

## INFORMATION AND CONSENT FORM

**Study Title:** A Randomized, Double-Blind, Placebo-Controlled Multicenter Study to Evaluate the Safety, Tolerability and Efficacy of ATI-501 Oral Suspension Compared to Placebo in Adult Subjects with Alopecia Areata, Alopecia Universalis or Alopecia Totalis

**Study #:** ATI-501-AUAT-201

**Sponsor:** Aclaris Therapeutics, Inc.

**Study Doctor:** <<investigator>>  
<<firm name>>  
<<street address>>, <<city>>, <<state>> <<zip>>

**Telephone Number:** <<000-000-0000>>

**After Office Hours:** <<000-000-0000>>

The study doctor wants to know if you would like to be part of a research study.

If you have any questions about or do not understand something in this form, you should ask the study doctor or study staff. You should also discuss your participation with anyone you choose in order to better understand this study and your options.

Participating in a research study is not the same as getting regular medical care. The purpose of regular medical care is to improve your health. The purpose of a research study is to gather information.

### WHAT IS THIS STUDY ABOUT?

Researchers want to find out more about an investigational drug called ATI-501 Oral Suspension. An "investigational" drug is a drug that is being tested as a potential treatment and is not approved for sale in the United States by the U.S. Food and Drug Administration (FDA).

Alopecia is the general medical term for hair loss. Alopecia areata (AA) starts as mild patchy hair loss but can progress. Severe AA occurs in two forms: alopecia totalis (AT) which is total hair loss on the scalp (head), or alopecia universalis (AU) which is hair loss of the scalp and the rest of the body including eyebrows and eyelashes.

Alopecia areata is an autoimmune disease. Your body's immune system protects you from disease and infection, but if you have an autoimmune condition or disease, your immune system attacks healthy cells in your body by mistake. In AA, the immune system attacks the cells in the hair follicles, which causes the hair to stop growing and fall out.

Currently, there is no proven treatment for AA. Increases in the understanding of what causes AA has led to the development of the new drug ATI-501, which may help to stop the body from attacking cells in the hair follicles.

The main purpose of this study is to see whether ATI-501 Oral Suspension can help regrow hair in people with stable patchy alopecia areata, alopecia universalis, or alopecia totalis. Researchers also want to find out if the three strengths of ATI-501 Oral Suspension are safe and tolerable.

This study will compare the three strengths of ATI-501 Oral Suspension (400 mg, 600 mg and 800 mg) with a placebo to see if using ATI-501 Oral Suspension is better than using a placebo. The placebo is a substance that looks and tastes like ATI-501 Oral Suspension but has no drug in it.

It is planned that about 120 people with stable patchy alopecia areata, alopecia universalis, or alopecia totalis will be in this study.

Be aware that this form refers to ATI-501 Oral Suspension and the placebo both as “study drug.”

### **HOW DOES ATI-501 ORAL SUSPENSION WORK?**

ATI-501 Oral Suspension is an investigational drug in an oral suspension that is designed to allow hair growth to occur again. ATI-501 Oral Suspension has not been studied in people with alopecia areata, and whether or not ATI-501 will work is unknown. Early signs of hair regrowth (if any occurs) may take up to three months.

### **IS THERE ANYTHING ELSE I CAN DO FOR MY PATCHY ALOPECIA AREATA?**

You do not have to be in this study to get help for your alopecia areata. Some other things you may be able to do are:

- Systemic corticosteroids (anti-inflammatory medications), either topically applied or injected into your scalp
- Immunosuppressive treatments (drugs that suppress your immune system) such as:
  - cyclosporine, methotrexate, or etanercept
- Phototherapy such as:
  - phototherapy with psoralen + UVA (PUVA)
  - narrow-band UVB
  - photodynamic therapy (PDT)
- Laser therapy
- Diphenylcyclopropenone (“DPCP”) or squaric acid dibutyl ester (“SADE”) – medications that are applied to your scalp to produce an allergic reaction (an approach known as topical immunotherapy)
- Topical anthralin (a tar-like topical medication)
- Topical minoxidil (a topical medication approved for male and female pattern baldness)

Although many products are used for this condition, their effectiveness is uncertain.

