ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

An observational study to describe the fertility journey of Evrysdi (risdiplam)-treated adult male individuals with spinal muscular atrophy

An observational study to look at the effects of Evrysdi (risdiplam) on fertility in adult male with spinal muscular atrophy

Trial Status	Trial Runs In	Trial Identifier
Recruiting	1 Countries	ML44914

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 study to describe the fertility journey of risdiplam-treated adult male individuals with spinal muscular atrophy

Genentech	Phase IV
Sponsor	Phase

ML44914
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
Male	18 to 50 Years of age	No

1. Why is the MARLIN clinical study needed?

Spinal muscular atrophy (SMA) is an inherited disorder which results in weakness and wasting of muscles used for movement. It is caused by the loss of certain specialized nerve cells in the brain and spinal cord that control muscle movement, known as motor neurons. Risdiplam is approved by the U.S. Food and Drug Administration (FDA) for the treatment of pediatric and adult patients with SMA. The purpose of this study, the MARLIN study, is to gather information about the fertility journey of adult male individuals with SMA who are taking or have taken risdiplam. The information that is learned may help other people with SMA in the future.

2. How does the MARLIN clinical study work?

Participation in this study is fully remote and involves the completion of questionnaires using an electronic platform. It does not include any procedures or doctor visits.
The questionnaires include the following topics: demographics, medical history and medications, risdiplam use, sexual history, and fertility journey (including any tests and treatments the participant has undergone in their attempt to conceive a child). Each questionnaire takes approximately 20-45 minutes to complete.

Participants who are actively trying to conceive a child will complete an initial questionnaire and subsequently complete a follow-up questionnaire once a year for up to 3 years. Participants who conceived before study enrollment but after treatment with risdiplam will only complete the initial questionnaire.

Participants who successfully conceive or stop trying to conceive before the end of the 3 years will be considered to have completed the study. If a participant has conceived at the end of 3 years but the outcome of the pregnancy is not yet known, they will complete an additional follow-up questionnaire to capture this outcome.

If the participant's sexual partner, surrogate, or gestational carrier agrees to take part in the study (optional), they will also be asked to complete similar questionnaires.

Participants can choose to withdraw and leave the clinical study at any time.

3. What are the main endpoints of the MARLIN clinical study?

The main clinical study endpoint is whether risdiplam-exposed adult males with SMA conceive.

The other clinical study endpoints are: (1) presence of confounding factors that may impact fertility, (2) fertility-related healthcare resource utilization, management and treatment decisions, and (3) outcomes of pregnancies.

4. Who can take part in this clinical study?

To participate in this clinical study, you must be an adult male between the ages of 18 and 50 years, diagnosed with SMA, and either currently receiving risdiplam treatment OR previously received risdiplam treatment. You must be actively trying to conceive a child OR have previously conceived a child in the past after taking risdiplam. Additionally, you must be able to complete a questionnaire in English, with or without assistance, and have access to a smartphone with internet connection.

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

There is no treatment given in this study.

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

Participants will not receive any direct medical benefit from participating in this study but the information that is learned may help other people with SMA in the future.