ForPatients
by Roche

Spinal Muscular Atrophy (SMA)

A clinical trial to compare how risdiplam is processed by the body in people with mild or moderate liver disease compared to people with healthy livers

A Study to Investigate the Effect of Hepatic Impairment on the Pharmacokinetics and Safety and Tolerability of a Single Oral Dose of Risdiplam Compared to Matched Healthy Participants With Normal Hepatic Function

Trial Status	Trial Runs In	Trial Identifier
Completed	1 Countries	NCT03920865 BP40995

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

Trial Summary:

This is a multi-center, open-label, non-randomized, parallel-group, 2-part study to evaluate the effect of hepatic impairment on the PK and safety and tolerability of a single oral dose of risdiplam compared to matched healthy participants with normal hepatic function.

Hoffmann-La Roche Sponsor	**Phase 1** Phase

NCT03920865 BP40995
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	>=18 Years & <= 70 Years	Accepts Healthy Volunteers

How does the BP40995 clinical trial work?
This clinical trial is recruiting people who have mild or moderate liver disease. It is also recruiting healthy volunteers with normal livers.

Risdiplam is currently being tested as a possible treatment for people with spinal muscular atrophy (or SMA). People with SMA can also be affected by liver disease, so it is important

for doctors to know if risdiplam works differently in these patients. By taking part in this trial, you will help us to find out how liver disease affects the way the body processes risdiplam, so that doctors know how best to use risdiplam in people who have SMA and liver disease.

How do I take part in this clinical trial? To be able to take part in this clinical trial, you must have been diagnosed with mild or moderate liver disease, or be in good health with a normal liver. Volunteers with a healthy liver will be matched to a person with liver disease based on sex, smoking status, age and BMI (body mass index).

You must not have previously been given risdiplam and you must not have diabetes. Women who are pregnant, breastfeeding or able to have children will not be able to join the trial.

If you think this clinical trial may be suitable for you and would like to take part, please talk to your doctor. If your doctor thinks that you might be able to take part in this clinical trial, he/she may refer you to the closest clinical trial doctor. They will give you all the information you need to make your decision about taking part in the clinical trial. You can also find the clinical trial locations on this page.

You will have some further tests to make sure you will be able to take the treatments given in this clinical trial. Some of these tests or procedures may be part of your regular medical care. They may be done even if you do not take part in the clinical trial. If you have had some of the tests recently, they may not need to be done again.

Before starting the clinical trial, you will be told about any risks and benefits of taking part in the trial. You will also be told what other treatments are available so that you may decide if you still want to take part.

While taking part in the clinical trial, men with female partners who are able become pregnant will need to either not have heterosexual intercourse or take 2 methods of contraception for safety reasons.

Everyone who joins this clinical trial will be given risdiplam, given as a liquid to drink once. You will have to stay in the Clinical Research Unit (or CRU) for 15 days in total, starting the day before you are given risdiplam, so that doctors can monitor how your body processes risdiplam.

How often will I be seen in follow-up appointments, and for how long?
You will be given the clinical trial treatment risdiplam once, the day after you arrive at the CRU. Once you have finished your 2 week stay at the CRU, you will still have to visit the CRU every 1–2 days for 2 weeks. These visits will include checks to see how your body is processing risdiplam and any side effects that you may be having. You are free to leave the clinical trial at any time.

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What happens if I am unable to take part in this clinical trial?
If this clinical trial is not suitable for you, you will not be able to take part. You will not lose access to any of your regular care.

For more information about this clinical trial see the **For Expert** tab on the specific ForPatient page or follow this link to ClinicalTrials.gov

Trial-identifier: NCT03920865