You should discuss your alternatives to participation in this research with the study doctor or study staff. In addition, you may discuss your options with your regular health care provider.

### **WHO IS PAYING FOR THIS STUDY?**

A company called Aclaris Therapeutics, Inc., the sponsor of the study, is paying for this study.

*<<Quorum may add site-specific conflict-of-interest language to the form based on information the site reports to Quorum.>>*

### **WILL IT COST ANYTHING TO BE IN THIS STUDY?**

It will not cost you anything to participate in this study.

### **HOW LONG WILL I BE IN THE STUDY?**

If you decide to be in this study and the study doctor says you can be in the study, your participation will include 10 visits to the study center and will last about 28 weeks, up to a maximum of 200 days, with a possibility of up to an additional 30 days for screening results to be received.

You will visit the study center to have the procedures and tests described in this form. Ask the study doctor or study staff about your study visit schedule.

### **WHAT WILL HAPPEN DURING THIS STUDY?**

If you decide to be in this study, you might have to stop using your regular medication or therapy during the entire study. The study doctor will discuss this with you in detail.

Each month, the study doctor or study staff will give you a one-month supply of study drug. You will also be given written instructions on how to take the study drug. Each day you will take one single-dose bottle of study drug followed by a full glass of water about 30 minutes after your morning meal. Then 8 to 12 hours later, you will take the second dose of study drug about 30 minutes after your evening meal followed by a full glass of water. You should not take the study drug on an empty stomach.

You will be assigned by chance (like flipping a coin) to 1 of the following study groups:

- Group 1: ATI-501 Oral Suspension, 400mg
- Group 2: ATI-501 Oral Suspension, 600mg
- Group 3: ATI-501 Oral Suspension, 800mg
- Group 4: Placebo Solution (a substance that looks and tastes like a drug, but has no drug in it)

You have an equal chance of being in any of the study groups.

Neither you nor the study doctor or study staff will be able to pick which study group you are in. You will not know, and the study doctor or study staff will not know which study group you are in. The study doctor or study staff can find out if it is necessary to know for your health. If this happens, the study doctor or study staff may not be able to tell you which study group you were in until everyone finishes the study.

While you are in the study, you must:

- Follow the instructions you are given.
- Come to the study center for all visits with the study doctor or study staff.
- Tell the study doctor or study staff about any changes in your health or the way you feel.
- Tell the study doctor or study staff if you want to stop being in the study at any time.
- Use birth control during the study and up to 30 days after the last dose of study drug.

### **What happens when I come for study visits?**

After you sign this form, the study doctor or study staff will perform the tests and procedures listed below when you come in for the study visits. If you would like more information about which tests and procedures will be done at each study visit, ask the study doctor or study staff.

- **Demographic Questions:** Ask you to give personal information, such as your name, date of birth, sex at birth, and race/ethnicity.
- **Health and Medication Questions:** Ask you to answer questions about your health, your medical history, your alopecia areata history, and the medications you take.
- **Physical Exam:** The study doctor or a member of the study staff will examine your overall general appearance, head, eyes, ears, nose, throat, stomach, nervous system, muscles and bones, lymphatics, and skin, and will listen to your heart and lungs.
- **Vital Signs:** Check your blood pressure by putting a band around your arm (this will squeeze your arm for about a minute), check your pulse, listen to you breathe in and out, and take your temperature.
- **Height, Weight:** See how tall you are, and see how much you weigh. This will only be done at your first visit.
- **Blood Testing:** Take some blood through a needle in your arm to do safety and laboratory tests. During the first visit, you will have 6 tubes of blood taken and at other visits, you will have 2 tubes of blood drawn. The maximum amount of blood you will have taken over 8 months is slightly less than ½ cup for the safety testing.
  - Some of your blood will be used to test for HIV, hepatitis, and tuberculosis.
    - The study doctor or study staff will tell you if these test results are positive.
    - If required by state law, the study doctor or study staff may report a positive test result to the local health department.
    - The results of these tests must be negative in order for you to be in the study.
- **Urine Testing:** You will have your urine tested.
- **Pregnancy Testing:** Test your blood and/or urine to see if you are pregnant. You will only have pregnancy testing if you are a woman and can have children.
  - The study doctor or study staff will tell you if the pregnancy test results are positive.
  - The results of the pregnancy testing must be negative in order for you to be enrolled in and continue in the study.
- **Electrocardiogram:** An electrocardiogram (ECG) measures the electrical activity of your heart.

