal muscular atrophy. Neurol Clin. 2015;33:831-46.

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