CLINICAL STUDY RESULTS

A study to learn about the safety of ritivixibat in adults with primary sclerosing cholangitis

Overall, the results suggest that ritivixibat was well-tolerated by participants with primary sclerosing cholangitis.
The results shown in this summary represent one clinical study.
Other clinical studies may produce different results.

This lay summary was created by Ipsen with the assistance of a third-party writing service provider

A3907-002
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What was the study about?

The purpose of this study was to learn how safe and well-tolerated ritivixibat is in people with primary sclerosing cholangitis (PSC) with or without bile tube stricture when given in repeated doses.

Bile tube strictures are narrow areas in one or more bile tubes. The bile tubes carry bile, a digestive acid, from the liver to the gut.

PSC is a rare and long-lasting liver disease where the bile tubes slowly become damaged and get narrower over time. As the bile tubes get narrower, the bile cannot flow properly and builds up in the liver, causing damage to the liver.

Currently, liver transplant is the only effective treatment available for PSC.

Ritivixibat, also known as A3907, is a new drug being developed. Researchers believe that it may help to lower the levels of bile in the body.

In healthy people, some of the bile that flows from the liver to the gut gets absorbed back into the blood and goes back to the liver. The rest of the bile is removed from the body through the urine. This keeps the level of bile in normal range. In people with PSC, this process is affected which increases the amount of bile in blood.

Ritivixibat is believed to interfere with this process of bile getting back to the liver and to increase removal of bile from the body through the urine. This is expected to lower the amount of bile, which may help decrease liver damage.

Ritivixibat is not yet approved to treat PSC.

Researchers wanted to know:
How many participants had medical problems* during the 12 weeks of treatment with ritivixibat, when given in repeated doses?

*A medical problem, also called an ‘adverse event’, is any change in participants’ health that they may experience, like if they felt unwell, experienced any kind of medical event, or noticed anything different about their bodies. These medical problems could be related or not related to the study drug.

The study took place between January 2023 and July 2025 at 6 study centres in France, Italy, Poland and Spain.

The study was stopped early by the sponsor due to difficulty in finding the required number of participants for the study.

Who took part in this study?

18
PARTICIPANTS

17
MEN

1
WOMAN

42 YEARS
AVERAGE AGE

To take part in the study, participants had to:

- Be aged 18 to 75 years,
- Be diagnosed with PSC that affects the large bile tubes, for more than 6 months. A smaller group of participants within the study had bile tube strictures that the researchers considered as clinically significant*, and
- Have liver blood tests that were not in the normal range.

* Clinically significant bile tube strictures means that the bile tube was narrowed by more than 75% and there were symptoms of liver diseases associated with it.

Participants could not take part in the study if:

- they had a health condition(s) or had received treatment(s) that could affect the result of the study.

What treatments were used in this study?

Study Treatment

Ritivixibat was given as a tablet of 10 milligrams (mg) by mouth. The dose of ritivixibat was based on the treatment group. The participants received either

- 10 mg once a day (1 tablet), or
- 30 mg once a day (3 tablets), or
- 30 mg twice a day (total of 6 tablets in one day).

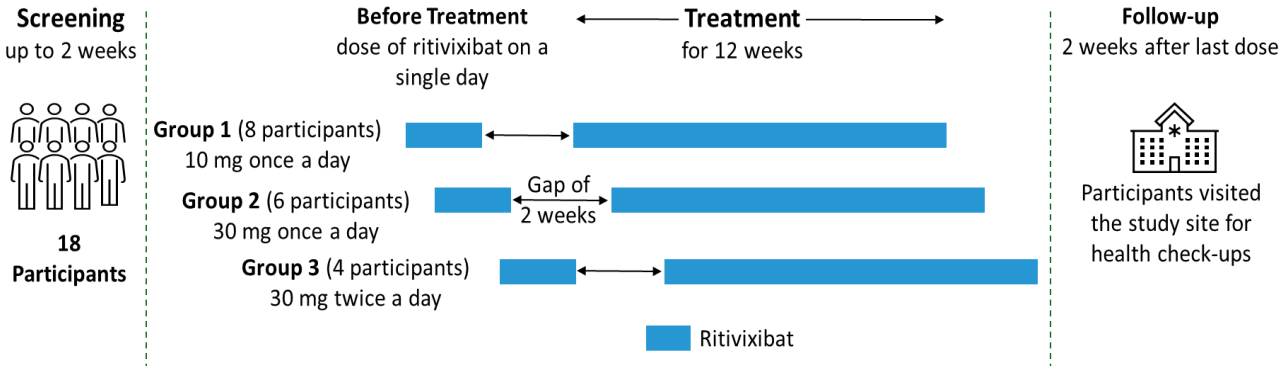
This study had 4 parts:
Screening: The study doctor checked if participants were suitable to take part in this study up to 2 weeks ahead of the 'before treatment' part.