- **Other Questions:** You will be asked about any changes in your health and the medications you are taking since the previous study visit.
- **Hair Loss Examinations:** The study doctor or study staff will examine your scalp to determine the amount of hair loss, the quality of your hair (by gently pulling at hairs at the border of areas of hair loss), and the appearance and severity of your hair loss. The study doctor will also assess the amount of hair loss you are experiencing on your face [eyebrows, eyelashes, nostrils, and beard (if you are male)] and the rest of your body.
- **Questionnaires:** You will be asked to complete a series of questionnaires at different visits to describe the appearance & severity of your hair loss and assess the impact of hair loss on your life. You will also be asked at certain visits to rate your satisfaction with the study drug results and any changes you may have observed in your hair growth and quality.
- **Target Patch Identification:** If you have patchy alopecia areata, you will determine your most bothersome patchy area on your scalp.
- **Photography:** The study doctor or study staff will take pictures of your head and scalp (your face will be included in these photographs) to document the hair loss. These photographs may be used for research purposes related to the study and for presentation to government health authorities, such as the U.S. Food and Drug Administration (FDA), at scientific meetings, for scientific publications, for general corporate purposes, and may be used for marketing purposes. Your identity will not be revealed in these photographs. Your identifying features, (eyes, mouth, tattoos) will be hidden in the photographs. **You do not have to let the study doctor or study staff take photographs if you don't want to; however, if you decline to have these pictures taken, you will not be allowed to participate in this study.**
- **Study Drug:** You will be given a supply of study drug and instructions for how to take the study drug.
- **Subject Dosing Calendar:** Each month, you will be given a written calendar worksheet to document that you have taken your morning and evening doses of study drug.

The following is a schedule of events and tests you will have at each visit:

Events and tests	Visit 1	Visit 2	Visit 3	Visit 4	Visit 5	Visit 6	Visit 7	Visit 8	Visit 9	Visit 10
Informed consent	✓									
Physical examination	✓								✓	
A discussion about your age, birth date, medical, surgical, and history of hair loss	✓									
Vital Signs	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓
Height and Weight	✓									
Blood for safety and urine test	✓	✓	✓	✓		✓			✓	✓
Blood test for pregnancy, if you are a woman and can have a baby	✓									
Urine pregnancy test, if you are a woman who can have a baby	✓	✓		✓	✓	✓	✓	✓	✓	✓
ECG	✓		✓						✓	
Scalp examination and measurement of hair loss	✓	✓	✓	✓	✓	✓	✓	✓	✓	✓
Identify your most bothersome patchy area, if applicable		✓								
Questionnaire completion		✓	✓			✓			✓	✓
Photographs of your head and scalp		✓					✓	✓	✓	
Study drug and instructions		✓	✓	✓	✓	✓	✓	✓		
Other Questions		✓	✓	✓	✓	✓	✓	✓	✓	✓

The results from the tests at Visit 1 will determine whether you are eligible to participate in the study. If you discontinue the study early for any reason, the tests for Visit 9 will be performed.

**WILL BEING IN THIS STUDY HELP ME?**

The study drug may help your alopecia areata, but there is no guarantee that being in this study will help you. Your alopecia areata might not get better or may even get worse while you are in this study. You may get placebo, which is a substance that looks and tastes like a drug but has no drug in it. Information from this study might help researchers to better understand alopecia areata or come up with new tests or medications to help others in the future.

**WHAT ARE THE RISKS TO ME IF I AM IN THIS STUDY?**

This is the first time that ATI-501 has been used on people with alopecia areata.

