

The LIGHTHOUSE Study



ATSN-201-1

What to Expect

An Overview of Study Visits and Procedures

THE LIGHTHOUSE STUDY

Here is an overview of the procedures you can expect during your visits to the study site if you decide to participate in the LIGHTHOUSE study.

Randomization

After your doctor determines if one or both eyes can be treated, a computer randomly assigns you to receive treatment or to be in the control group. If you're in the control group, you may receive treatment after 12 months.

Eye Exam

The study doctor will perform an eye exam during almost every visit (except for the day of surgery). During this exam, your eyes will be numbed and dilated with eye drops so that the study doctor can measure your eye pressure and evaluate the retina and other parts of your eye.

Blood Draw and Other Samples

You will periodically have blood draws so that we can monitor safety, perform genetic testing, and measure your immune response and study drug distribution after the gene therapy. You will also be asked to provide urine, nasal, saliva, and tear samples during the study. If you are a female who is able to get pregnant, a pregnancy test will be done.

Visual Acuity Tests

You will be asked to read letters of different sizes from a chart while wearing special glasses. This exam measures how well you are able to see letters under dim and bright lighting conditions.

Microperimetry

You will be asked to click a button when you notice lights appear in your peripheral vision while you look straight ahead. These tests create a "map" of your vision based on how much light you see.

Reading Speed

Each of your eyes will be tested to see how quickly you can read (for participants age 8 and older).

Optical Coherence Tomography (OCT)

An instrument will use light to scan your eye and create 3-dimensional images that can be used to evaluate the thickness of the layers of your retina.

Fundus Autofluorescence and Fundus Photography

Instruments will take photographs of the back of your eye.

Questionnaires and Global Impression of Change and Severity

You will be asked to answer questions about how your vision affects your life and changes in your symptoms.

Exit Interview

You or your caregiver (if participant is younger than 11 years old) may be asked about your experience participating in the study. If selected, the exit interview will last approximately 30 minutes and may take place over the phone or an internet platform (Zoom).

Assigned to Unilateral Treatment (one eye treated)

VISIT	DATE SCHEDULED	Eye Exam	Blood Draw and/or Samples	Visual Acuity Tests	OCT & Fundus Autofluorescence	Fundus Photography	Reading Speed	Microperimetry	Questionnaires
Screening/ Baseline*		●	●	●	●	●	●	●	●
Pre-surgery		●	●	●					
Day 0	Study Drug Administration (Unilateral)								
Day 1		●	●	●	●				
Day 7		●	●	●	●				
Day 14		●	●	●	●				
Day 28		●	●	●	●			●	
Week 6		●		●	●				
Week 8		●	●	●	●			●	
Week 12		●	●	●	●	●	●	●	●
Month 6		●	●	●	●		●	●	●
Month 9		●		●	●			●	
Month 12		●	●	●	●	●	●	●	★
Month 24		●		●	●	●		●	
Month 36		●		●	●	●		●	
Month 48		●		●	●	●		●	
Month 60		●		●	●	●	●	●	●

*The screening/baseline visit could take 3 or more days to complete over multiple visits.

★ If selected to participate in an exit interview, exit interviews will be conducted at or after Month 12

This is not a comprehensive list and there may be additional procedures completed during your visits if the study doctor determines it is necessary. Some visits may need to take place over multiple days.

Example Visit Calendar

Unilateral Treatment

The calendar below provides an example visit calendar through Month 9 if one eye is treated on January 6, 2026.

