



Participant User Guide

Register for an Account

- Step 1: Select the appropriate Account Type. If you need more information to help you choose, click “Not sure? Help me choose”.
 - If you have a diagnosis of Paroxysmal Nocturnal Hemoglobinuria, select **Participant Account**.
 - If you are entering information for someone else who has Paroxysmal Nocturnal Hemoglobinuria (or you have Paroxysmal Nocturnal Hemoglobinuria and are also entering information for yourself), select **Caregiver Account**.

A screenshot of the AA·MDS International Foundation website's account registration page. The page has a white background with a light blue and orange decorative border. At the top, it says "Featuring" above the AA·MDS logo. Below the logo, the heading "Select Account Type" is centered. There are two white rectangular boxes with rounded corners. The left box contains the text "I have a rare disease, condition, and/or diagnosis." followed by "Participant Account". The right box contains "I am a family member or guardian of someone with a rare disease." followed by "Caregiver Account". At the bottom left, there is a link "Return to login" with a circular arrow icon. At the bottom right, there is a link "Not sure? Help me choose.".

- Step 2: Read the Terms and Conditions and Privacy Policy and attest to the statements provided. When you are finished with this page, click “Next”.

The screenshot shows the 'Participant Registration' page for AA-MDS International Foundation. At the top, it features the AA-MDS logo and a progress bar with five steps: Terms & Conditions (active), Contact Info, Notifications, Review & Submit, and Confirmation. Below the progress bar, there is a paragraph of text explaining the purpose of the Terms and Conditions and Privacy Guidelines. Below this text are four checkboxes for user consent, each followed by a line of text. At the bottom left, there is a 'Return to login' link, and at the bottom right, there is a 'Next' button.

Featuring
AA•MDS
INTERNATIONAL FOUNDATION

Participant Registration

Terms & Conditions | Contact Info | Notifications | Review & Submit | Confirmation

Below are links to the IAMRARE Terms of Use and Privacy Guidelines. The purpose of these documents is to outline your rights and responsibilities when using the platform. These documents include: 1) Standard policies for all studies on this platform, 2) A privacy statement that details how your data can be used, 3) Information outlining the unacceptable uses of the platform, and 4) Information about how to address questions and issues.

- You are at least 18 years of age, the age of majority in your state, province or country, and able to consent on behalf of yourself and/or an individual that you have legal responsibility for. *
- You agree to support the Platform's research activities by providing truthful, appropriate information and to not do anything that will put the Services or the information in the Platform at risk. *
- You understand that NORD will use reasonable efforts to keep the information you enter on the Services safe, but no data transmissions over the Internet can be guaranteed to be 100% secure. The information you provide will be available to authorized users at NORD for platform maintenance and research activities, as well as to the sponsor of the studies you consent to participate in. *
- You agree to the [Terms and Conditions](#) & [Privacy Policy](#). *

[Return to login](#) [Next](#)

- Step 3: Enter your personal information in the spaces provided. When you are finished with this page, click “Next”.

The screenshot shows the 'Participant Registration' page for AA-MDS International Foundation, Step 3: Personal Information. The progress bar shows 'Terms & Conditions' as completed and 'Contact Info' as the current step. Below the progress bar, there are three input fields: 'Country of Residence' (a dropdown menu), 'First Name' (a text input field), and 'Last Name' (a text input field). Below these is an 'E-mail' input field. At the bottom left, there is a 'Return to login' link, and at the bottom right, there are 'Previous' and 'Next' buttons.

Featuring
AA•MDS
INTERNATIONAL FOUNDATION

Participant Registration

Terms & Conditions | Contact Info | Notifications | Review & Submit | Confirmation

Country of Residence *

First Name * Last Name *

E-mail *

[Return to login](#) [Previous](#) [Next](#)

