

Informed Consent Form (ICF) Cover Page

Protocol Title: A Phase 1/2 Dose Escalation Study of Subretinally Injected ATSN-101
Administered in Patients with Leber Congenital Amaurosis Caused by Biallelic Mutations in
GUCY2D

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UNIVERSITY OF PENNSYLVANIA

**COMBINED ADULT INFORMED CONSENT AND
HIPAA AUTHORIZATION FORM**

ATSN-101-ATSN-101-1A Phase 1/2 Dose Escalation Study of Subretinally Injected
ATSN-101 Administered in Patients with Leber Congenital Amaurosis
Caused by Biallelic Mutations in *GUCY2D*

Principal Investigator:

[REDACTED]
University of Pennsylvania

Phone during office hours:

[REDACTED]

Phone after office hours:

[REDACTED]

STUDY SUMMARY

You are being invited to participate in a research study. Your participation is voluntary and you should only participate if you completely understand what the study requires and what the risks of participation are. You should ask the study team any questions you have related to participating before agreeing to join the study. If you have any questions about your rights as a human research participant at any time before, during or after participation, please contact the Institutional Review Board (IRB) at [REDACTED] for assistance.

The research study is being conducted to evaluate the safety of a gene therapy treatment, called ATSN-101, for Leber congenital amaurosis caused by mutations in the *GUCY2D* gene (*GUCY2D*-LCA). You are being asked to participate in this research study because you have *GUCY2D*-LCA. This study used to be called DFI14738 and the study drug used to be called SAR439483.

If you agree to join the study, you will be asked to complete the following research procedures:

- A surgical procedure to inject the study drug beneath your retina in one eye.
- Vision tests: Eye exam, visual acuity test, pupillometry, full-field stimulus test, mobility test, oculomotor instability test, photoaversion (light discomfort) test
- Tests to look at images of your eyes: Optical coherence tomography, fundus photography
- Questionnaires: Visual function questionnaire, Patient's Experience Due to Changes in Vision Questionnaire

Your participation will last for approximately 5 years (52 weeks of study observation period and an additional 208 weeks of safety follow-up).

There is no guarantee of benefit from participating in this study. The most common risks of participation are:

- Inflammation of the eye in response to the study drug
- Risks related to the surgical procedure to inject the study drug, such as damage to the retina, cataract development, infection, bleeding within the eye, corneal abrasion, and increased pressure in the eye, redness, pain, or swelling
- Worsening vision as a result of the surgical procedure
- Reactions to anesthesia used during the procedure, which may include nausea, disorientation, low blood pressure, prolonged unconsciousness or drowsiness, pain, allergic reaction, vein irritation, irregular or fast heartbeat, pneumonia, problems with breathing, stroke or death.

Your participation in this study is your choice. Deciding not to participate will not affect your regular medical care. Please note that this is just a summary and there are other factors to consider before agreeing to participate such as additional procedures, use of your personal information, costs, and other possible risks not discussed here. If you are interested in

participating, a member of the study team will review the full information with you. You are free to decline or stop participation at any time during or after the initial consenting process.

You have been invited to participate in study ATSN-101-1, being done to test an experimental gene therapy (ATSN-101) for the treatment of Leber Congenital Amaurosis due to mutations in *GUCY2D* (*GUCY2D*-LCA). The information in this document is designed to help you decide whether or not you will take part in this study. This document contains two parts:

1. Information about the study
2. The informed consent form, where you sign and agree that you will take part (if this is what you decide to do).

The study is being conducted by Atsena Therapeutics, Inc. (the “Sponsor” or “Atsena Therapeutics, Inc.”). The study doctor's institution is being paid by the Sponsor for the services it provides in conducting this clinical study.

Your participation in this study is your choice. You are completely free to accept or refuse; take all the time you need to make a decision. Any questions that you have about the study will be answered by the study team before you decide. If you decide to take part, you can also leave the study at any time without giving a reason if you do not wish to provide one. It will not affect your regular medical care. There are no alternative treatments available for *GUCY2D*-LCA.

Before your participation in the study can be confirmed, you will have to complete initial tests for the study doctor (or the doctor conducting the study) to make sure that this is an appropriate study for you.

Please discuss any questions you may have with the study doctor. The rules and procedures for this study were reviewed and approved by the appropriate committees (IRB) responsible for ensuring that the rights of people in the study are respected.

OVERVIEW OF THE STUDY

Participation in this study means that you must follow some rules. Your safety and the quality of the data that will be collected depend on this. If you do not think you can follow these rules, it is better that you do not participate:

- Provide truthful information about your medical conditions and drug(s) that you have been taking or are taking
- Inform the study doctor or study staff before stopping or modifying your treatments, or before starting any new treatment
- Attend the scheduled visits and allow the tests or procedures to be done as planned
- Follow the study doctor's instructions regarding administration of medications that must be taken as part of this study
- Inform the study doctor or study staff as soon as possible of any side effects or health problems even if you do not think they are caused by the gene therapy treatment (ATSN-101), related procedures, or other medications administered as part of this study
- Agree, if you are a woman of childbearing potential (WOCBP) or a male with a partner WOCBP, to use effective birth control during the study as described in this document

