

# Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

**Assent consent: 6-11 years old**

**Programme title:** The use of odevixibat for the treatment of cholestatic liver disease in patients with Alagille Syndrome, through a medical need programme (MNP).

**Responsible for the programme:**

Albireo AB, represented in Belgium by Tanner Pharma IE, 11 Bellevue Court, Frankfield, Douglas, Cork, T12 E06D, Ireland.

**Committee for Medical Ethics:** Cliniques Universitaires St.-Luc

Address: Promenade de l'Alma 51 bteB1 43.03 – 1200 Bruxelles

e-mail: [commission.ethique-saint-luc@uclouvain.be](mailto:commission.ethique-saint-luc@uclouvain.be)

TEL: 02/ 764 55 14

**Contact details of the mediator for the Saint-Luc clinics:**

[mediateur-saintluc@uclouvain.be](mailto:mediateur-saintluc@uclouvain.be)

**Treating physician:** *Please complete the following information before providing it to the patient*

Name: .....

Hospital address: .....

.....  
.....

Contact number: .....

Emergency number: .....

Contact number in case of questions related to this programme:

.....

**Contact details of the hospital's data protection officer:**

.....

**Contact details of the mediator of the centre:**

.....

# Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

## Assent consent: 6-11 years old

You can ask your parent(s)/guardian(s) to read this assent form to you. Please ask someone to explain anything that you do not understand.

### Invitation to join the program

Your doctor believes that this new drug called odevixibat (A4250) can help children like you that are feeling sick because of a problem with their liver.

### What is the purpose of this program?

This program is for people with ALGS who cannot get access to the drug through a clinical study. Odevixibat has already been tested in some children with ALGS. The doctors and nurses will explain the program to you. They will answer all your questions.

### What will happen to me if I take part in the program?

The program starts with a doctor's appointment and some tests, for example blood tests. The program doctor will review your medical records and results from previous tests you had. Once you and your parents agree for you to be in the program, you will have additional tests. You will have more blood draws for different tests and if you are a girl you will have a pregnancy test.

Some of the tests that you will have when you come to the doctor's office for program visits are described further below:

- Blood draws to check your health that require fasting: Some blood tests are only accurate if they are done after fasting. Fasting means going without food or drinks other than water for a certain number of hours. Before you come for the program visits you need to fast for at least 4 hours.
- Pregnancy test for girls: If you are a girl and are old enough to have a baby, then you should read this part. If you have started your periods, you will have blood tested to be sure you are not pregnant.

### What is the medicine like?

You will be given the new drug called odevixibat.

You should take the drug every day in the morning with your breakfast and drink some water soon afterwards. You will be asked to swallow the pills whole, but if you can't swallow the pills, the doctor may allow your parent(s)/guardian(s) to mix the drug in your food.

### What will I have to do?

Please try to do what the program doctor or nurse tells you to do.

Remember to tell your parent(s)/guardian(s) and the doctor how you are feeling, especially if something makes you feel bad, afraid, sick or uncomfortable.

### Do I have to take part?

It is your choice if you want to be in this program or not. You can say "no" even if your parent(s)/guardian(s) want you to take part. If you say "yes" now to being in the program, and you change your mind about it later, you can stop being in the program. No one will be angry if you say "no" or decide to stop being in the program. Just tell the doctor or your parent(s)/guardian(s) if you want to stop at any time. You do not have to be in this program to get help from the doctors and nurses for your itching or liver problem.

## **Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome**

### **Assent consent: 6-11 years old**

#### **Will taking part help me?**

You may or may not feel better while you are taking part in the program. Taking part in the program may provide information about the new drug, which may mean a better drug can be available in the future.

#### **Will the new drug hurt me?**

Program doctors, nurses and other people think that it is safe for children to take the new drug. It is not likely that something will go wrong because you are in the program but no one is sure. Many grown-ups and children have taken odevixibat. Odevixibat could cause runny poo or pain in your stomach. There is a risk that your itching could get worse if the new drug does not work.

#### **Will facts about me be kept private?**

Yes, your information will be kept private. Nobody except the doctors and nurses who look after you at the hospital/clinic and some people from the company that makes the new drug or other companies helping with the program will know anything about you.

#### **Who has checked if the program is safe?**

The medical need program was notified to the Federal Agency for Medicines and Health Products of Belgium and they have not objected to the implementation of the medical need program.

#### **Who will answer my questions about the program?**

The program doctor or nurses working on the program at the hospital/clinic will answer all of your questions. You can also ask your parent(s)/guardian(s) and they will talk to the doctors or nurses.

## Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

### Assent consent: 6-11 years old

#### II Informed consent

##### Participant

You will get a signed and dated copy of this document.

1	I have read about the program or somebody has told me about it.
2	I know that I do not have to take part if I do not want to.
3	I understand what this program is about.
4	I want to take part in this program.
5	All my questions were answered.

If you **do not** want to take part in this program, do not write your name below.

If you **do** want to take part, write your name and today's date here.

If you do not like writing, you could draw a smiley face and someone else can write your name and date for you.

Your name and surname: \_\_\_\_\_

Today's date: \_\_\_\_\_

Active assent should be obtained whenever appropriate and possible.

## Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

### Assent consent: 6-11 years old

Statement of person conducting assent discussion:

- I have explained all aspects of the program to the patient to the best of his or her ability to understand.
- I have answered all the questions of the patient relating to this program.
- The subject agrees to be in the program.
- I believe the subject's decision to enroll is voluntary.
- The program doctor and program staff agree to respect the subject's physical or emotional dissent at any time during this program when that dissent pertains to anything being done solely for the purpose of this program.

\_\_\_\_\_  
Signature of Physician/person who explained the program

\_\_\_\_\_  
Date (dd/Mmm/yyyy)

\_\_\_\_\_  
Printed name of Physician/person who explained the program

# Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

## Assent consent: 12-17 years old

**Programme title:** The use of odevixibat for the treatment of cholestatic liver disease in patients with Alagille Syndrome, through a medical need programme (MNP).

**Responsible for the programme:**

Albireo AB, represented in Belgium by Tanner Pharma IE, 11 Bellevue Court, Frankfield, Douglas, Cork, T12 E06D, Ireland.

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# Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

## Assent consent: 12-17 years old

### **Introduction**

The program doctors and nurses will explain the program and answer any questions that you have. You can talk to your parent(s)/guardian(s) and ask to read the information the program doctor gives them. If you are 16 years or older, we recommend that you read the parent's informed consent form along with your parent(s)/guardian(s). If you agree to be in this program, you will be asked to sign and date this form. Your parent(s)/guardian(s) will sign another informed consent form.

### **What is the purpose of this program?**

A medical need Program provides access to investigational drugs to treat patients with serious diseases or conditions for which there are no comparable or satisfactory therapy options available, including clinical trials. Odevixibat is an investigational medicine, it is not yet approved by the Health Authority that approves drugs in Belgium for the treatment of ALGS. Your doctor has recommended that you take part in this program for people with ALGS who cannot get access to the investigational drug odevixibat through a clinical study. Odevixibat has already been tested in some children with ALGS.

### **Do I have to take part?**

You do not have to be in the program if you don't want to. It is your own choice if you want to be in this program or not. No one will be angry if you choose not to take part. For you to be in this program, both you and your parent(s)/guardian(s) must agree to you being in this program, but it is still up to you to decide.

Your doctors or your parent(s)/guardian(s) cannot make you take part in the program if you don't want to be in it. If you say "yes" now and you change your mind later, you can stop being in the program at any time, just tell the doctor or your parent(s)/guardian(s). If you leave the program, you will be asked to come in for one last visit.

The program doctors and nurses will provide you medical care as they have in the past, regardless of whether you are in the program or not.

### **What will happen to me if I take part in the program?**

The program starts with a doctor's appointment and some tests, for example blood tests. The program doctor will review your medical records and results from previous tests you had. Once you and your parents agree for you to be in the program, you will have additional tests. You will have more blood draws for different tests and if you are a girl you will have a pregnancy test.

Some of the tests that you will have when you come to the doctor's office for program visits are described further below:

- Blood draws to check your health that require fasting: Some blood tests are only accurate if they are done after fasting. Fasting means going without food or drinks other than water for a certain number of hours. Before you come for the program visits you need to fast for at least 4 hours.

### **What treatment will I be given if I take part?**

Everyone who takes part in this program will be given the new drug, odevixibat to take. The program doctor will tell you how many pills (capsules) to take. Depending on how much you weigh, you will have to take between 2 and 6 capsules every day.

# Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

## Assent consent: 12-17 years old

The pills should be taken in the morning and swallowed whole. Do not chew them. If you can't swallow pills, the pills may be opened and sprinkled in a small amount of yogurt, apple sauce or fruit purée. This should be followed by water and then your breakfast. At program visits you will be asked what you think of taking the drug.

### What will I have to do while I am in the program?

- You should follow the instructions of the program doctor and nurses.
- You should tell the program doctor or your parent(s)/guardian(s) if you feel unwell at any time during the program.
- If you are a girl who is able to become pregnant and sexually active, or if you are a boy who is sexually active, you must use suitable methods of birth control while you are in the program.

