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FDA grants priority review to Roche’s risdiplam for SMA

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Filing submission includes 12-month data from pivotal FIREFISH and SUNFISH trials in a broad population ofpeople living with Types 1, 2 or 3 SMA

Roche has announced that the U.S. Food and Drug Administration (FDA) has accepted the New Drug Application (NDA) and granted Priority Review for risdiplam, an investigational survival motor neuron-2 (SMN-2) splicing modifier for SMA. Risdiplam is designed to increase and sustain SMN protein levels both throughout the central nervous system and peripheral tissues of the body. The FDA is expected to make a decision on approval by May 24, 2020.

“The FIREFISH and SUNFISH trials were designed to represent the real world spectrum of people living with SMA and include many people previously underrepresented in clinical trials,” said Levi Garraway, M.D., Ph.D., Roche’s Chief Medical Officer and Head of Global Product Development. “We look forward to working closely with the FDA to explore broad access to risdiplam for all individuals in the community who might benefit.”

If approved, risdiplam, an orally administered liquid, would be the first at-home administered medicine for people living with SMA. In addition to the studies included in the NDA submission, risdiplam is being studied in a broad clinical trial programme in SMA, with patients ranging from newborns to 60 years old, and includes patients previously treated with SMA therapies.

Roche leads the clinical development of risdiplam as part of collaboration with the SMA Foundation and PTC Therapeutics, and would commercialize the medicine in the United States if approved.

Priority Review designation is granted to medicines that the FDA considers to have the potential to provide significant improvements in the safety and effectiveness of the treatment, prevention or diagnosis of a serious disease. Previously, the FDA also granted Orphan Drug Designation for risdiplam in January 2017, followed by Fast Track Designation in April 2017.