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
Active, not recruiting	1 Country	NCT05232929 ML43702

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

Long-term Follow-up Study of Patients With Spinal Muscular Atrophy Receiving Risdiplam Treatment

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 SMA. In this study, participants will be followed for the duration of the study 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. An optional sub study will assess the feasibility, acceptability, and adherence of remote assessment of motor and bulbar functions in participants with SMA using wearable and smartphone-based biosensors. Approximately 39 participants from the main study are planned to be enrolled in the sub study.

Genentech, Inc.
Sponsor

Phase 4
Phase

NCT05232929 ML43702
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	All Ages	No

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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.

Inclusion Criteria:

- Clinical diagnosis of SMA
- Prescribed or continued risdiplam based on clinical judgment of prescriber, as per the Evrysdi® USPI, after U.S. FDA approval (07 August 2020)

Sub study:

Participants in the main study (ML43702) are eligible to be included in the sub study only if all of the following criteria apply:

- Age # 10 years at the time of signing Informed Consent Form
- Willingness and ability to use smartphone technology
- Fluency in English (written and spoken as per the judgment of the investigator)
- Willingness and ability to complete all aspects of the sub study, including respiration and swallowing measurements using respiratory inductance plethysmography (RIP) belts and surface electromyography (sEMGs)
- Hammersmith Functional Motor Scale-Expanded (HFMSE) > 10
- Functional Oral Intake Scale (FOIS) >1
- Willingness to be video recorded during in-clinic SMA-DAT tasks and ADAM sensor assessments and training
- Availability of a caregiver who is willing to participate throughout this sub study

Exclusion Criteria:

- Hypersensitivity to risdiplam
- Participated in a registrational trial for risdiplam (i.e., Firefish [NCT02913482], Sunfish [NCT02908685], Jewelfish [NCT03032172], and Rainbowfish [NCT03779334])

Sub study:

Potential participants will be excluded from the sub study if they meet any of the following criteria:

- Current respiratory infection that, in the opinion of the investigator, would interfere with the conduct of the sub study
- History or known presence of any significant psychiatric disorder such as schizophrenia, bipolar disorder, or substance use disorders
- Current active clinically significant anxiety or depressive disorder, as judged by the investigator, that is likely to impede a participant's ability to participate in the sub study
- Wearing a pacemaker (due to incompatibility with the ADAM sensor)
- Inability to tolerate the performance of sub study procedures