In animal testing, the following temporary side effects occurred on the skin where ATI-501 was applied:

- Redness
- Swelling
- Skin peeling

Healthy volunteers took an oral form of ATI-501, and the most frequently reported drug-related side effects include:

- Abdominal pain
- Flatulence
- Diarrhea
- Headache

People who took FDA-approved oral medications similar to ATI-501 experienced:

- Infections (bacterial, viral, fungal, or other infections)
- Abnormal blood tests (decreases in white blood cells and platelets, and increases in liver function tests)
- Cancers
- Increased lipids (total cholesterol, high-density lipoprotein cholesterol [also known as HDL, the good cholesterol], and low-density lipoprotein cholesterol [also known as LDL, the bad cholesterol])

Since ATI-501 Oral Suspension is an investigational drug, there may be other risks that are unknown. Please tell the study doctor or study staff right away if you have any side effects. Please tell them if you have any other problems with your health or the way you feel during the study, whether or not you think these problems are related to the study drug.

It is possible that using ATI-501 Oral Suspension may change how your regular medications or supplements work. It is very important that you tell the study doctor about any medications or supplements before you take them during the study. You must also avoid receiving any vaccinations during the study and for 6 weeks after you take the last dose of study drug. Additionally, you should avoid routine contact with children and others who have received recent vaccinations during the time you are taking study drug and for 6 weeks following completion of the study.

#### Could I have an allergic reaction?

Sometimes people have allergic reactions to drugs. All drugs have a potential risk of an allergic reaction, which if not treated promptly, could become life-threatening. Some things that happen during an allergic reaction that could be a sign or symptom of a life-threatening allergic reaction (anaphylaxis) are:

- A rash
- A fast pulse
- Sweating
- A feeling of dread
- Swelling around the eyes and mouth

- Swelling of the throat
- Wheezing
- Having a hard time breathing
- A sudden drop in blood pressure (making you feel dizzy or lightheaded)
- Inability to breathe without assistance

You should get medical help and contact the study doctor or study staff, if you have any of these or any other side effects during the study.

What if I am using placebo instead of active drug during the study?

Some people in the study will get placebo instead of ATI-501 Oral Suspension. Placebo solution is a substance that looks and tastes like a drug but has no drug in it. If you use placebo during the study, it is possible that your alopecia areata may get worse. Please ask the study doctor or study staff if you have any questions about placebo.

If I stop using my regular medication or therapy, what are the risks?

If you stop your regular medication or therapy to be in the study, your alopecia areata symptoms might come back or get worse or your health might get worse. Please tell the study doctor or study staff right away if you have any problems when you stop or change your regular medication or therapy.

What are the risks of giving blood for this study?

The study doctor or study staff will take your blood by sticking a needle in your arm. Some problems you might have from this are:

- Pain
- Bruising
- Bleeding or oozing
- Dizziness
- Infection

Risks of study photography

There are no expected physical risks if the study doctor or study staff takes photos of your head and scalp during the study. It is possible that people who see the study photos will recognize you.

Loss of confidentiality

There is a risk of loss of confidentiality of your information. You will read more about the protection of your information later in this form. Please ask the study doctor or study staff if you would like to know more about how your information will be protected while you are in this study.

What are the risks if I am pregnant or nursing a child during the study?

If you are a woman who can have a baby, you must not get pregnant during the study or for 30 days after the last study drug dose. In animal testing, there were potential toxic effects to both the embryo and fetus, indicating a risk to pregnancies. If you are pregnant or nursing a child while using ATI-501 Oral Suspension, there may be risks to you, the embryo, fetus, or nursing child. Nobody knows what all the risks are right now.

If you are a woman who can have children, the study doctor or study staff will talk to you about birth control, which you must use during the study and up to 30 days after the last dose of study drug.

If you think you are pregnant during the study, you must tell the study doctor or study staff immediately.

Women who become pregnant during the study will have to leave the study. The study doctor or study staff may ask for information about the pregnancy and the child's health at birth, and for up to 6 weeks after the birth, and may share this information with the sponsor.

What are the risks of fathering a child during the study?

If you are a sexually active male, you must not father a child during the study or for 30 days after the last dose of study drug. In animal testing, there were potential toxic effects to both the embryo and fetus, indicating a risk to pregnancies. There may be risks to an unborn embryo or fetus that you father during or after the study. Nobody knows what all the risks are right now. You must agree to use a barrier method of contraception from the first dose of study drug to at least 30 days after the last dose of study drug.

