January 14, 2020

Dear Members of SMA Europe,

Following your recent request to receive more information about Roche's Pre-Approval Access / Compassionate Use (PAA/CU) plans for the investigational medicine risdiplam, we would like to provide you with the below information.

While there have been very important treatment advances for patients with SMA in the last few years, we recognise that there continues to be high unmet medical need within the community and patients experiencing life-threating or severe conditions may not have access or be eligible to satisfactory treatments or to ongoing clinical trials.

As a result of this remaining need, we are pleased to inform you that Roche has initiated a global PAA/CU programme for risdiplam in countries where applicable laws and regulations allow such programmes and which fulfill the criteria based on applicable company policy. A PAA/CU programme enables patients who are facing the most urgent medical need and have no other treatment options, to access our investigational therapies before these receive regulatory approval.

In countries where the PAA/CU programme already is or will be implemented, Roche is currently offering patients with the most urgent medical need, Type 1 SMA, the opportunity for access to risdiplam based on their treating physician's decision. Being mindful, however, that patients with other types of SMA are also facing life-endangering situations, the programme will be expanded to patients with Type 2 SMA at the moment of filing of the regulatory application for risdiplam in each country. This means that in participating countries within the European Union, the programme is already available for patients with Type 1 SMA and will expand to patients with Type 2 SMA upon filing of our Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA), which is currently planned for mid 2020.

It is important to highlight that PAA/CU for investigational medicines must always comply with applicable country-specific laws and regulations, which differ across countries, and as a result, local variations in the programme will occur. We recommend that people interested in accessing risdiplam via PAA/CU, discuss their options with their treating physician. The decision to apply for the programme is one that should be made by the treating physician. If the risdiplam PAA/CU programme is deemed to be the best path forward, requests must be submitted by the physician to the local Roche affiliate.

Achieving broad and sustainable access through regulatory approval and reimbursement is our key priority, and we are collaborating with Health Authorities, government agencies and other key stakeholders around the world with the aim to make risdiplam available to all patients who can benefit from the treatment as soon as possible.

We want to thank you for your collaboration and we look forward to providing further updates about our programme as they become available.

Sincerely,

Fani Petridis

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