



The ACCESS-1 **Study Schedule and Planner**

For adults with Paroxysmal Nocturnal Hemoglobinuria (PNH) who are new to treatment* or have not received treatment* in the last 6 months.

*Treatment with a complement inhibitor



Print code

REGN -Access-1 Study - Study Schedule and Planner - 11-Jul-2022 - V2.0

Thank you for joining the ACCESS-1 Study.

This booklet explains what will happen at study visits. It also has space for you to keep track of scheduled appointments and helpful information and reminders. It is important that you attend all study visits so the doctor can keep an eye on your health and understand how the study drug may be affecting your body.

Your participation is greatly appreciated. You may or may not directly benefit from being in this study, but information learned may help others with PNH in the future.

As with all medicines, there is a chance for side effects. You will be closely monitored throughout the entire study.

The study team will happily answer any questions you may have. Remember, participation is voluntary, and you can choose to leave the study at any time.

With much appreciation,
The ACCESS-1 Study team

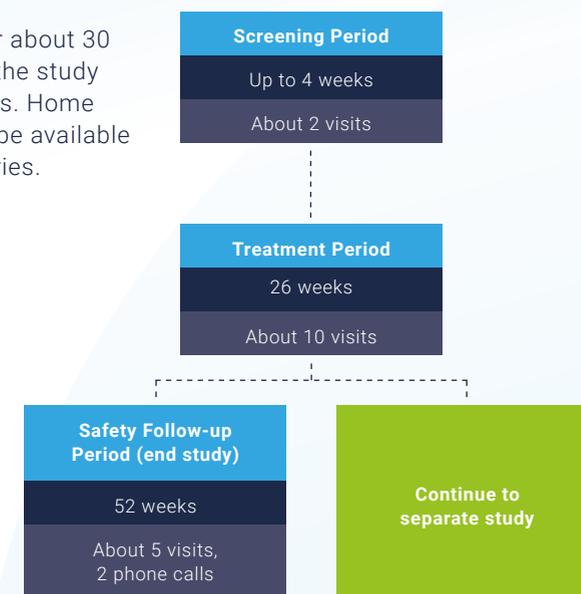
Study purpose

The purpose of this study is to learn more about the experimental combination treatment of **pozelimab** and **cemdisiran**—to see how safe and effective they are compared to an already approved treatment, ravulizumab. These study drugs are known as “complement inhibitors.”

Study duration and visits

You will be in the main study for about 30 weeks and will have visits with the study team for tests and health checks. Home healthcare or virtual visits may be available for certain visits in some countries.

- If you stop taking the study drug for any reason, you will have checkups for about 1 year (52 weeks) after your final dose (**Safety Follow-up**).
- If you complete the main study, you may have the option to continue into another study.



Screening

Visit 1 Date:_____ Time:_____

Visit 2 Date:_____ Time:_____

Before starting the study, you will need to give your permission to join by reading and signing an **Informed Consent Form**.



The study doctor will then collect information and run tests to make sure you are eligible for the study. **You will also be required to be up-to-date with certain vaccinations in order to lower your risk of infection.**

HEALTH INFORMATION



Medical history



Medicine review



Transfusion review

TESTS AND HEALTH CHECKS



Questionnaires



Height



Weight



Vital signs



Physical exam



Heart activity (electrocardiogram)



Blood test*



Urine test



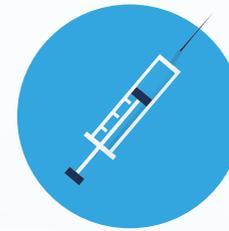
Vaccinations (if needed)

There may be additional tests. Talk to the study doctor for more information.

*Participants who are able to become pregnant will have their blood tested for pregnancy.

Study Treatment

You will be randomly assigned (similar to flipping a coin) to receive either the study drug combination (pozelimab + cemdisiran) or another treatment, called ravulizumab.



**POZELIMAB* +
CEMDISIRAN**

Pozelimab and cemdisiran work together to reduce the levels and activity of C5, a protein involved in the destruction of red blood cells in people with PNH.

Pozelimab is a type of drug called a monoclonal antibody that is designed to block the activity of C5.

Cemdisiran is designed to block genetic material that codes for C5. As a result, less C5 is made.