Objectives

The purpose of the study is to evaluate a gene therapy (ATSN-101) that has been developed for the treatment of *GUCY2D-LCA*. In *GUCY2D-LCA*, both copies of the gene *GUCY2D* are not working due to the presence of mutations. Mutations in a gene are errors in the DNA sequence encoding that gene. The absence of a working copy of *GUCY2D* in your retina is what has caused your loss of vision. The gene therapy being tested in this study (ATSN-101) delivers a working copy of *GUCY2D* directly to the retina (the tissue in the back of the eye). The working copy of *GUCY2D* is carried by a virus called adeno-associated virus type 5 (AAV5). AAV5 does not cause disease and has been developed for the purposes of gene delivery. Administration of the gene therapy will be performed by a surgeon in the operating room. The virus carrying *GUCY2D* (AAV5-*GUCY2D*, also called ATSN-101) will be injected beneath the retina. This is referred to as a subretinal injection. In order to gain access to the retina and perform this injection, the surgeon will also perform a procedure called a vitrectomy, which involves removal of some of the gel that fills the eye.

This study is divided into two parts, Part A and Part B. During Part A, increasing doses of ATSN-101 will be administered to adults, and participants will be evaluated for side effects of the treatment and any impact on their visual function. Each participant will be assigned to receive one dose (either low, medium or high) in one eye, and the dose will be given as a one-time injection. There is no intention of retreatment of that eye at this time. The goal of Part A will be to evaluate the safety of ATSN-101 and to determine the best dose to use in future studies. During Part B, an additional group of adults as well as a group of children will be administered the dose that was determined in Part A. The goal of Part B is to gather additional

information regarding potential side effects of ATSN-101 and the impact of ATSN-101 on vision in both adults and children.

Study Team

There will be a large group of doctors and scientists who will care for you during the study. The team will be led by [REDACTED] who will have overall responsibility. Most of the study will take place at The University of Pennsylvania Scheie Eye Institute; however, the administration of the gene therapy will take place at Wills Eye Hospital. [REDACTED], who is an Attending Surgeon and Director of Retina Research at Wills Eye Hospital will lead the Wills Eye Hospital study group and will perform the subretinal injection to deliver the gene therapy treatment. Additionally, the mobility course assessment may take place either at the Center for Hereditary Retinal Degenerations at the University of Pennsylvania Scheie Eye Institute or at the Children's Hospital of Philadelphia (CHOP), which is affiliated with the University of Pennsylvania.

How long will I be in the study?

The study will last approximately 268 weeks and is planned to include approximately 15 participants at the University of Pennsylvania. The study will be divided into four periods:

1. Screening Period: This period will last approximately 53 days, and may involve multiple visits to the clinical site for testing.
2. Baseline Period: This period will last 3 days, and may involve multiple visits to the clinical site for testing.
3. Study Observation Period: This period will begin when ATSN-101 is administered and will last approximately 52 weeks. This period will involve 18 visits to the clinical site for testing. You may be asked to come in for additional visits during this time period for additional evaluation should the study doctor consider them necessary.
4. Safety Follow-up Period: This period will last approximately 208 weeks, and will involve 5 visits to the clinical site for testing. You may be asked to come in for additional visits during this time period for additional evaluation should the study doctor consider them necessary.

Additional visits other than those described above might be required should the study doctor consider them necessary for your safety.

Study Treatments

During this study, you will receive a subretinal injection of the gene therapy under investigation, ATSN-101. In addition, you will take four additional medications that are given to help reduce the risk of side effects of the gene therapy and the surgery required for subretinal injection of the gene therapy. These medications include three different forms of a type of medicine called a corticosteroid, which is a medication that is given to reduce potential inflammation within the eye. Inflammation can potentially be caused by surgery or ATSN-101. The preclinical animal studies showed that a regimen of topical, oral, and injected corticosteroids helped reduce the risk

of eye inflammation. The doses and schedule of these steroids were chosen based on the safety data from these preclinical animal studies. During surgery, a corticosteroid medication called triamcinolone will be injected into a space around the eye. For a short time before and after the surgery, you will be asked to take another corticosteroid called prednisone, which is taken as a pill. Finally, you will be asked to use a solution of a corticosteroid called prednisolone as an eye drop for a short time after the surgery. In addition to these three corticosteroid medications, you will also be asked to use an eye drop solution containing trimethoprim and polymyxin, which are two antibiotics. The antibiotic eye drops are used to help prevent infection following surgery and will be used for a short time following surgery. Local and possibly general anesthesia may be used during the surgery. During this study, additional medications could be added as needed by the study doctor.

Schedule of Visits and Activities

See following page. The study doctor may decide to perform some of the assessments below on additional days other than those listed if necessary during the course of the study. In addition, some tests listed below may not be performed if they are determined to be unnecessary by the study doctor during the course of the study.