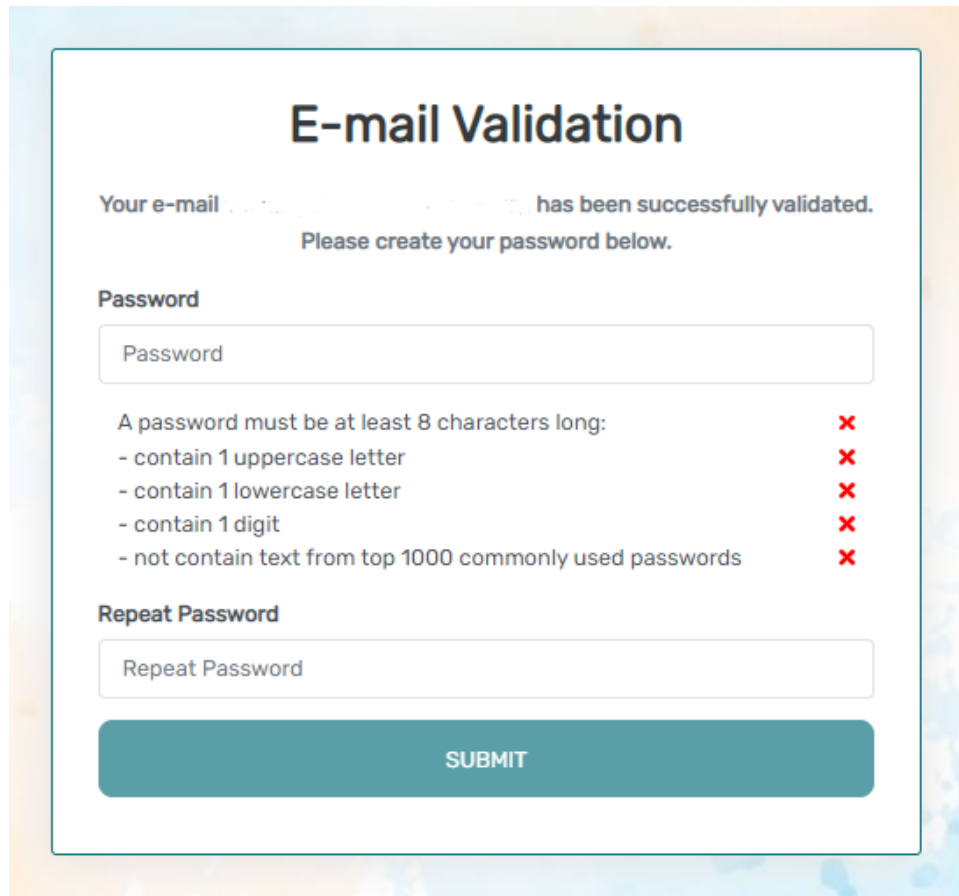
- Step 4: Select whether you are interested in being contacted by NORD regarding available studies. When you are finished with this page, click “Next”.

The screenshot shows the 'Participant Registration' page for AA·MDS International Foundation. At the top, it says 'Featuring' above the AA·MDS logo. Below the logo is a progress bar with five steps: 'Terms & Conditions', 'Contact Info', 'Notifications', 'Review & Submit', and 'Confirmation'. The 'Review & Submit' step is currently active, indicated by a dark teal bar under the progress bar. Below the progress bar, there is a question: 'I am interested in NORD contacting me regarding available studies.' with two radio button options: 'Yes' and 'No'. At the bottom left, there is a link 'Return to login'. At the bottom right, there are two buttons: 'Previous' and 'Next'.

- Step 5: Select “Next” so that an activation link is sent to your e-mail to complete registration.

This screenshot is identical to the one above, but the progress bar now shows that the 'Review & Submit' step is complete and the 'Confirmation' step is active. Below the progress bar, the text reads: 'An activation link will be sent to youremail@email.com. Click "Next" to send this e-mail and continue.' At the bottom right, the 'Next' button is highlighted with a red arrow, indicating it should be clicked to proceed.

- Step 6: Click the link you are sent via e-mail. Please check your Spam folder if you do not see the e-mail. You will be taken to the following screen in a new tab within your browser. Set your password and click “Submit”.



E-mail Validation

Your e-mail [redacted] has been successfully validated.
Please create your password below.