### Things that could make you feel bad, afraid or uncomfortable

Remember to tell your parent(s)/guardian(s) and the doctor how you are feeling while you are in the program, especially if you feel sick or feel different than usual.

Odevixibat could cause diarrhea (runny stools) and stomach pain. Your condition could get worse if odevixibat doesn't work. Your condition may also stay the same.

Any of the things listed below may happen to you. If so, you should tell your parent(s)/guardian(s) and the doctor.

- You may feel tired.
- You may feel embarrassed by the questions the doctor or nurse asks you.
- You might have diarrhea or stomach pain.

### Possible risks from program procedures

- **Blood draws:** A blood draw may cause faintness, inflammation (swelling/redness) of the vein, pain, bruising, or bleeding at the site of puncture. There is also a slight risk of infection. If you don't like the blood drawing, a local anesthetic cream can be used at the injection site before doing the test. This will numb the skin and make the draw less painful.
- **Fasting:** Fasting could cause dizziness, headache, stomach discomfort, or fainting.

You might also feel other things. If you do, you must tell the doctor or your parent(s)/guardian(s) right away; for example, if you feel sick or if you take any new medicines. You or your parent(s)/guardian(s) can call the doctor at the telephone number on the first page of this form.

### Pregnancy

Because odevixibat is a new drug, doctors don't know if it might have a bad effect on an unborn baby. If you are a girl and have had your first period, the doctor or nurse will test your blood to make sure you are not pregnant. The doctor or nurse will tell you if the test results show you are pregnant. The program doctor or nurse may also tell your parent(s)/guardian(s) about the results of the pregnancy test. A pregnancy test does not stop you from becoming pregnant.

### Birth Control

If you are a girl and have started having your period, are physically able to have children, and are sexually active, or if you are a boy and are sexually active, you must use birth control consistently and correctly during the program. Some types of birth control will not work when



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you are taking certain medicines. The program doctor will discuss with you the best method of birth control for you.

If you are pregnant, planning to become pregnant during the program, you cannot be in the program.

If you think that you have become pregnant during the program, you must stop taking the medicine and tell the doctor immediately and you will have to leave the program. The program doctor may ask for information about the pregnancy and the birth of the baby.

If you are a boy and your female partner becomes pregnant while you are in this program, please inform the program doctor immediately. The program doctor will ask to speak to your partner. If she provides consent, the doctor will collect information about the pregnancy and the baby.

The doctor may share this information with the sponsor.

#### **Will taking part help me?**

You may or may not feel better while you are taking part in the program. Taking part in the program may provide information about the new drug, which may mean a better drug can be made in the future.

#### **What if something goes wrong?**

All patients taking part in this program are insured. The insurance covers any costs in case there is any harm as a result of the new medicine or tests. Your parent(s)/guardian(s) will receive more details regarding the insurance.

#### **Who is organizing and paying for this program?**

Albireo AB is the company developing odevixibat and organising this program. Albireo AB is providing odevixibat free of charge in this program.

#### **Will information about me be kept private?**

During the program the doctor will collect personal information (data) about you which they will try to keep private. Before the program doctor sends the information out of the hospital, it will be protected by a code so that no-one will be able to find out that the data is about you. Only the program staff at the hospital and some members of the sponsor company and other businesses helping with the program will see your data. It is also possible that some government authorities may need to see your data.

#### **Who has checked if the program is safe?**

The medical need program was notified to the Federal Agency for Medicines and Health Products of Belgium and they have not objected to the implementation of the medical need program.

#### **Contacts and Questions**

You can ask questions about the program at any time. You or your parent(s)/guardian(s) can call the program doctor if there is anything you are unsure about. The phone number is on the first page of this document.

Please ask your program doctor to explain anything that you do not understand. If you understand everything and you decide to take part in the program, please sign your name on the following Assent form.

**You will be given a copy of your signed and dated assent form to keep.**

## Paediatric Assent Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

### Assent consent: 12-17 years old

#### II Informed consent

##### Participant

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Statement of person conducting assent discussion:

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\_\_\_\_\_  
Signature of Physician/person who explained the program

\_\_\_\_\_  
Date (dd/Mmm/yyyy)

\_\_\_\_\_  
Printed name of Physician/person who explained the program

# Informed Consent Medical Need Programme Odevixibat for the Treatment of Cholestatic Liver Disease in Patients with Alagille Syndrome

## Informed consent form for the parent or guardian

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