Phase	Screening	Baseline	Main Study Period								Extension Study Period			
Day/Week	8 weeks to 4 days before procedure	1 to 3 days before procedure	Day of Procedure	Day 2 through Day 10-, Day 12, Day 14	4 weeks ± 5 days	8 weeks ± 7 days	12 weeks ± 7 days	24 weeks ± 2 weeks	36 weeks ± 3 weeks	52 weeks ± 3 weeks	78 weeks ± 4 weeks	104 weeks ± 4 weeks	<u>W156</u> <u>W208</u> <u>+4Wks</u>	<u>End-of-Treatment</u> <u>W260</u> <u>+4Wks</u>
Visit clinical or surgical site	X	X	X	11 visits	X	X	X	X	X	X	X	X	X	X
Review of selection criteria	X	X												
Informed consent/assent	X													
Physical exam	X													
Fasting		Night before	Prior to procedure											
Subretinal injection of gene therapy (ATSN-101)			X											
Corticosteroid and/or antibiotic medications		1 day before	X	Through Day 9										
Electrocardiogram	X													
Vital signs	X	X	X	X	X	X	X	X	X	X				
Urine sample	X	X		Once on Day 7	X					X				
Blood testing	X	X		Once on Day 7	X		X	X		X				
Visual acuity test	X	X		X	X	X	X	X	X	X	X	X	X	X
Low luminance VA		X			X	X	X	X	X	X				

Eye exam	X	X		X	X	X	X	X	X	X	X	X	X	X
Fundus photography	X	X		Day 2, Day 3	X	X	X	X	X	X	X	X	X	X
Fundus autofluorescence imaging		X			X						X	X	X	X
Intraocular pressure	X	X		X	X	X	X	X	X	X	X	X	X	X
Full-field stimulus test	X	X		2 to 6 times	X	X	X	X	X	X	X	X	X	X
Photoaversion test		X			X	X	X	X	X	X				
Optical coherence tomography	X				X	X	X	X	X	X	X	X	X	X
Pupillometry	X	X			X	X	X	X	X	X				
Oculomotor instability	X	X			X	X	X	X	X	X				
Visual function questionnaire		X			X		X	X		X	X	X	X	X
Patient's Experience Due to Changes in Vision Questionnaire					X		X	X		X	X	X	X	X
Mobility testing		X			X		X	X		X	X	X	X	X

Required Tests/Procedures

In addition to access and use of your medical records, the study staff will need to perform some required tests and procedures during the study.

- **Vitrectomy and subretinal injection:** The surgeon will perform a vitrectomy, removing some of the gel that fills the eye, in order to gain access to the retina. The Surgeon will then perform an injection of ATSN-101 solution beneath the retina. Imaging techniques, including video recording and photographs of the surgery and optical coherence tomography, may be used to help document the procedure. You will need to fast overnight or approximately 12 hours prior to the procedure as instructed by the study doctor.
- **Physical examination:** Measurement of height, weight and examination of major body organs.
- **Vital signs:** Measurement of blood pressure, heart rate, and body temperature.
- **Electrocardiogram:** Recording of the electrical activity of your heart over a period of time using electrodes placed on your skin.
- **Blood tests:** Blood will be drawn to monitor safety, test for HIV, test for pregnancy or menopause (in female participants), perform genetic testing, and monitor for immune reactions to the gene therapy. The total amount of blood drawn during the course of this five year study is approximately 30 tablespoons for participants.
- **Urine testing:** To evaluate for bleeding and infection. For female participants who are of childbearing potential, urine samples will be collected for pregnancy testing.
- **Genetic testing:** If determined necessary by the Investigator, genetic testing will be performed to confirm the presence of mutations in both copies of the gene *GUCY2D* and other genetic causes of inherited retinal disorders.
- **Visual acuity test (including the low luminance test where a neutral density filter is placed in front of the eye being tested):** You will be asked to read letters on a chart, if possible, or visualize other objects to determine your level of vision.
- **Eye exam:** The investigator will evaluate the retina and other parts of your eye. Eye drops will be administered to dilate the eye.
- **Fundus photography and fundus autofluorescence imaging:** Photographs will be taken of the back of the eye.
- **Intraocular pressure measurement:** An instrument will be used to measure the pressure within the eye.
- **Full-field stimulus testing:** You will be placed in front of an instrument and flashes of short duration will be presented one at a time. You will press a button if a flash is seen.
- **Photoaversion test:** This test will measure how sensitive your eyes are to different levels of light.

- Optical coherence tomography: This test uses a very thin laser beam to evaluate the thickness of different layers of your retina.
- Pupillometry: In this test, a light is presented to your eye while a video recording of the eye is being made to monitor constriction of a structure within the eye called the pupil.
- Oculomotor instability: In this test, video recordings are made to assess stability of your eye gaze.
- Visual function questionnaire: You will be asked a series of questions about how your vision affects your life. The visual function questionnaire may be administered either in person, or by phone up to three days (plus or minus two days) prior to your scheduled study visit.
- Patient's Experience Due to Changes in Vision Questionnaire: You will be asked about changes in vision. The Patient's Experience Due to Changes in Vision Questionnaire may be administered either in person, or by phone up to three days (plus or minus two days) prior to your scheduled study visit.
- Mobility testing: In this test, you will be asked to perform a series of mobility courses under different light conditions. Video recording of the test will be obtained in order to analyze the results. The mobility test may occur at The University of Pennsylvania Scheie Eye Institute or at the Children's Hospital of Philadelphia.

Unless you agree to the retention of your samples, they will be destroyed at the end of the study or after the time requested by local law.