Password

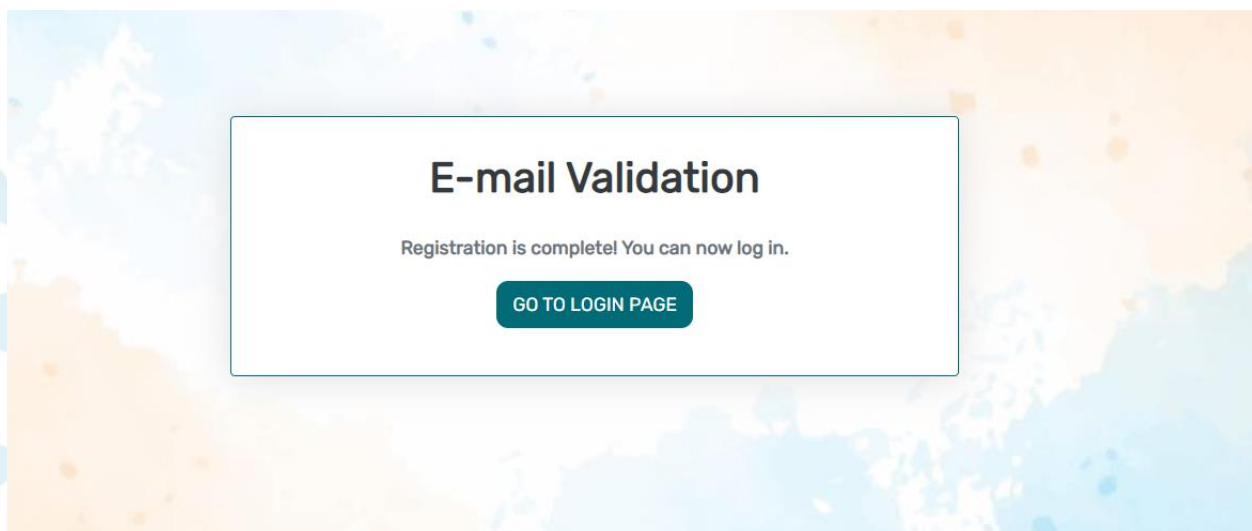
A password must be at least 8 characters long: ✘

- contain 1 uppercase letter ✘
- contain 1 lowercase letter ✘
- contain 1 digit ✘
- not contain text from top 1000 commonly used passwords ✘

Repeat Password

SUBMIT

- Step 7: Your validation is now complete. Select “Go to Login Page”.

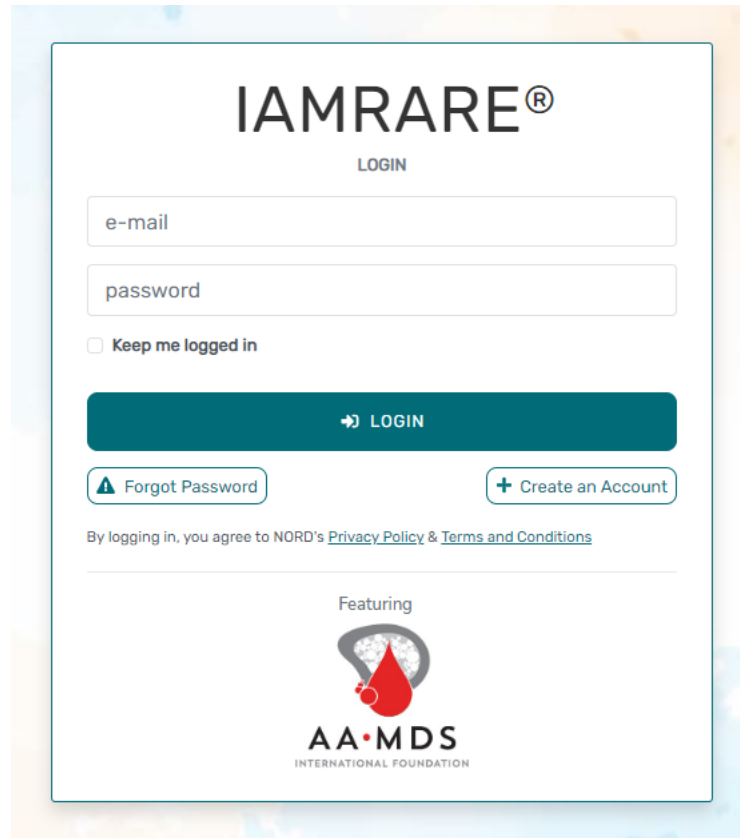


E-mail Validation

Registration is complete! You can now log in.