If you think your partner is pregnant during the study, you must tell the study doctor or study staff immediately. The study doctor or study staff may ask for information about the pregnancy and the child's health at birth, and for up to 6 weeks after the birth, and may share this information with the Sponsor.

**COULD I HAVE ANY OTHER PROBLEMS WITH MY HEALTH IF I AM IN THIS STUDY?**

It is possible that you could have problems and side effects of ATI-501 Oral Suspension that nobody knows about yet, which include your alopecia areata getting worse.

Ask the study doctor if you have questions about the signs or symptoms of any side effects that you read about in this consent form.

**WILL I RECEIVE ANY NEW INFORMATION DURING THE STUDY?**

If the study doctor or study staff learns any new information that might change your mind about continuing in the study, the study doctor or study staff will tell you about it.

## **WHAT IF I GET HURT OR SICK WHILE I AM IN THIS STUDY?**

If, during the course of this study, any injury occurs to you as a direct result of the administration of the study drug or properly performed procedures, the Sponsor agrees to pay all medical expenses necessary to treat such injury: 1) to the extent you are not otherwise reimbursed by medical insurance; and 2) provided you have followed the directions of the study doctor.

The compensation offered by the Sponsor for any injury which occurs to you as a direct result of the administration of the study drug or poorly performed procedures by the study doctor or study staff, will be paid after the Sponsor receives all appropriate documentation and completes its review, to its satisfaction, of all documentation regarding your claim for injury.

You will not lose or give up any of your legal rights or release the Sponsor, the study doctor, the study staff, or study site from liability for negligence or intentional misconduct by signing this consent document.

There are no plans to provide financial compensation for such things as lost wages, disability, or discomfort due to injury.

## **WILL I RECEIVE PAYMENT?**

*<<Quorum will add site-specific compensation language to the form based on information the site reports to Quorum.>>*

## **DO I HAVE TO BE IN THIS STUDY?**

Your participation in this study is voluntary. You can decide not to be in the study and you can change your mind about being in the study at any time. There will be no penalty to you, and you won't lose any benefits except for benefits having to do with the study. If you want to stop being in the study, tell the study doctor or study staff.

The study doctor or study staff or Sponsor can remove you from the study at any time, even if you want to stay in the study. This could happen if, for example:

- The study doctor or study staff believes it is best for you to stop being in the study.
- You do not follow directions about the study.
- The Sponsor stops the study for any reason.

If you stop being in the study early, the study doctor or study staff may ask you some questions about being in the study. The study doctor or study staff may ask you to participate in some procedures or tests to help you leave the study safely and/or to collect more information for the study.

If you leave the study, the study doctor and study staff will still be able to use your information that they have already collected.

If you change your mind later, be aware that your samples may or may not be withdrawn from the research, depending on the sponsor's policies. You can ask the study doctor or study staff about this.

## **HOW WILL MY INFORMATION BE KEPT CONFIDENTIAL?**

Your identity will be protected as required by law and according to any policies the study center or sponsor may have. Be aware that your study records (which include your medical records, your signed consent form, and other information) will be shared as needed for the study. For example, the U.S. Food and Drug Administration (FDA), the sponsor, and Quorum Review may look at your study and medical records.

Your blood and urine samples will not be labeled with your name or other directly identifying information. Your samples will have a code (a unique subject identifier) instead. The list that matches the code with your name will be stored separately from your samples. Your samples will be kept only until we are able to complete the tests described in this form, and then your samples will be destroyed.

A description of this clinical trial will be available on <http://www.ClinicalTrials.gov>, as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

## **WHO CAN I TALK TO ABOUT THIS STUDY?**

In the event of an emergency, dial 911 immediately.

If you require emergency care, be sure to tell the emergency care provider about your participation in this study. Contact the study doctor or study staff as soon as possible.

You can ask questions about the study at any time. You can call the study doctor or study staff at any time if you have any concerns or complaints. You should call the study doctor or study staff at the phone number listed on page 1 of this form if you have questions about the study procedures, study costs (if any), study payment (if any), or if you get hurt or sick during the study.