BENEFITS AND RISKS

Foreseeable risks and discomforts from study drugs

There are discomforts or risks of side effects that might occur with the use of the study drugs or procedures. Some of them are still unknown at this stage. The study doctor is trained to take the appropriate measures to reduce risks and limit any discomforts you may experience.

Discomforts and risks associated with taking study drugs observed in previous studies with people/animals are listed below.

ATSN-101 (The Gene Therapy Drug): Because this product has never been tested in humans before, all possible risks that may be associated with this gene therapy are unknown. In other clinical studies of products similar to ATSN-101, a common risk associated with the gene therapy product itself is inflammation of the eye. This inflammation can result in damage to the retina and other parts of the eye and worsening of vision. Loss of vision due to inflammation of the eye caused by subretinal injection of ATSN-101 was observed in animal model studies of this therapy. Administration of ATSN-101 carries the potential risk of damage to your existing vision due to inflammation of the eye caused by subretinal injection of ATSN-101.

Due to the risk of ocular inflammation, corticosteroids are given as a pill, eye drop, and as an injection into a space around the eye. Corticosteroids reduced the risk of inflammation of the eye in animal model studies of this therapy and are given as part of this study to reduce the risk of inflammation of the eye. Toxicology studies for this therapy did not identify toxicities outside of the injected eye. Other important risks are associated with the surgical procedure used to administer the gene therapy (vitrectomy and subretinal injection), and those are described below.

Corticosteroids (prednisone, prednisolone, and triamcinolone): The side effects associated with corticosteroids can include weight gain, elevated pressure within the eye, cataract, high blood pressure, stomach irritation, stomach ulcers, loss of bone mass, death of bone tissue, loss of muscle mass, mood changes, high blood sugar, reduced function of the adrenal gland, increased white blood cell count, and increased risk of infection. In children, corticosteroids can cause impaired growth with long-term use. In general, the risks associated with corticosteroids increase with dose and time of exposure.

Antibiotic eye drop (trimethoprim and polymyxin): This medication can be associated with rash, burning/stinging sensation of the eyes, eyelid swelling, eye itching, and eye redness.

Foreseeable risks and discomforts from study procedures

Vitrectomy and Subretinal Injection: These procedures carry the following risks, many of which can result in worsened vision: damage to the retina, cataract development (sometimes requiring additional surgery), infection, bleeding within the eye, corneal abrasion resulting in corneal clouding, and increased pressure in the eye. Patients can experience redness, pain, and swelling following the procedure. There is a small risk of penetration of the eye or optic nerve from the corticosteroid injection that will be performed during the surgery, and also the possibility of bleeding. Local and possibly general anesthesia may be used during the procedure. Risks of

general anesthesia include nausea, disorientation, low blood pressure, prolonged unconsciousness or drowsiness, pain, allergic reaction, vein irritation, irregular or fast heartbeat, pneumonia, problems with breathing, stroke or death.

Blood sampling: Risks associated with blood sampling include discomfort, redness, psychological stress, bruising at the needle site of venipuncture, and bleeding.

Electrocardiogram: Risk of redness and/or irritation where the electrodes are applied to the skin.

Risks related to other study procedures: Risks associated with ophthalmologic tests include risks from eye drops. Patients may experience redness, discomfort or an allergic reaction to the eye drops used to dilate the pupil or anesthetize the cornea. Angle closure glaucoma (glaucoma caused by a sudden increase in pressure within the eye) may be caused by the dilating drops in a susceptible individual. If a patient has high blood pressure or heart disease, dilating drops can, in rare circumstances, temporarily worsen these conditions. In rare instances, measuring intraocular pressure may cause temporary irritation or scratch the front of the eye, but these effects will disappear with time. There is a small risk of falling down when performing the mobility course test; however, the study team will be watching you during the test to keep you safe.

Potential impact on candidacy for future treatment: In the event that a future alternative treatment arises, it is possible that the procedures or interventions on this protocol may complicate your candidacy for such intervention.

Risks for the unborn baby or through breastfeeding

ATSN-101 has not been studied in pregnant women and may expose the unborn baby to unknown risks including the possibility of birth defects. If you are pregnant or breastfeeding, you cannot participate. You and your partner must also follow the contraception guidelines outlined below during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics) in order to prevent pregnancy.

Risks of Genetic Testing

This research includes genetic testing. Even without your name or other identifiers, your genetic information is unique to you. The researchers believe the chance that someone will identify you is very small, but the risk may change in the future as people come up with new ways of tracing information.

There can be a risk in knowing genetic information. New health information about inherited traits that might affect you or your blood relatives could be found during a research study. Even though your genes are unique, you share some of the same genes with your blood relatives. Although we are not able to know all of the risks from taking part in research on inherited traits, we believe that the risks to you and your family are very low, because your samples will be coded.

Very rarely health or genetic information could be misused by employers, insurance companies, and others. For example, it could make it harder for you to get or keep a job or insurance, or life insurance companies may charge a higher rate based on this information. We believe the chance these things will happen is very small, but we cannot make guarantees.