GO TO LOGIN PAGE

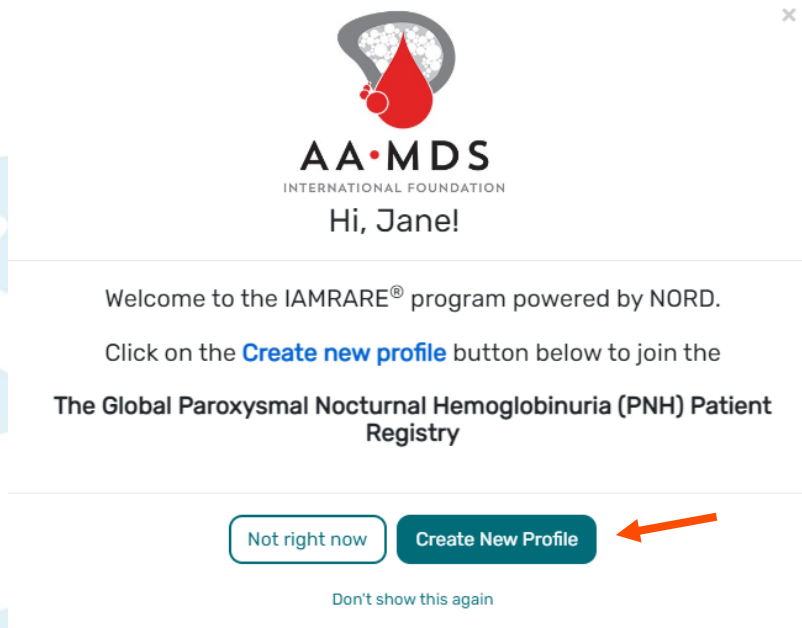
- Step 8: Log in using your new e-mail and password.



The image shows a login form for IAMRARE. At the top, it says "IAMRARE®" and "LOGIN". Below that are two input fields: "e-mail" and "password". There is a checkbox labeled "Keep me logged in". A large teal button with a right arrow and the word "LOGIN" is centered. Below the button are two smaller buttons: "Forgot Password" (with a warning triangle icon) and "Create an Account" (with a plus icon). At the bottom, there is a line of text: "By logging in, you agree to NORD's [Privacy Policy](#) & [Terms and Conditions](#)". Below this is a section titled "Featuring" with the AA·MDS International Foundation logo, which consists of a stylized red blood drop and the text "AA·MDS INTERNATIONAL FOUNDATION".


Create Profile

- Step 1: To start, click Create New Profile.



The image shows a "Create Profile" dialog box. At the top right is a close button (X). The AA·MDS International Foundation logo is centered, followed by the text "Hi, Jane!". Below this is a message: "Welcome to the IAMRARE® program powered by NORD. Click on the [Create new profile](#) button below to join the **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**". At the bottom, there are two buttons: "Not right now" and "Create New Profile". An orange arrow points to the "Create New Profile" button. Below the buttons is a link: "Don't show this again".


- Step 2: Fill out the Participant Profile

 Complete Participant Profile ✕

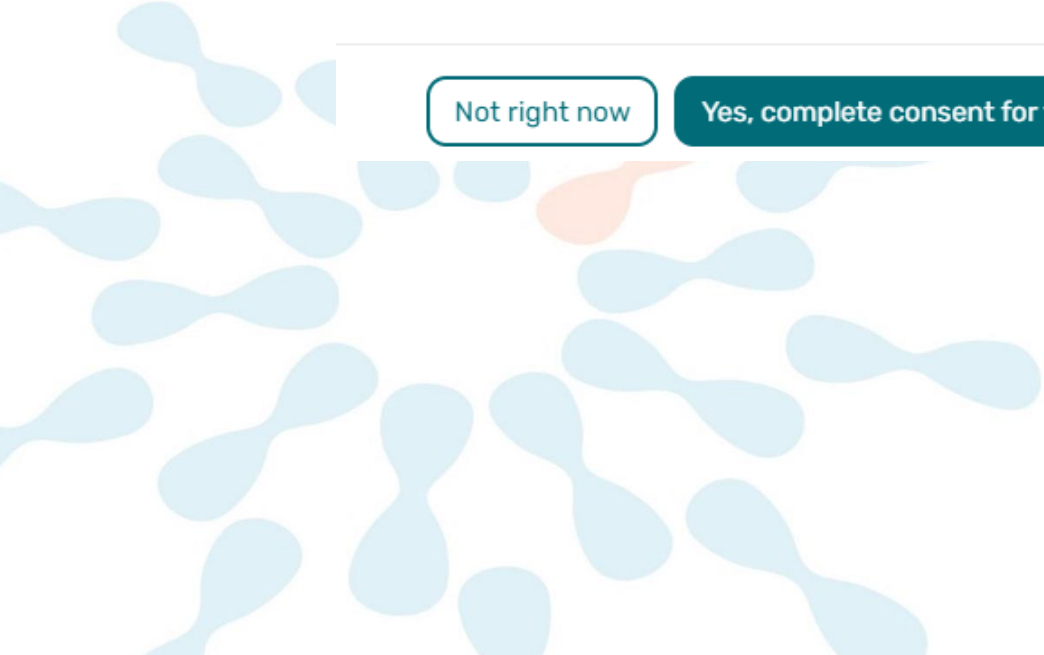
Preferred First Name * <input type="text" value="Jane"/>	Current Last Name * <input type="text" value="Doe"/>
First Name on Birth Certificate * <input type="text" value="First Name on Birth Certificate"/>	Middle Name on Birth Certificate * <input type="text" value="Type 'NA' if none"/>
Last Name on Birth Certificate * <input type="text" value="Last Name on Birth Certificate"/>	Date of Birth * <input type="text" value="mm/dd/yyyy"/>
Sex Recorded on Birth Certificate * ⓘ <input type="text"/>	
Country of Residence * <input type="text" value="United States"/>	State/Province/Region of Residence * <input type="text"/>
Country of Birth * <input type="text"/>	City/Municipality of Birth * <input type="text"/>

