What is a clinical research study?

A clinical research study, or clinical trial, is a scientific study in which participants are assigned to one or more investigational drugs to answer questions about the treatment to see if it works, works better than other treatments, and has side effects.

What is an investigational drug?

An investigational drug is a substance that is being tested in clinical research studies. It has been reviewed by an Ethics Committee (EC) for testing in people and may or may not be approved by the government health agency for treatment for this indication.

What risks are involved if my child participates in the BOLD study?

There are possible risks involved with any clinical research study. The study doctor will review the risks with you, and your child will be closely monitored throughout the study.

What are the costs to take part in the BOLD study?

Study participants and their parents/caregivers will not be charged for the study drug, any study doctor visits, laboratory work, tests, or procedures that are needed for the study.

You may be eligible for reimbursement of your travel expenses to and from the study clinic. Your study doctor can provide additional details regarding travel reimbursement.

Thank you for your interest in the BOLD study for children with BA who have had a Kasai HPE.

BOLD
The 1st global phase 3 study in biliary atresia

For more information, please visit BOLDtrial.com, or contact:

Albireo

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Biliary atresia is a **BIG** diagnosis for someone so little.

Biliary atresia wasn't part of your hopes and dreams for your new baby. But a clinical study might be.

The BOLD study for children with biliary atresia (BA) who have had a Kasai hepatoportoenterostomy (HPE) is now enrolling.

Talk to your child's doctor about the BOLD study.

Protocol number:
A4250-011

Why is the BOLD study important?

Biliary atresia (BA) is a condition in infants in which the bile ducts in and around the liver are abnormally narrow, blocked, or absent. This causes bile to build up in the liver, leading to liver damage and loss of liver function. Infants with BA are typically treated through a Kasai hepatoportoenterostomy (HPE), a surgical procedure to restore bile flow. However, many infants go on to need a liver transplant even after having a Kasai HPE.

This study is testing an investigational drug called odevixibat to see if it can help delay the time until a liver transplant is needed in infants with BA who have had a Kasai HPE. In a previous clinical research study of odevixibat in children, most of the side effects reported were mild, lasted only a short time, and were assessed by the study doctor to be not related to odevixibat.

Pharmaceutical companies use clinical research studies like this one to learn more about investigational drugs before they are made available to the public. Thank you for considering enrolling your child in this study.

What is the purpose of the BOLD study?

The main purpose of this study is to learn how well odevixibat works in BA and how safe it is compared with placebo. A placebo is a substance with no medicinal effect that looks, smells, and tastes like odevixibat but does not contain any active drug. Researchers use a placebo to see if the investigational drug works better or is safer than taking nothing.

What does odevixibat do?

Odevixibat is in a class of drug that affects the ability of bile acids to travel to the colon and be removed from the body. Bile acids are produced in the liver and help in digestion of fats in the small intestine.

Who can participate in the BOLD study?

To be eligible for this study, your child must:

- Be diagnosed with BA
- Have been no more than 90 days old at the time of the Kasai HPE
- Have had a Kasai HPE within 3 weeks from when randomized into the study
- Weigh greater than or equal to 3.5kg at time of first dose

This is not a complete list of study requirements. The study doctor will review the full requirements for this study with you.

How long will the BOLD study last?

Your child's total participation time in this study is expected to be about 2 years. This includes a screening period that typically takes a few days but could last up to 3 weeks, a treatment period of 104 weeks, and a follow-up period of 4 weeks.

What will happen in the BOLD study?

If eligible to participate in the BOLD study, your child will be randomly assigned (like the flip of a coin) to receive either odevixibat or placebo (both referred to as "study drug"). There is a 50% chance that they will receive odevixibat and a 50% chance that they will receive placebo. Neither you nor the study doctor/staff will know which study drug your child is getting.

Your child will take the study drug every morning, together with food. The study drug will be provided in capsule form. The contents of the capsule can be mixed with formula, breast milk, or soft foods.

Over the course of the 104 week treatment period, your child will be scheduled for 10 visits to the study clinic and an additional follow-up telephone call or onsite visit. Laboratory tests, a physical exam, and other assessments and questionnaires will be conducted at the in-clinic visits.

After the study ends, your child may be able to continue receiving odevixibat (or begin receiving it, if they received placebo during the study) through an open-label extension study.

Why should my child and I take part in this study?

There are currently no medications available to treat BA. Clinical research studies like this one may help change that. Through your child's participation in this study, your family may help make a difference for future patients with BA.