A federal law (Genetic Information Non-Discrimination Act, GINA) helps reduce the risk from health insurance or employment discrimination. The law does not include other types of misuse by life insurance or long term care insurance. This law generally will protect you in the following ways:

- Health insurance companies and group health plans may not request your genetic information from this research.
- Health insurance companies and group health plans may not use your genetic information when making decisions about your eligibility or premiums.
- Employers with 15 or more employees may not use your genetic information from this research when making a decision to hire, promote, or fire you or when setting the terms of your employment.

All health insurance companies and group health plans must follow this federal law. This law does not protect you against genetic discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. If you want to learn more about GINA, you can find information about it on the internet or ask the study staff.

Birth Control

If you are a woman who can have children, you must not get pregnant or breastfeed a child prior to administration of the study drug or during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics). You must use two methods of effective birth control prior to administration of the study drug and during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics). You will also have pregnancy tests as required by the protocol because you must not be pregnant at the time the study begins or during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics). The study doctor will discuss acceptable birth control methods with you.

If you become pregnant or think you could be pregnant, you must tell the study doctor within 24 hours of finding out. You will be asked to provide information about you and your baby for 6 to 8 weeks after the birth.

If you are a man, you must not father a child during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics). You and your partner are required to use birth control methods so that your female partner does not become pregnant, if she can have children. The study doctor will discuss acceptable birth control methods with you. Birth control must be used prior to administration of the study drug and up to 3 months after the administration of the study drug.

If you father a child during the three months following administration of ATSN-101 and the associated medications (corticosteroids, antibiotics), you must tell the study doctor within 24 hours of finding out that your partner is or might be pregnant. The study doctor will ask you and your partner for authorization to collect information about her pregnancy and your baby for 6 to 8 weeks after the birth.

Potential Study Benefits

This study is experimental research and there is no guarantee that you will get any benefit from participating in it. If treatment with ATSN-101 for your *GUCY2D*-LCA is effective, you may experience an improvement in vision in the treated eye.

YOUR RIGHTS AND REGULATORY REQUIREMENTS

Stopping Study Drug or Study Participation

Once ATSN-101 has been administered into your eye, it cannot be removed. Therefore, you cannot withdraw from receiving this treatment once it has been administered. You can stop participation in the study for any reason at any point. You simply have to inform the study doctor. The data that was collected before you left the study will be analyzed as initially planned.

If you decide to stop participating in the study and no longer authorize any further collection of your personal data (withdrawal of consent), your remaining samples for future research will not be used anymore and could be destroyed (unless otherwise required to be kept because of applicable laws) but your coded data that was collected before you left the study will be kept as required by clinical regulations. You will be requested to return to the site to complete the final study visit (260 weeks), if possible.

Otherwise, your data will be stored for 15 years after the end of the study or more if required by regulations. Also, the study doctor may remove you from the study at any time. This could happen for many different reasons, for example, if there are concerns about your health, if you do not follow study instructions, or if the study gets stopped. If this happens, the study doctor will inform you and ensure that your participation in the study is stopped properly.

Once ATSN-101 is administered, you must immediately notify the study doctor if you are hospitalized. The study will be immediately stopped in case of any serious adverse event which leads to hospitalization of a study participant and could be considered related to the ATSN-101 and/or study procedures. You will be informed about the study suspension, the reason for the suspension and necessary instructions will be provided to you. Once it is confirmed that it is safe for the study to continue, you will receive all available information allowing you to confirm your decision for you to continue or not to participate in the study.

Post-Study Access to Study Drug

This study is being performed to evaluate whether ATSN-101 is safe and whether it provides effective treatment for *GUCY2D*-LCA. The results of this study will determine whether ATSN-101 will be available for use in additional patients with *GUCY2D*-LCA in future studies. In addition, the results of this study will determine whether ATSN-101 will be available for use in the treatment of your non-treated eye in a future study, if such treatment is determined to be appropriate.

Compensation and Expenses

You will have no specific expenses related to your participation in this study. The Sponsor will pay for visits, medical care, and treatments required for this study. Travel expenses will also be paid for you and an accompanying person for each study visit.

Compensation for Injury

In the event you become ill or injured as a direct result of the administration of the study drug or any procedure required under the study Protocol (written study plan), necessary medical care will be made available to you. Reimbursement for reasonable medical expenses to treat such illness or injury may be available from the Sponsor provided that: (1) the Study Drug or required procedure was administered or performed in accordance with the Protocol and all written instructions from Sponsor regarding the administration of the Study Drug or performance of such procedures; and (2) the injury is not attributable to any negligence or misconduct by the University of Pennsylvania in attending to you. It is also possible that your insurance company or you will be responsible for payment. If the Sponsor covers these expenses they will need to know some information about you such as your name, gender, date of birth, Medicare Claim Number (if you have one), and social security number (if you do not have a Medicare Claim Number). This information will be used to check to see if you receive Medicare, and if you do, report the payment they make to Medicare. This information may be collected directly from you, or from the study doctor, study staff, or other health care providers who treated your illness or injury. This information may be shared with others, including the Sponsor's representatives and the Centers for Medicare & Medicaid Services (the government agency responsible for administering the Medicare program). The Sponsor and its representatives will not use this information for any other purpose.

