

4th June 2025

Dear Members of SMA Europe,

As part of our ongoing partnership and following your request to receive updates about risdiplam, we are pleased to share that the European Commission (EC) has approved a new, room-temperature stable tablet formulation of risdiplam.

The approval of the 5 mg tablet provides an alternative to the already approved oral solution. The risdiplam tablet can either be swallowed whole or dispersed in water and can be taken with or without food. It does not require refrigeration when stored at room temperature. In response to SMA community feedback, the tablet was developed to support further flexibility and choice for people living with SMA.

The tablet formulation is approved for people living with SMA aged two years or older, who weigh more than 20kg (44 lbs) and are able to swallow without the use of a feeding tube. For more details, please see our press release [here](#).

### **Evidence supporting the EC approval**

The approval of the risdiplam tablet was based on the results of a bioequivalence study ([NCT04718181](#)) which demonstrated that the tablet formulation and the oral solution are bioequivalent. This means that the tablet provides the same risdiplam exposure, and therefore it is expected to have the same established efficacy and safety as the oral solution.

### **What happens next?**

The EC approval means that the 5 mg risdiplam tablet is approved for use across all 27 European Union member states, as well as Iceland, Norway and Liechtenstein. In light of the approval, health authorities in each country will review reimbursement of the risdiplam tablet for use in their national health system. Roche is actively engaging with reimbursement and health technology assessment bodies across Europe to support access to the risdiplam tablet as quickly as possible.

The risdiplam oral solution will remain available for those receiving other doses of risdiplam and for those who may prefer the oral solution. As part of this label extension, the risdiplam summary of product characteristics (SmPC) has been updated to include guidance on tablet administration and storage. Additionally, the SmPC was also updated with additional guidance (driven by SMA community requests, and based on the food-effect assessment in study [NCT04718181](#)), that risdiplam (oral solution or tablet) can be taken with or without food.

We would like to thank the SMA community once again for your continued partnership, which enables milestones like this one. Without your trust, advice, and participation in clinical research, none of this would be possible.

Below we have addressed some frequently asked questions about the risdiplam tablet. If you have any additional questions, please do not hesitate to reach out.

Sincerely,



Louisa Townson, on behalf of the Roche Global SMA Team  
Global Patient Partnership

## **Frequently Asked Questions**

### **How is the risdiplam tablet administered?**

- The risdiplam tablet can either be swallowed whole or mixed in a small amount of water.
- Note: the tablet should NOT be cut, crushed, chewed, or dissolved on the tongue, or mixed in food.

### **How often should the risdiplam tablet be taken?**

- The risdiplam tablet is taken once per day, and at approximately the same time every day.
- Risdiplam (tablet or oral solution) can be taken with or without food.

### **Is the tablet the same as the liquid?**

- The tablet contains the same active “ingredient” as the liquid, though some additives (e.g., sweeteners) may differ.
- To verify that the tablet and oral solution behave in the same way when administered, the risdiplam tablet was compared to the oral solution in a clinical trial ([NCT04718181](#)). The study showed that whether swallowed whole or dispersed (mixed) in water, the tablet resulted in the same concentration of risdiplam in the body as the oral solution.
- The safety of risdiplam was also found to be the same in either tablet or liquid form.

### **How big are the tablets?**

- Risdiplam tablets are 6.5 mm (0.25 inches) in diameter and 4.1 mm (0.15 inches) in height. For context, they are roughly about half the size of a kernel of corn.

### **Can the tablet be used with feeding tubes?**

- If administration through a feeding (nasogastric/gastrostomy) tube is required, the risdiplam oral solution should be used.
- Finding a suitable treatment option to best fit your needs should be discussed with your doctor.

### **What if I cannot swallow a tablet?**

- If there are difficulties with swallowing the tablet whole, there is the possibility to mix the tablet in water instead, and in this instance, a straw may be used.
- Finding a suitable treatment option to best fit your needs should be discussed with your doctor and instructions should be followed from patient information leaflets provided.

### **How should the tablet be stored?**

- The risdiplam tablet is room-temperature stable (20°C to 30°C).
- Risdiplam tablets are supplied in blister packs (with 4 blisters each with 7 tablets). The tablets should be stored in the original blister packs. They should not be stored in a pill box or other container, as they may be affected by moisture or light.