

Spinal Muscular Atrophy (SMA)

**A clinical trial of risdiplam for the treatment of very young babies diagnosed with spinal muscular atrophy but are not yet presenting with symptoms**

A Study of Risdiplam in Infants With Genetically Diagnosed and Presymptomatic Spinal Muscular Atrophy

**Trial Status**  
Active, not recruiting

**Trial Runs In**  
7 Countries

**Trial Identifier**  
NCT03779334  
2018-002087-12,2023-506009-20-00  
BN40703

The source of the below information is the publicly available website ClinicalTrials.gov. It has been summarised and edited into simpler language.

**Trial Summary:**

A global study of oral risdiplam in pre-symptomatic participants with spinal muscular atrophy (SMA).

**Hoffmann-La Roche**  
Sponsor

**Phase 2**  
Phase

**NCT03779334 2018-002087-12,2023-506009-20-00 BN40703**  
Trial Identifiers

**Eligibility Criteria:**

**Gender**  
All

**Age**  
≥1 Day & ≤ 6 Weeks

**Healthy Volunteers**  
No

**1. Why is the BN40703 clinical trial needed?**

Spinal muscular atrophy (SMA) is a rare genetic disorder that causes progressive weakness and wasting (atrophy) in muscles used for movement (skeletal muscles). It is caused by the loss of certain specialized nerve cells (motor neurons) in the brain and spinal cord that control muscle movement. If untreated, this results in loss of movement and even death. Early treatment has been associated with better outcomes. Risdiplam (also known as RO7034067) is the first drug to be given by mouth (orally) for treatment of SMA. Risdiplam is approved (also known as Evrysdi) for use in participants with SMA in

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approximately 81 countries. However, it is considered as an experimental drug (referred to as study medication) for the purpose of this study.

This clinical trial aims to understand the effects, good or bad, of risdiplam in very young babies (infants) with SMA.

## **2. How does the BN40703 clinical trial work?**

This clinical trial has recruited infants with a health condition called spinal muscular atrophy (SMA). Infants who take part in this clinical trial will be given risdiplam once a day for up to 5 years. The trial doctor will see the infants together with parents/caregiver 15 times in the clinic for the first two years, followed by 2 visits per year for each additional year. These visits will include administration of the daily dose of risdiplam, collecting blood samples, conducting study specific tests such as muscle function tests, electrocardiogram (ECG) measurements, eye and vision tests. Total time of participation in the clinical trial will be at least 5 years, the participant may continue treatment till the end of study if risdiplam is not commercially available in the country of the site. Participants can stop trial treatment and leave the clinical trial at any time.

## **3. What are the main endpoints of the BN40703 clinical trial?**

The main clinical trial endpoint (the main results measured in the trial to see if the drug has worked) is to determine the percentage of participants that can sit without support after 12 months of treatment. This will be assessed in a limited set of infants. The other clinical trial endpoints are:

- Percentage of participants developing clinical features of SMA.
- Time to death and/or permanent ventilation.
- Percentage of participants alive and alive without permanent ventilation.
- Percentage of participants who are sitting without support for 5 seconds and for 30 seconds, and who are standing alone for at least 3 seconds and walking alone at least 3 steps. This will be assessed in all participants.
- Percentage of participants who can swallow and feed by mouth.

Additionally, the safety of the study medication will be determined along with its effects on the human body.

## **4. Who can take part in this clinical trial?**

Infants could take part in this clinical trial if they were no older than 42 days of age at the time of first dose (Day 1); a minimum age of 7 days at first dose was required for the first infant to be enrolled and were diagnosed with SMA and did not show any symptoms at the time of screening. Infants were not able to take part in this trial if they had any significant existing disease conditions or inadequate access to blood via their veins. Participants also

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were not able to take part if they have received any treatment such as *SMN2*-targeting antisense oligonucleotide, *SMN2*-splicing modifier, or gene therapy in the past.

## **5. What treatment will participants be given in this clinical trial?**

Every infant who joins this clinical trial will receive once daily a dose of risdiplam in the morning. The participants who can swallow will receive it via a syringe inserted between gum and cheek of the participant. Participants who are unable to swallow the study medication and who have a feeding tube in situ should receive the study medication by bolus via the tube.

This is an open-label trial, which means everyone involved, including the participant and the clinical trial doctor, will know the clinical trial treatment the participant has been given.

## **6. Are there any risks or benefits in taking part in this clinical trial?**

There may be risks or side effects related to the study drug or other study procedures that are unknown at this time. Most trials involve some risks to the participant. In all clinical studies so far, risdiplam has been assessed to be well-tolerated. People who would like to participate will be told about any risks and benefits of taking part in the clinical trial, as well as any additional procedures, tests, or assessments they will be asked to undergo. All of these will be described in an informed consent document (a document that provides people with the information they need to decide to volunteer for the clinical trial).

### **Risks associated with the clinical trial drug**

Participants may have side effects (an unwanted effect of a drug or medical treatment) from the drug used in this clinical trial. Side effects can be mild to severe, even life-threatening, and vary from person to person. Participants will be closely monitored during the clinical trial; safety assessments will be performed regularly.

### **Risdiplam**

Participants will be told about the known side effects of risdiplam, and possible side effects based on human and laboratory studies or knowledge of similar drugs. Risdiplam will be given as a solution by mouth or by a feeding tube. Participants will be told about any known side effects of oral administration or administration via a feeding tube.

### **Potential benefits associated with the clinical trial**

Participants' health may or may not improve from participation in the clinical trial. Still, the information collected may help other people with similar medical conditions in the future.