Consent to the Study

- Step 1: Click on “Yes, complete consent for this study.”



Would you like to consent to participate in the **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry?**



- Step 2: Scroll down and read through the consent form thoroughly. Once you finish reading, read through the statements thoroughly. If you are comfortable consenting to participate in the study, please read each statement and authorize your consent. After checking the boxes, click “Continue to Opt-Ins.”

any questions or want anything explained further, please contact the Registry Staff at: nordregistry@rare-diseases.org. It is our responsibility to answer your questions.

An Institutional Review Board (IRB) has reviewed this Registry to ensure that it meets ethical and regulatory standards for protecting your rights. An IRB is an independent group that reviews research proposals to make sure they properly protect participants. For questions about those protections and your rights as a Study Participant in this Registry or to discuss other study-related concerns or complaints with someone who is not part of this Registry team, please contact North Star Review Board at 877-673-8438 (toll free) or info@northstarreviewboard.org. You may want to contact the IRB if:

- You have questions, concerns, or complaints that are not being answered by the research team.
- You are not getting answers from the research team.
- You cannot reach the research team.
- You want to talk to someone else about the research.
- You have questions about your rights as a research subject.

Please do not sign this form unless you have had all your questions answered.

Authorization

The following statements are intended to ensure that you have had the time and opportunity to consider whether you want to participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient on behalf of the Study Participant, have had your questions answered, and agree to participate in the study as described. You will be asked to acknowledge that you have:

- Read the consent form and you and the Participant have no further questions about the Registry and your participation
- That you wish to provide personal data to the Registry for the purposes of the Study
- And that you wish to provide the Participant's pseudonymized data for future research
- That you have explained the study to the Participant to the extent they are able to understand

This is a web-based form and by answering "Yes" to all of the following statements, you are giving your consent to participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry on behalf of the study participant, just as if you had signed your name to a paper document. After signing, a copy of the consent form will be emailed to you. If you cannot comfortably answer "Yes" to these four statements or you have further questions, please do not check the consent boxes in the following section.

I have read (or someone has read to me) this Consent and Authorization Form to provide the Study Participant's personal and medical data to be shared for the purpose of research. All my questions about the Registry have been answered to my satisfaction and I understand the purpose of the Registry and the risks of participation.

I wish to provide the Study Participant's research data to the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry for the purposes described above under Study Aims.

I wish to provide the Study Participant's research data that has been pseudonymized to the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry for future research within recognized ethical standards for scientific research, as described under How We Use Your Data.

I have explained the study to the Study Participant to the extent they are able to understand, and the Study Participant has given their assent to participate in this study.

[Continue to Opt-Ins](#)

- Step 3: Step 3: Select your opt-ins, then click Save and Review.

Opt-Ins for The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry

Select Opt-Ins for this study

- Interest in hearing about other studies from [Aplastic Anemia and MDS International Foundation](#)
- Interest in hearing about relevant clinical trials
- Interest in donating specimens or DNA (biobanking) for future research
- Interest in learning more about [PNH](#) educational programs and resources from [Aplastic Anemia and MDS International Foundation](#)

[CANCEL](#) [SAVE](#)

- Step 4: Download a copy of your consent or click Close to continue.