● Clinic Visit ● Surgery Day

JANUARY						
S	M	T	W	T	F	S
				1	PS	3
4	5	D0	D1	8	9	10
11	12	D7	14	15	16	17
18	19	D14	21	22	23	24
25	26	27	28	29	30	31

FEBRUARY						
S	M	T	W	T	F	S
1	2	D28	4	5	6	7
8	9	10	11	12	13	14
15	16	W6	18	19	20	21
22	23	24	25	26	27	28

MARCH						
S	M	T	W	T	F	S
1	2	W8	4	5	6	7
8	9	10	11	12	13	14
15	16	17	18	19	20	21
22	23	24	25	26	27	28
29	30	W12				

APRIL						
S	M	T	W	T	F	S
			1	2	3	4
5	6	7	8	9	10	11
12	13	14	15	16	17	18
19	20	21	22	23	24	25
26	27	28	29	30		

MAY						
S	M	T	W	T	F	S
					1	2
3	4	5	6	7	8	9
10	11	12	13	14	15	16
17	18	19	20	21	22	23
24	25	26	27	28	29	30
31						

JUNE						
S	M	T	W	T	F	S
	1	2	3	4	5	6
7	8	9	10	11	12	13
14	15	16	17	18	19	20
21	22	23	24	25	26	27
28	29	30				

JULY						
S	M	T	W	T	F	S
			1	2	3	4
5	M6	7	8	9	10	11
12	13	14	15	16	17	18
19	20	21	22	23	24	25
26	27	28	29	30	31	

AUGUST						
S	M	T	W	T	F	S
						1
2	3	4	5	6	7	8
9	10	11	12	13	14	15
16	17	18	19	20	21	22
23	24	25	26	27	28	29
30	31					

SEPTEMBER						
S	M	T	W	T	F	S
		1	2	3	4	5
6	7	8	9	10	11	12
13	14	15	16	17	18	19
20	21	22	23	24	25	26
27	28	29	30			

OCTOBER						
S	M	T	W	T	F	S
				1	2	3
4	5	M9	7	8	9	10
11	12	13	14	15	16	17
18	19	20	21	22	23	24
25	26	27	28	29	30	31

NOVEMBER						
S	M	T	W	T	F	S
1	2	3	4	5	6	7
8	9	10	11	12	13	14
15	16	17	18	19	20	21
22	23	24	25	26	27	28
29	30					

DECEMBER						
S	M	T	W	T	F	S
		1	2	3	4	5
6	7	8	9	10	11	12
13	14	15	16	17	18	19
20	21	22	23	24	25	26
27	28	29	30	31		

Remaining Visits:

January 6, 2027 (M12); January 6, 2028 (M24); January 6, 2028 (M36); January 6, 2029 (M48); January 6, 2030 (M60)

Assigned to Bilateral Treatment (both eyes treated)

VISIT	DATE SCHEDULED	Eye Exam	Blood Draw and/or Samples	Visual Acuity Tests	OCT & Fundus Autofluorescence	Fundus Photography	Reading Speed	Microperimetry	Questionnaires
Screening/ Baseline*		●	●	●	●	●	●	●	●
Pre-surgery		●	●	●					
Day 0A		Study Drug Administration (First Eye)							
Day 1A		●	●	●	●				
Day 7A		●	●	●	●				
Day 14A		●	●	●	●				
Day 0B		Study Drug Administration (Second Eye)							
Day 1B		●	●	●	●				
Day 7B		●	●	●	●				
Day 14B		●	●	●	●				
Day 28		●	●	●	●			●	
Week 6		●		●	●				
Week 8		●	●	●	●			●	
Week 12		●	●	●	●	●	●	●	●
Month 6		●	●	●	●		●	●	●
Month 9		●		●	●			●	
Month 12		●	●	●	●	●	●	●	★
Month 24		●		●	●	●		●	
Month 36		●		●	●	●		●	
Month 48		●		●	●	●		●	
Month 60		●		●	●	●	●	●	●

*The screening/baseline visit could take 3 or more days to complete over multiple visits.



If selected to participate in an exit interview, exit interviews will be conducted at or after Month 12

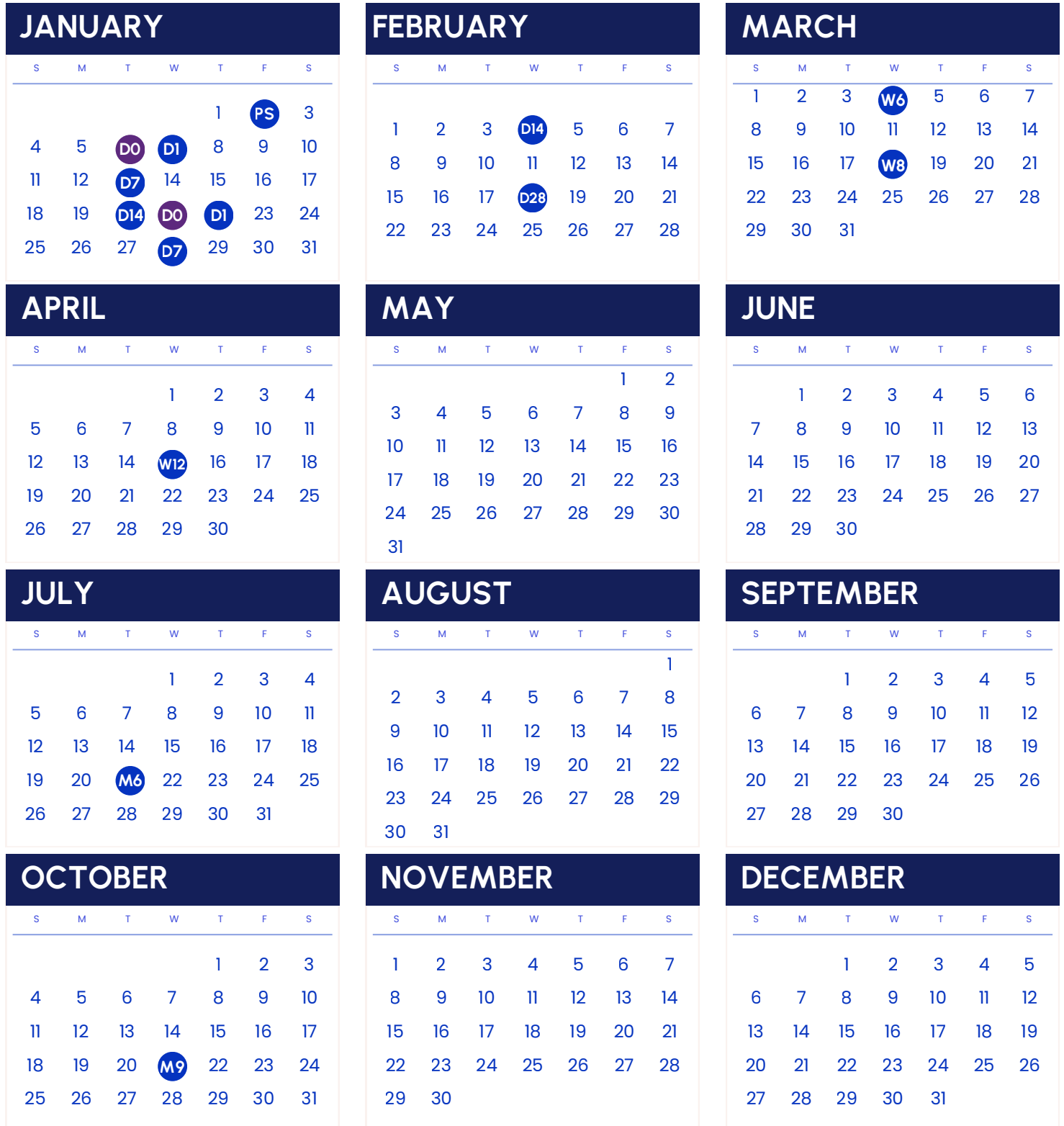
This is not a comprehensive list and there may be additional procedures completed during your visits if the study doctor determines it is necessary. Some visits may need to take place over multiple days.