There are no plans for the University of Pennsylvania to pay you or give you other compensation for the injury. If you think you have been injured as a result of taking part in this research study, tell the person in charge of the research study as soon as possible. The researcher's name and phone number are listed on the first page of this consent form.

You will not lose any of your legal rights as a research subject by signing this consent form. If you think you have been injured as a result of taking part in this research study, tell the person in charge of the research study as soon as possible. The researcher's name and phone number are listed in the consent form.

You will not lose any of your legal rights when you sign this form.

What if New Information Becomes Available About the Study?

The study doctor will keep you informed during the study of any new information that could make you change your mind about your participation in the study, in particular if a new treatment for your disease becomes available. We will notify you as soon as possible if such information becomes available.

Will I receive the results of research testing?

It is possible that the tests and procedures you undergo as part of this study could reveal an unexpected finding about your health not related to the purpose of the study. You have the right to decline being notified of such findings by ticking the box on the consent form.

Most tests done in research studies are only for research and have no clear meaning for health care. Some research results related to blood testing, urine testing and electrocardiograms that are

performed at the University of Pennsylvania will be put in your medical record at this institution, and these may be returned to you upon request, or if there are any abnormalities for which the Study Doctor would recommend medical follow-up. In these instances, the Study Doctor will speak with you regarding plans for what is recommended.

Electronic Medical Records and Research Results

An Electronic Medical Record (EMR) is an electronic version of the record of your care within a health system. An EMR is simply a computerized version of a paper medical record.

A clinical trial management system (CTMS) is used to register your information as a participant in a study and to allow for your research data to be entered/stored for the purposes of data analysis and any other required activity for the purpose of the conduct of the research.

If you are receiving care or have received care within the University of Pennsylvania Health System (UPHS) (outpatient or inpatient) and are participating in a University of Pennsylvania research study, information related to your participation in the research (i.e., laboratory tests, imaging studies and clinical procedures) may be placed in your existing EMR maintained by UPHS. Information related to your participation in clinical research will also be contained in the CTMS.

If you have never received care within UPHS and are participating in a University of Pennsylvania research study that uses UPHS services, an EMR will be created for you for the purpose of maintaining any information produced from your participation in this research study. The creation of this EMR is required for your participation in this study. In order to create your EMR, the study team will need to obtain basic information about you that would be similar to the information you would provide the first time you visit a hospital or medical facility (i.e., your name, the name of your primary doctor, the type of insurance you have). Information related to your participation in the study (i.e., laboratory tests, imaging studies and clinical procedures) may be placed in this EMR.

Once placed in your EMR or in the CTMS, your information may be accessible to appropriate UPHS workforce members that are not part of the research team. Information within your EMR may also be shared with others who are determined by UPHS to be appropriate to have access to your EMR (e.g., health insurance company, disability provider, etc.).

Study Information

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.

Study Results

When this study is completed, a simple summary of the overall results will be prepared for the general public. If you do take part in the study, you will be informed at your last visit about the internet address where the summary will be available via the internet.

CONFIDENTIALITY AND PRIVACY AUTHORIZATION

Who can see or use my information? How will my personal information be protected?

We will do our best to make sure that the personal information obtained during the course of this research study will be kept private.

Records of your participation in this study will be held confidential to the extent permitted by the applicable laws and regulations, and consistent with the Health Insurance Portability and Accountability Act Authorization you are signing. However, we cannot guarantee total privacy. Your personal information may be given out if required by law.

If information from this study is published or presented at scientific meetings, your name and other personal information will not be used. The study doctor, the Sponsor, or persons working on behalf of the Sponsor and/or its representatives, the Food and Drug Administration (FDA), governmental agencies in other countries and the Institutional Review Board will be able to inspect and copy your research records which identify you by name.

The data collected from your participation in the study will be analyzed with those of all other study participants. These data will be analyzed in order to evaluate the safety and effectiveness of ATSN-101 and as requested by regulatory authorities. Atsena Therapeutics, Inc. will also have to use your data for other things, such as to plan other studies, to better understand the disease including diagnosis and associated health problems as well as to understand how ATSN-101 and similar therapies work in the body.

The Study data sent by the Study doctor or the Healthcare Institution to the Sponsor generally does not include your name or address or social security number. Instead, the Study doctor or the Healthcare Institution uses your initials and assigns a code number to your records sent to the Sponsor. However, your entire record may be reviewed or copied at the Healthcare Institution by the Sponsor and/or its representatives, and regulatory authorities or other oversight bodies. The purpose of these reviews is to assure the quality of the Study conduct and/or the data, or for other purposes allowed by law.

If you test positive for HIV, by law we have to report the positive test results to the City of Philadelphia Health Department and/or the PA Department of Health. Personal identifiers such as name, sex, date of birth, address, and phone number will be reported. For more information about the requirements reporting infectious diseases to the City of Philadelphia Health Department, please visit <https://hip.phila.gov/ReportDisease>. For more information about the requirements of reporting infectious diseases to the PA Health Department, please visit www.health.pa.gov and type 'Reportable Diseases' into the site search bar.

What may happen to my information and samples collected on this study?