View Consent/Assent
Review consent: The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry

Consent to Participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry and to Allow Data to be Shared for Future Research
Consent for a Person with a Legally Authorized Representative

Title: Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry
Principal Investigator: Alice Houk, Senior Director of Patient and Professional Services, Aplastic Anemia and MDS International Foundation (AA-MDSIF)
Email: nordregistry@aremdiseases.org
Phone: (800) 747-2820
Sponsor: Aplastic Anemia and MDS International Foundation (AA-MDSIF)
4330 East West Highway, Suite 230
Bethesda, Maryland 20814 USA

This document is intended to give you the information you need to make an informed and voluntary decision whether or not to provide the personal and medical information of the individual in your charge to the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry. As the guardian or legally authorized representative for the Study Participant, we encourage you to discuss the Registry with the Study Participant to the extent compatible with their understanding. Much of the information in this form is required by the regulations designed to protect research participants, and the headings and structure of the document were chosen to be sure that all the required information was included. While this information is meant to answer most of the questions we anticipate, it may not answer all of your questions. If you have questions about anything you read, or other questions about the Registry that are not answered here, please contact the Principal Investigator at nordregistry@aremdiseases.org or (800) 747-2820.

Key Information
Things you should know:

- The purpose of the study is to learn about the natural progression of Paroxysmal Nocturnal Hemoglobinuria (PNH), its symptoms and treatments. The information collected may become a resource for current and future research. Information about PNH is contributed by participants and caregivers who choose to volunteer for the Registry.
- If you choose to participate, you will be asked to complete online surveys at the start of the study, and at least once per year. Some of the surveys may be updated at any time. The surveys vary in length and do not need to be completed all at one time.
- Risks or discomforts: Some of the survey questions ask about the impact of PNH on your daily life, your economic status, mood, and other topics that you may find unpleasant or disquieting. There is a risk of breach of confidentiality, however there are protections in place to reduce this risk. While these are the risks we can foresee, it is possible that other risks may arise in the future. Please refer to the Risk and inconvenience section of this form for more information.
- There are no direct benefits for you to participate in the study. Indirect or future benefits could include the knowledge gained from the study. You may opt to receive a gift for providing your information to the Registry.

Taking part in this research project is voluntary. You do not have to participate, and you can stop at any time. Please take time to read this entire form and ask questions before deciding whether to take part in this research project.

Definitions
"Study Participant" refers to the person with Paroxysmal Nocturnal Hemoglobinuria (PNH). Registry information will be collected on patients who have PNH. "You" refers to the person reading this form and providing the information; in this case a family member or guardian who is legally responsible for the healthcare of the Study Participant. "We" refers to the organization, Aplastic Anemia and MDS International Foundation (AA-MDSIF).

Study Aims
The data collected in this Registry will be used by researchers to study Paroxysmal Nocturnal Hemoglobinuria (PNH) with the following goals:

1. To describe the people who have PNH and to better understand the variability and stages of PNH. To do this, we will ask you about the Study Participant's diagnosis, treatment, medical history, social and economic environment, and treatment outcomes.
2. To understand how PNH changes over a person's lifetime and to learn about clinical practice patterns and variations over the course of treatment.
3. To help to develop best practices, management guidelines and recommendations so that clinicians can know how to give the best care to improve the quality of life and outcomes of people with PNH.
4. To identify people with PNH who might be willing to take part in other research studies or clinical trials. You will be able to choose whether you want to hear about these other studies.

[Download PDF](#) [Close](#)

- Step 5: You will now have access to start taking surveys.

ENROLLED STUDIES

The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry
Consented
You have 11 pending surveys.

[Search Studies](#)

Surveys 11 pending

Getting Started
Not Started


[Take Survey](#)

- Please note, to complete some of the surveys, it may be helpful to gather any PNH treatment notes you have in advance.

View Responses and Reports

- Step 1: Once you have submitted a survey, you are able to view your responses to that survey as well as the graphs for any questions that are programmed to show graphs. Click “View Responses” to see your completed survey. Click “Reports” to see any available graphs.

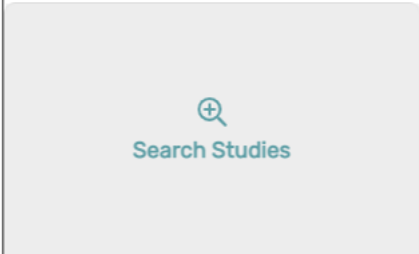
ENROLLED STUDIES



The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry ⓘ

Consented

● You have 10 pending surveys.



Surveys 🔔 10 pending

✓ Getting Started <i>Completed on 4-Apr-2023</i>	→	View Responses ⓘ
	→	Reports
● Demographics <i>Not Started</i>		Take Survey