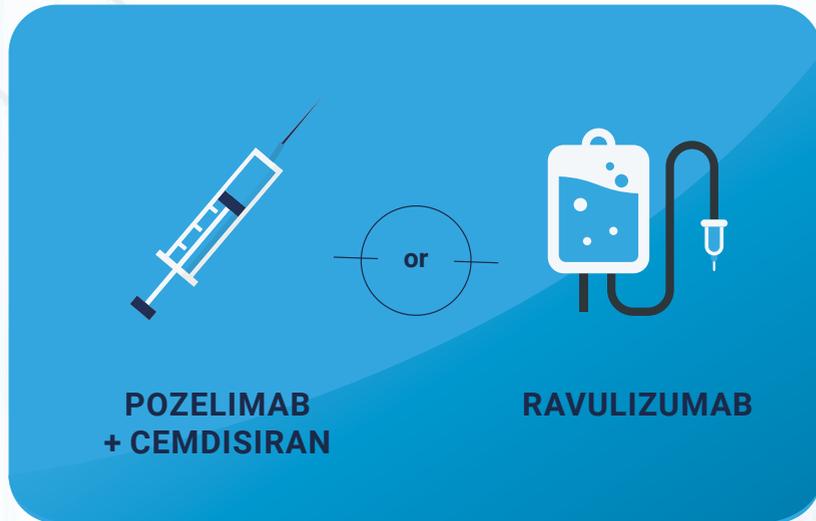
RAVULIZUMAB

Ravulizumab is a drug that is approved for the treatment of PNH in some countries. It is a monoclonal antibody that is designed to block the activity of C5.

OR

*The first dose of pozelimab is given as an infusion.

Taking the Study Treatment



Pozelimab and cemdisiran are injected under the skin. On Day 1, the first dose of pozelimab is given as an infusion,* followed by injections of both study drugs. After Day 1, each study drug is given as an injection **every 4 weeks**.

- The study staff may be able to train you or a caregiver to give your injections at home.

Ravulizumab is given as an infusion* on Day 1, Day 15, and then **every 8 weeks**. You may have the option of receiving your infusions at home from a home healthcare nurse.

In addition to required vaccinations, you may also take antibiotics while receiving study treatment and after completing your final dose to help lower the risk of infection (based on the advice of your doctor).

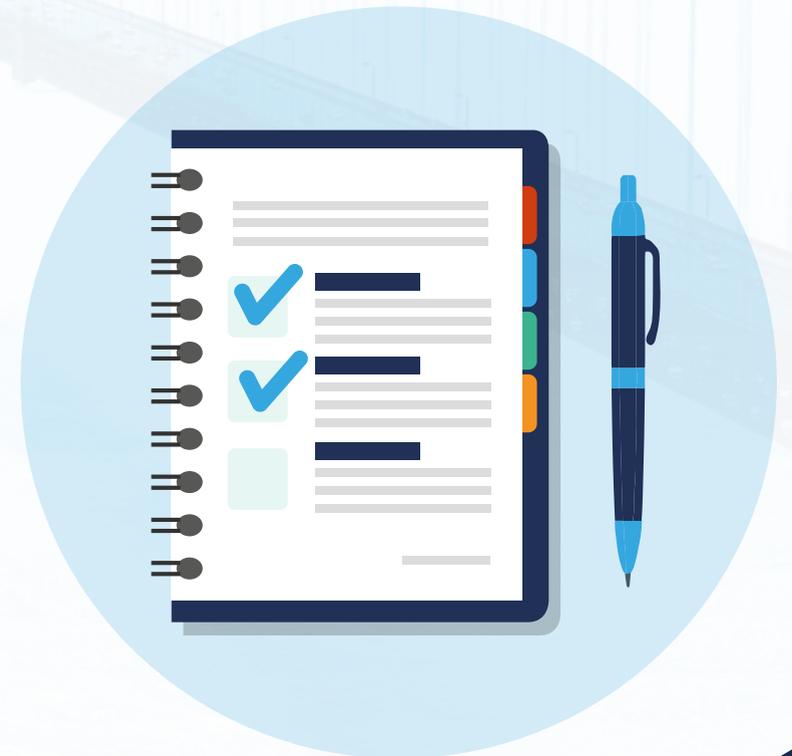
Patient diary

If you are receiving doses of study drug at home, you will use a diary to keep track of:

- Time and date of study drug injections.
- Where on the body the injections are given.

Use the separate electronic diary to keep track of your health, any PNH symptoms you may have, and general quality of life. You will be asked to bring the diary to each study visit.

Report any side effects that you may have between study visits to the study doctor right away.



*An infusion means the medicine is given slowly through a needle into a vein in the arm.

Treatment Period

26 weeks | Visits 3 to 12

HEALTH INFORMATION



Medicine review



Transfusion review



Review side effects

TESTS AND HEALTH CHECKS



Questionnaires*



Weight



Vital signs



Physical exam



Heart activity (electrocardiogram)



Blood tests



Urine test**

There may be additional tests. Talk to the study doctor for more information.

Remember to use your electronic diary every day to complete the questionnaire as well as the home dosing diary if you are receiving study treatment at home.

You will be monitored throughout the study for any side effects from the study drug.

*You will answer questions about your PNH symptoms every day for 14 days before Visit 3 and throughout the Treatment Period.