There were 3 groups in this study. Group 1 received ritivixibat 10 mg once a day. Group 2 received ritivixibat 30 mg once a day. Group 3 received ritivixibat 30 mg twice a day. This group of participants had PSC with clinically significant bile tube strictures.

Before Treatment: All participants received ritivixibat for a single day and their blood samples were collected. This was to confirm the amount of ritivixibat in the blood was at an acceptable level to allow them to continue the study. If their participation was confirmed, they came back to the study site 2 weeks after this dose to start the treatment.

Treatment:
A total of 18 participants received ritivixibat , and the planned treatment period was 12 weeks.
Participants in Group 2 began after at least three participants in Group 1 completed 2 weeks of treatment with no safety concerns. Participants in Group 3 began after at least 3 participants in Group 2 completed 2 weeks of treatment with no safety concerns.
This study was “open label”. This means that the researchers and the participants knew which treatment was given to each participant.

Follow-up: Two weeks after the last dose, the participants visited the study site for health check-ups.



An individual participant could be in the study for up to 18 weeks (around 4 and half months).

What did researchers find out in the study?

Ritivixibat was observed to be safe and well tolerated by participants with PSC

How many participants had medical problems during the 12 weeks of treatment with ritivixibat, when given in repeated doses?

Out of the 18 participants, 16 participants received ritivixibat for the full 12 weeks of treatment.

Among these 16 participants, 81% (13 out of 16) participants experienced at least one medical problem (also called an 'adverse event'). Medical problems include any health changes reported during the study, whether or not they were related to ritivixibat.

Participants who experienced a medical problem

What were the other findings in the study?

How did the amount of bile in blood and urine change from the start of treatment until the end of 12 weeks of treatment?

The researchers did not find any notable change in the amount of bile in blood and urine from the start of treatment until the end of 12 weeks of treatment. However, due to low number of participants, the researchers could not make a meaningful conclusion about these results.

How did the treatment make participants feel?

During the study, participants were asked to report any 'adverse events', that is, if they felt unwell, experienced any kind of medical event, or noticed anything different about their bodies. Researchers recorded all adverse events reported by participants, whatever the cause. For example, if some participants caught COVID-19 this was reported as an adverse event, although it was not related to the study treatment.

If the study doctor thinks an adverse event may be related to the study treatment, it is called a 'side effect'. A side effect is considered 'serious' when it is life-threatening, causes lasting problems, or leads to hospitalisation.

This section mentions overall side effects that happened from the start of the study (before screening) until the follow-up.

- No deaths or serious side effects happened in this study

Overall, 39% (7 out of all 18 participants who received ritivixibat) experienced side effects (adverse events that the study doctor considered related to ritivixibat).

One participant stopped taking part in the study because of side effects.

The most common side effects reported by 2 or more participants are shown below, both as a percentage (%) followed by the actual number of participants in the group (e.g. 22% or 4 out of 18).

Side Effects	Ritivixibat (18 Participants)
Diarrhoea	22% (4 out of 18) 
Increase of a protein called alanine aminotransferase in the blood	11% (2 out of 18) 
Increase of a protein called aspartate aminotransferase in the blood	11% (2 out of 18) 
Passing gas	11% (2 out of 18) 
Stomach pain	11% (2 out of 18) 

More information

To learn more about this study, please visit:

- ClinicalTrials.gov and search for study NCT05642468 or
- Clinicaltrialsregister.eu/ctr-search/search and search for study 2022-500790-14-00.

For more information about current treatments available, please speak to your healthcare provider. If you have any questions about this study, please contact the sponsor, Ipsen at:

 clinical.trials@ipsen.com

Future research

There is no future studies planned on this topic.

Study identification and other information

FULL STUDY TITLE: An Open Label, Phase 2 Study to Evaluate the Effect of A3907 on Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics in Adults with Primary Sclerosing Cholangitis (PSC)

STUDY NUMBERS: Europe: 2022-500790-14-00|United States: NCT05642468

PROTOCOL: A3907-002

OTHER INFORMATION: Phase 2 studies test how safe and/or effective a study treatment was during the study.

We thank all the volunteers who took part in this study. Without their support, advances in treatments for medical conditions would not be possible.

We would also like to thank the people who took the time to review this document to make it easier for a general audience to read.