Your samples may be used to create products, including some that may be sold and/or make money for others. If this happens, there are no plans to tell you, or to pay you, or to give any compensation to you or your family. Most uses of biospecimens or information do not lead to commercial products or to profit for anyone.

Whole genome sequencing will not be conducted on your samples. Whole genome sequencing involves analyzing your entire personal genetic code.

Optional Future Use of Data and/or Specimens

Your de-identified information and samples may be stored for future research purposes. This can be done without again seeking your consent in the future, as permitted by law. The future use of your information and samples only applies to the information and samples collected on this study.

Your leftover and/or extra samples collected during the study can be very useful for additional research, which may include genetic analyses for your disease or for other related diseases. There are no plans to tell you about any of the specific research that will be done. The use of your data and samples for additional research is optional and requires your consent. You can withdraw your consent at any time. In that case, samples will be destroyed and data already available will be anonymized unless otherwise required by applicable laws. Your samples may be stored for up to 15 years and your data around these samples may be stored for up to 15 years after the end of the study. These samples will be stored at Atsena Therapeutics, Inc. (or its designee) and only Atsena Therapeutics, Inc. employees or affiliates will have access.

Genetic tests give information about how you respond to the study drug based on characteristics that you may have inherited. They may also help understand your health conditions. To obtain that information, samples will be collected from you (e.g., blood, saliva etc.). Genetic analyses during the study will only provide information about the risk you have to develop the underlying disease being studied.

The following identifiers will be retained with your information and samples: the identifiers used during the study (i.e., subject ID that is assigned to you during the study).

We may share your identifiable information and samples with: Academic and medical institutions who are conducting research related to your condition and vision, Atsena Therapeutics, Inc., and any subcontractors of Atsena Therapeutics, Inc. for this trial, which may be located inside or outside the country.

We will not follow up with you to tell you about the specific research that will be done. We will not give you any results from these studies. It is possible that you may have chosen not to participate in these future research studies, had you been approached for participation.

There is a risk of breach of confidentiality (unintentional release of your information). We will do our best to make sure that this doesn't happen. However, we cannot guarantee total privacy. We will protect your confidentiality during storage and sharing by keeping your paper files in

locked rooms, keeping your electronic files stored on protected computers and servers, using coded information whenever we can, and transferring any of your records via secured channels.

You will likely not directly benefit from future research with your information and samples. Research with your identifiable information and samples may help others by improving our understanding of health and disease, improving health care and making safer or more effective medical therapies, and developing new scientific knowledge.

If you have questions about the storage of your information and samples, or have changed your mind, you can contact [REDACTED]. If you change your mind, the research results that has already been done using your samples will be retained, but your samples will be destroyed and your information will not be used in any further research.

HIPAA AUTHORIZATION

Protected Health Information (PHI) is information about you that could be used to identify you.

Please note that we may contact your caring physicians to obtain your past and present medical records for this study.

What information about me may be collected, used or shared with others?

Your protected health information that will be collected include, but are not limited to:

- Identifiers: your name, date of birth, address, telephone number, and email address
- Health Information: personal and family medical history, and results from examinations, tests and procedures.

This information could be used to identify you and may be linked to information about a disease or condition, to the types and results of various tests and procedures, to DNA samples, to results from genetic analysis, and to blood samples. This may include information in your existing medical records and information created or collected during the Study.

Why is my information being used?

Your information is used by the research team to contact you during the study. Your information and results of tests and procedures are used to:

- do the research
- oversee the research
- to see if the research was done right
- to evaluate and manage research functions.

Who may use and share information about me?

The following individuals may use or share your information for this research study:

- The Penn investigator for the study and the study team
- Wills Eye Hospital (where the subretinal injection will take place)
- Dr. Allen Ho (the study surgeon at Wills Eye Hospital) and his staff
- The Children's Hospital of Philadelphia (where the mobility course may take place) and the staff there who help with the mobility course.
- Other healthcare providers, such as laboratories, which are part of the Study
- Other authorized personnel at Penn, including offices that support research operations
- Other research personnel with access to the databases for research and/or study coordination and as otherwise approved by the IRB

Who, outside of the School of Medicine, might receive my information?

- The study Sponsor, Atsena Therapeutics, Inc.
- The Sponsor's affiliates, agents, monitors, contractors, service providers (through its clinical research organization), actual, prospective, and/or future (as yet unknown) licensees, collaborators, and/or partners (including their further licensees, collaborators, and/or partners) and their respective representatives and service providers, including for future research purposes

Oversight organizations

- The Food and Drug Administration
- Health authorities in other countries
- The Office of Human Research Protections
- The Office of Biotechnology Activities and their committees overseeing gene therapy research
- The study data and safety monitoring board

Once your personal health information is disclosed to others outside the School of Medicine, it may no longer be covered by federal privacy protection regulations.

The Principal Investigator or study staff will inform you if there are any additions to the list above during your active participation in the trial. Any additions will be subject to University of Pennsylvania procedures developed to protect your privacy.

The Study data sent by the Study doctor or the Healthcare Institution to the Sponsor generally does not include your name, address, or social security number. However, your entire record may be reviewed or copied at the Healthcare Institution by the Sponsor and/or its representatives, and regulatory authorities or other oversight bodies. The purpose of these reviews is to assure the quality of the Study conduct and/or the data, or for other purposes allowed by law.