Quorum Review reviewed this study. Quorum Review is a group of people who review research studies to protect the rights and welfare of research participants. Review by Quorum Review does not mean that the study is without risks. If you have questions about your rights as a research participant, if you are not able to resolve your concerns with the study doctor or study staff, if you have a complaint, or if you have general questions about what it means to be in a research study, you can call Quorum Review or visit the Quorum Review website at [www.QuorumReview.com](http://www.QuorumReview.com).

Quorum Review is located in Seattle, Washington.

Office hours are 8:00 AM to 5:00 PM Pacific Time, Monday through Friday.

Ask to speak with a Research Participant Liaison at 888-776-9115 (toll free).

## **HOW WILL MY INFORMATION BE USED AND SHARED FOR THIS STUDY?**

This section explains who will use and share your health information if you agree to be in this study. You must authorize this use and sharing of your information by signing this form or you cannot be in the study.

Initials \_\_\_\_\_ Date \_\_\_\_\_

Version 1, dated 05/01/18

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The study doctor and study staff will collect, use, and share health information about you, including any information needed to do the study and other identifying information about you, such as your name, address, phone number, or social security number. The information used and shared will include:

- information from your medical records
- information collected about you during the research, including study visits, notes, tests, procedures, photographs, etc.

Your information may be used and shared with these people for the following purposes:

- The study doctor and study staff to conduct this research.
- The sponsor, Aclaris Therapeutics, Inc.; people who work with or for the sponsor; and other researchers involved in this study. These people will use your information to review the study, to check the safety and results of the study drug, and to seek government approval of ATI-501 Oral Suspension.
- Others required by law to review the quality and safety of research, including the U.S. Food and Drug Administration (FDA), Department of Health and Human Services, Office for Human Research Protections, other government agencies in the United States and other countries, and Quorum Review.
- A public health authority that is authorized by law to collect or receive such information for the purpose of preventing or controlling disease, injury, or disability, and conducting public health surveillance, investigations, or interventions.

After your information is shared with the people and companies listed above, the law may not require them to protect the privacy of your information.

To maintain the integrity of this research, you might not have access to any health information developed as part of this study until it is completed. At that point, you generally would have access to your health information.

You can cancel your authorization to use and share your information at any time by writing a letter to the study doctor. If you cancel your authorization, you will not be able to continue in the study.

If you cancel your authorization, the study doctor and study staff will still be able to use and share your information that they have already collected.

This authorization to use and share your information expires in 50 years.

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Signature of Participant

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Date

<<Quorum staff: Include the following for Indiana sites:

In **Indiana**, you must complete the following information:

Participant's Street Address

Participant's City, State, ZIP>>

### REGULAR DOCTOR OR SPECIALIST NOTIFICATION OPTION

For your safety, you or the study doctor should tell your regular health care provider that you are in this study. This is recommended so that your primary care doctor may contact the study doctor if they have any concerns or questions about your care.

Please indicate below whether you want us to notify your regular doctor or your specialist of your participation in this study.

Yes, I want the study doctor to inform my regular doctor/specialist of my participation in this study:

\_\_\_\_\_  
Name of Doctor

\_\_\_\_\_  
Phone

- No, I do not want the study doctor to inform my regular doctor/specialist of my participation in this study.
- I do not have a regular doctor/specialist.
- The study doctor is my regular doctor/specialist.

**CONSENT**

I have read this form, and I have been able to ask questions about this study. The study doctor or study staff has talked with me about this study. They have answered all my questions. I voluntarily agree to be in this study.

By signing this form, I do not give up any of my legal rights. I will get a signed copy of this consent form.

\_\_\_\_\_  
Printed Name of Participant

\_\_\_\_\_  
Signature of Participant

\_\_\_\_\_  
Date

I attest that the individual providing consent had enough time to consider this information, had an opportunity to ask questions, and voluntarily agreed to participation in this study.

\_\_\_\_\_  
Printed Name of Person Explaining Consent

\_\_\_\_\_  
Signature of Person Explaining Consent

\_\_\_\_\_  
Date

I attest that I or my representative discussed this study with the individual providing consent.

\_\_\_\_\_  
Signature of Principal Investigator or Sub-Investigator