ForPatients
*by Roche*

Spinal Muscular Atrophy (SMA)

# WeSMA is a long-term follow-up study for individuals taking Evrysdi (risdiplam)

Long-Term Follow-Up Study of Risdiplam in Participants With Spinal Muscular Atrophy (SMA)

| Trial Status | Trial Runs In | Trial Identifier |
|---|---|---|
| Recruiting | 2 Countries | NCT05232929 ML43702 |

*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 multi-center, longitudinal, prospective, non-comparative study to investigate the long-term safety and effectiveness of risdiplam, prescribed based on clinician judgment as per the Evrysdi® U.S. Package Insert (USPI) in adult and pediatric participants with spinal muscular atrophy (SMA). In this study, participants will be followed for up to 5 years from enrollment or until withdrawal of consent, loss to follow-up, or death. Participants who discontinue risdiplam may still remain in the study, if they agree to continue participating in the follow-up assessments.

| | |
|---|---|
| **Genentech, Inc.**<br>Sponsor | **Phase 4**<br>Phase |

**NCT05232929 ML43702**
Trial Identifiers

## *Eligibility Criteria:*

| Gender | Age | Healthy Volunteers |
|---|---|---|
| All | All Ages | No |

### 1. Why is the WeSMA clinical trial needed?

Our objective is to further understand long-term safety and how well risdiplam works for people of all ages living with SMA. This study will help doctors and researchers understand the long-term use and will help others with SMA by contributing to clinical research on Evrysdi.

**2. How does the WeSMA clinical trial work?**

WeSMA is a decentralized clinical trial meaning that participants will have 2 treatment options:

- Participants can enroll at a clinical research site. This is for individuals who already receive routine care from a participating WeSMA clinical research site.
- Participants can enroll in the WeSMA study remotely.

Study participants will have study visits twice during the first year (at month 6 and month 12) and once a year after that, up to 5 years. The visits capture data from the participant`s medical record from the physical therapy assessments of movement, strength, and function that are typically done during participant`s routine visits, these include World Health Organization (WHO) Gross Motor Milestones, Hammersmith Infant Neurological Exam (HINE-2), Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND), Revised Upper Limb Module (RULM), Hammersmith Functional Motor Scale (HFMSE), Motor Function Measure 32 Items (MFM-32), and 6 Minute Walk Test (6MWT).

In addition participants and or legal guardians/caregivers will be asked to download a study app on their smartphone or mobile device to complete questionnaires and surveys. This will also be done twice during the first year (at month 6 and month 12) and once a year after that up to 5 years.

This will be done from the comfort of participant`s home. In addition, participants may have the option to do telehealth virtual visits with the study site when they are not able to visit the clinic in person.

The total time of participation in the clinical trial will be up to 5 years. Participants can leave the clinical trial at any time.

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

The main clinical trial endpoints are to see how well and safe a treatment works in practice over a long period of time (up to 5 years).

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

People can take part in this trial if they have a confirmed diagnosis of 5q-AR SMA, are able to provide informed consent or assent when appropriate, and are currently prescribed Evrysdi which is FDA approved for patients of all ages. This means people can participate as young as 1 day old to adults of all ages.

People may not be able to take part in this trial if they have hypersensitivity (allergic) to Evrysdi or have participated in other clinical trials for Evrysdi (Firefish, Sunfish, Jewelfish, and Rainbowfish).

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

This clinical trial is for people that are currently receiving Evrysdi

*** Evrysdi will not be provided to participants by Genentech, they should continue to take Evrysdi as prescribed by their doctor"**

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

The procedures in this study are typically standard of care practice for people living with SMA, meaning most procedures are already being done during routine doctor visits. The risk from being in this study is not greater than the risks related to routine medical care or the natural progression of the health condition. This will be described in detail in the informed consent document (a document that provides people with the information they need to decide to volunteer for the clinical trial).

Participants may have side effects (an unwanted effect of a drug or medical treatment) from taking Evrysdi. Side effects can be mild to severe, even life-threatening, and vary from person to person. Participants are encouraged to inform their doctor if they are experiencing side effects.

Participants will be told about the known side effects of Evrysdi and possible side effects based on human and laboratory studies or knowledge of similar drugs.

**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.