How long may the School of Medicine use or disclose my personal health information?

Your authorization for use of your personal health information for this specific study does not expire.

Your information may be held in a research database. However, the School of Medicine may not re-use or re-disclose information collected in this study for a purpose other than this study unless:

- You have given written authorization
- The University of Pennsylvania's Institutional Review Board grants permission
- As permitted by law

Can I review my research records?

You may have the right to see and copy your protected health information related to the Study for as long as this information is held by the Study doctor or the Healthcare Institution. However, to ensure the scientific integrity of the Study, you agree that you may not be able to review some of your records related to the Study until after the Study has been completed.

Can I change my mind about giving permission for use of my information?

Yes. You may withdraw or take away your permission to use and disclose your health information at any time. You do this by sending written notice to the investigator for the study. If you withdraw your permission, you will not be able to stay in this study.

If you revoke this Authorization, the Study doctor or the Healthcare Institution will no longer use or disclose your protected health information under the Authorization for this Study, unless the Study doctor or the Healthcare Institution needs to use or disclose some of your protected health information to preserve the scientific integrity of the Study or if the protected health information relates to the safety of the Study drug. Information given to the Sponsor and/or its representatives before you revoke this Authorization may still be used.

What if I decide not to give permission to use and give out my health information?

You will not be able to be in this research study.

You will be given a copy of this Research Subject HIPAA Authorization describing your confidentiality and privacy rights for this study.

By signing this document you are permitting the School of Medicine to use and disclose personal health information collected about you for research purposes as described above.

Who can I call with questions, complaints or if I'm concerned about my rights as a research subject?

If you have questions, concerns or complaints regarding your participation in this research study or if you have any questions about your rights as a research subject, you should speak with the Principal Investigator listed on page one of this form. If a member of the research team cannot be reached or you want to talk to someone other than those working on the study, you may contact the Office of Regulatory Affairs with any question, concerns or complaints at the University of Pennsylvania by calling (215) 898-2614.

Consent

I declare that I have understood all the above information related to ATSN-101-1, that I have had the opportunity to ask questions, that I voluntarily agree to participate, and have personally printed, signed and dated this form (below). A copy of this combined consent and HIPAA authorization form will be provided to me. All information, including the reasons for conducting the study, its organization, the study drugs, all required tests and procedures as well as the associated risks were explained to me. I am aware that the study doctor may provide additional important health information associated with my participation or information that may affect my

willingness to continue the study. I am aware that my withdrawal from this study for whatever reason will not affect in any way the quality of health care provided to me. By signing this informed consent and HIPAA authorization, I agree to participate in the study and understand that I am not giving up any of my legal rights. I declare that I have truthfully answered all questions about my medical history and that I will follow all rules listed in the document. If I want to get any additional information about this study or the development of the study drug, I can ask the study doctor, the respective ethics committee, or the Sponsor.

I understand that by signing this form, I also voluntarily consent to:

- My medical and personal data being collected during the study as it was described to me, and accessed and reviewed by authorized people.
- My coded data being used by the Sponsor and Sponsor's affiliates, agents, monitors, contractors, service providers (through its clinical research organization), actual, prospective, and/or future (as yet unknown) licensees, collaborators, and/or partners (including their further licensees, collaborators, and/or partners) and their respective representatives and service providers, including for future research purposes.
- My coded data being sent to persons or organizations located in countries that do not have equivalent levels of data protection as in my country. I understand that the Sponsor monitors this sharing and takes all possible measures to protect my privacy.
- My biological samples being collected and analyzed and that those tests may include genetic analysis.

The collection of extra samples, storage and use for future research of my data and leftover/extra samples, including genetic testing	Yes ☐	No ☐
Being informed about new findings concerning my health	Yes ☐	No ☐
I agree for the study doctor to inform my family doctor about my participation in the study.	Yes ☐	No ☐
I received information about my rights according to Health Insurance and Portability and Accountability Act (HIPAA)	Yes ☐	No ☐

The study doctor may notify your physician of your participation in the study and may share relevant medical information during the study if necessary for managing your health and safety throughout. If you agree, please indicate name and contact details of your physician below:

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PARTICIPANT

Name		Signature		Date	
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PARENT OR LEGALLY ACCEPTABLE REPRESENTATIVE (LAR)

Name		Signature		Date	
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WITNESS

Name		Signature		Date	
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PERSON OBTAINING CONSENT

I declare that I have explained the study and confirm that all of the study participant's questions have been answered before the document was signed and the study participant has been provided with a signed copy of the current version of informed consent document.

Name		Signature		Date	
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GLOSSARY

Anonymized Data: All the information that can be linked to you has been removed from your data.

Coded Data: Your contact and personal information are separated from medical information but can be linked by authorized people through a key.

Gene: Genes are the units of deoxyribonucleic acid (DNA), the chemical structure carrying your genetic information that determine many human characteristics such as eye color, height, and weight.

Personal Data: Any information related to you such as name, contact and personal information, or your complete medical record.

Study Doctor: The study doctor is the doctor in charge of the study at the medical office (study site) you visit. The study doctor receives fees from the Sponsor to conduct the study.