

Roche's Risdiplam meets primary endpoint in FIREFISH trial

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Roche has announced positive topline results from the pivotal Part 2 of the FIREFISH study, evaluating risdiplam in infants aged 1-7 months with Type 1 spinal muscular atrophy (SMA). The primary outcome measure of the study was the proportion of infants sitting without support for at least five 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). Safety for risdiplam in the FIREFISH study was consistent with its known safety profile and no new safety signals were identified. To date, more than 400 patients have been treated with risdiplam across all studies, with no treatment related safety findings leading to study withdrawal in any risdiplam trial.

"This large, global trial confirms the efficacy of risdiplam in an advanced and difficult-to-treat population, including many infants whose disease had already progressed significantly before starting treatment," said Levi Garraway, M.D., Ph. D., Roche's Chief Medical Officer and Head of Global Product Development. "We are very encouraged by these results and we look forward to sharing them with regulators. We also thank the entire SMA community for their continued partnership."

Risdiplam is an investigational survival motor neuron-2 (SMN2) splicing modifier, designed to increase and sustain SMN protein levels throughout the central nervous system and in peripheral tissues. Roche leads the clinical development of risdiplam as part of a collaboration with the SMA Foundation and PTC Therapeutics. Data from the FIREFISH study will be presented at an upcoming medical congress.

Risdiplam is being studied in a broad clinical trial programme in SMA, with patients ranging from birth to 60 years old, and includes patients previously treated with SMA-targeting therapies. The clinical trial population represents the broad real-world spectrum of people living with this disease with the aim of ensuring access for all appropriate patients.

On November 11 2019, Roche announced positive results from Part-2 of SUNFISH, a study evaluating the efficacy and safety of risdiplam in people aged 2-25 years with Type 2 or 3 SMA. Also in November 2019, the U.S Food and Drug

Administration granted Priority Review for risdiplam with a decision for approval by May 24, 2020.