Example Visit Calendar

Bilateral Treatment

The calendar below provides an example visit calendar through Month 9 if the first eye is treated on January 6 and the second eye is treated on January 21st.





● Clinic Visit ● Surgery Day



Remaining Visits:

January 21, 2027 (M12); January 21, 2028 (M24); January 21, 2028 (M36); January 21, 2029 (M48); January 21, 2030 (M60)

Assigned to Control Group

VISIT	DATE SCHEDULED	Eye Exam	Blood Draw and/or Samples	Visual Acuity Tests	OCT & Fundus Autofluorescence	Fundus Photography	Reading Speed	Microperimetry	Questionnaires
Screening/ Baseline*		●	●	●	●	●	●	●	●
Pre-surgery		●	●	●					
Day 0		 Phone call							
Day 1		 Phone call							
Day 7		 Phone call							
Day 14		 Phone call							
Day 28		●	●	●	●			●	
Week 6		●		●	●				
Week 8		●	●	●	●			●	
Week 12		●	●	●	●	●	●	●	●
Month 6		●	●	●	●		●	●	●
Month 9		●		●	●			●	
Month 12		●	●	●	●	●	●	●	★

*The screening/baseline visit could take 3 or more days to complete over multiple visits.

 If selected to participate in an exit interview, an exit interview will be conducted at or after Month 12

After Month 12, individuals in the Control group may choose to cross over and receive ATSN-201. Visits will restart at Day 0 and include 12 months of the Main Study Period, followed by 4 additional years of follow-up.

This is not a comprehensive list and there may be additional procedures completed during your visits if the study doctor determines it is necessary. Some visits may need to take place over multiple days.

Example Visit Calendar

Control Group

The calendar below provides an example calendar of in-person visits through Month 9 if assigned to the control group after screening.

 Clinic Visit  Phone Call



Remaining Visits:
January 6, 2027 (M12)

VITRECTOMY WITH SUBRETINAL INJECTION



On "Day 0" of the study, you will receive the study drug in one eye through a surgical procedure called a vitrectomy with subretinal injection. Here is an overview of how the surgery works:

Before the Surgery:

Starting 2 days before surgery, you will be asked to begin taking a corticosteroid pill called prednisone. You will also be asked to fast starting 12 hours before surgery.



Day of the Surgery:

The surgery will be performed in an operating room in a hospital or surgical center. On the day of the surgery, you may receive general anesthesia or conscious sedation. 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 inject the study drug beneath the retina. They will also inject an antibiotic and corticosteroid around the eye during surgery and administer an IV steroid in order to reduce the risk of infection and inflammation from the surgery.

After the Surgery:

You will be asked to continue taking the steroid pills and use antibiotic eye drops and steroid eye drops after the surgery. You will also return to the clinic several times after surgery to perform different tests and evaluations.



If receiving bilateral treatment (treatment in both eyes), the surgeries will be done separately for each eye. Surgeries will take place 14-28 days apart.