April 7, 2020

Dear members of SMA Europe,

As part of our ongoing partnership and following your request to receive important information about the risdiplam clinical development program, we wanted to update you on the status of regulatory filings and reviews of risdiplam around the world.

In November 2019, the U.S. Food and Drug Administration (FDA) granted Priority Review for risdiplam with a decision for approval expected by May 24, 2020. As agreed with the FDA, in February of this year we submitted additional data which included 12-month data from the pivotal SUNFISH Part 2 study, the only placebo-controlled study ever undertaken in people aged 2-25 years with Type 2 or 3 SMA. These data were presented at the 2nd International Scientific and Clinical Congress on Spinal Muscular Atrophy in February 2020.

The FDA recently informed us that the volume of additional data submitted by Roche requires a three-month review extension of the New Drug Application (NDA) of risdiplam. We, therefore, expect a decision from the FDA by August 24, 2020. We want to reassure you that we are working collaboratively with the FDA in order to support their review of the NDA for risdiplam.

Additionally and as part of our continued commitment to the global SMA community, we are pleased to announce that regulatory filings have been submitted in Brazil, Chile, Indonesia, Russia, South Korea and Taiwan. Furthermore, a filing submission in China is imminent and we are currently on track to submit a Marketing Authorization Application (MAA) to the European Medicines Agency in mid-2020, as well as in other countries around the world.

We appreciate that the SMA community is facing a number of new and unexpected challenges during these unprecedented times. Please know that we are working expeditiously to make risdiplam available to all patients that can benefit from the treatment, as soon as possible.

We want to thank everyone in the community, especially the patients and families who have participated in the risdiplam clinical development programme – it is because of you that we are here today, and we are incredibly grateful.

If you have any questions about this update, please do not hesitate to contact me.

Sincerely,

Fani Petridis

Fani Petridis, on behalf of the Roche Global SMA Team
Senior Global Patient Partnership Director, Rare Diseases