**Participants who are able to become pregnant will have their urine tested for pregnancy.

Treatment Period

26 weeks | Visits 3 to 12

Use the chart below to keep track of your scheduled visits and important information.

Remember to bring your study diaries with you to each visit.

Visit	Week	Scheduled date/time	Treatment visit?*(Y/N)	Notes and reminders
3	Week 0 (Day 1)		Y	
4	Week 2			
5	Week 4			
6	Week 8			
7	Week 10			

*Pozelimab and cemdisiran are given on Day 1, then every 4 weeks. Ravulizumab is given on Day 1, Day 15, and then every 8 weeks.

Treatment Period

26 weeks | Visits 3 to 12

Visit	Week	Scheduled date/time	Treatment visit?*(Y/N)	Notes and reminders
8	Week 12			
9	Week 16			
10	Week 18			
11	Week 20			
12	Week 24			

Safety Follow-up Period

52 weeks | 5 visits, 2 phone calls

If you stop taking the study drug for any reason, or if you do not wish to continue to another study, you will have checkups for about 1 year after your final dose.

You will not have all tests at every visit.

HEALTH INFORMATION



Medicine review



Review side effects

TESTS AND HEALTH CHECKS



Weight



Vital signs



Physical exam



Blood test



Urine test*

There may be additional tests. Talk to the study doctor for more information.

You will be monitored throughout the study for any side effects from the study drug.

*Pozelimab and cemdisiran are given on Day 1, then every 4 weeks. Ravulizumab is given on Day 1 and Day 15, then every 8 weeks.

*Participants who are able to become pregnant will have their urine tested for pregnancy once a month.

Safety Follow-up Period

52 weeks | 5 visits, 2 phone calls

Use the chart below to keep track of your scheduled visits and important information.

Visit	Week*	Scheduled date/time	Notes and reminders
1	Week 4		
2	Week 8		
3	Week 12		
4	Week 16		
5	Week 26		
6	Week 38		Phone call
7	Week 52		Phone call

*Number of weeks after your final dose of study drug.

Transition (if needed)

Up to 10 weeks | Up to 3 visits

If you are receiving ravulizumab and wish to enter a separate follow-on study, you may have a few extra visits between studies.

You will not have all tests at every visit.

HEALTH INFORMATION



Medicine review



Transfusion review

TESTS AND HEALTH CHECKS



Weight



Vital signs



Blood test



Urine test*

There may be additional tests. Talk to the study doctor for more information.

You will be monitored throughout the study for any side effects from the study drug.

*Participants who are able to become pregnant will have their urine tested for pregnancy.

Transition (if needed)

Up to 10 weeks | Up to 3 visits

Use the chart below to keep track of your scheduled visits and important information.

Visit	Week*	Scheduled date/time	Notes and reminders
1	Week 2		
2 (if needed)	Week 6		
3 (if needed)	Week 10		

*Number of weeks after completing the Treatment Period.

Study contact information

Contact the study team if you have any questions or if you need to reschedule an appointment.

Study doctor's name:

Phone number:

Study coordinator's name:

Phone number:

Email:

Glossary



Blood test:

Blood tests can measure how much study drug is in the body and how it may be affecting other parts of the body. Your blood may also be tested to see if you have ever been infected with the hepatitis B or C virus or with the bacteria that causes tuberculosis (TB). Participants who are able to become pregnant will also have their blood tested for pregnancy.



Heart activity:

A painless test called an electrocardiogram (ECG) will be used to check the electrical activity of your heart. You will lie down, and small, sticky pads (electrodes) will be placed on your chest. The pads are connected with wires to a computer.



Height:

A member of the study team will measure how tall you are without shoes.



Informed consent:

If you agree to join the study, you will sign forms to give your permission, or consent.



Medical history:

The doctor will ask questions about your health, including your PNH and any surgeries or medical procedures you may have had.



Medicine review:

The study doctor will ask questions about medicines you are taking now or have taken in the past, including any blood transfusions and vaccinations.



Physical exam:

The doctor will check different parts of your body, such as the heart, lungs, skin, throat, and stomach.



Questionnaires:

You will use an electronic device to answer questions about how you are feeling, how tired you are, your health, your symptoms of PNH, and how satisfied you are with taking the study drug.



Transfusion review:

The study doctor will ask you about any blood transfusions you have needed in the past or during the study between visits.



Urine test:

A sample of your urine will be tested for sugar, protein, and blood. Urine tests can also see how your kidneys are working. Participants who are able to become pregnant will also have their urine tested for pregnancy.



Vaccinations:

In order to lower your risk of infection, you are required to have certain vaccinations before participating in the study. If needed, you may need to be re-vaccinated during the study.



Vital signs:

The study team will measure your blood pressure (how your heart is pumping), body temperature, and pulse (number of times your heart beats in 1 minute).



Weight:

The study doctor will use a scale to weigh you without shoes while wearing light clothing.

