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Risdiplam Meets Primary Endpoint in Pivotal FIREFISH Trial in People with Type 1 Spinal Muscular Atrophy (SMA)

TOKYO, January 23, 2020 -- Chugai Pharmaceutical Co., Ltd. (TOKYO: 4519) announced today positive top line results from the pivotal Part 2 of FIREFISH, a study evaluating risdiplam in people with Type 1 spinal muscular atrophy (SMA).

The study met its primary endpoint of the proportion of infants sitting without support for at least 5 seconds at 12 months of treatment assessed by the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development – Third Edition (BSID-III). No treatment related safety findings leading to study withdrawal have been seen in any risdiplam trial to date. Safety for risdiplam was consistent with its known safety profile and no new safety signals were identified.

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

Roche's risdiplam meets primary endpoint in pivotal SUNFISH trial in people with type 2 or 3 spinal muscular atrophy (Press release issued by Roche on December 11, 2019)
https://www.roche.com/media/releases/med-cor-2019-11-11.htm

About FIREFISH trial
FIREFISH is a two part, open, one arm pivotal study in infants aged 1-7 months with Type 1 SMA. Part 1 (n=21) determined the dose for the confirmatory Part 2. Part 2 (n=41) evaluated efficacy measured as the proportion of infants sitting without support for at least 5 seconds at 12 months of treatment assessed by the Gross Motor Scale of the BSID-III.
Roche is leading the clinical development of risdiplam in collaboration with the SMA Foundation and PTC Therapeutics.

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). In Japan, risdiplam was designated as an orphan drug for the treatment of SMA in March 2019.

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About spinal muscular atrophy (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 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)]

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

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