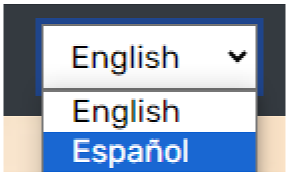
Participant User Guide

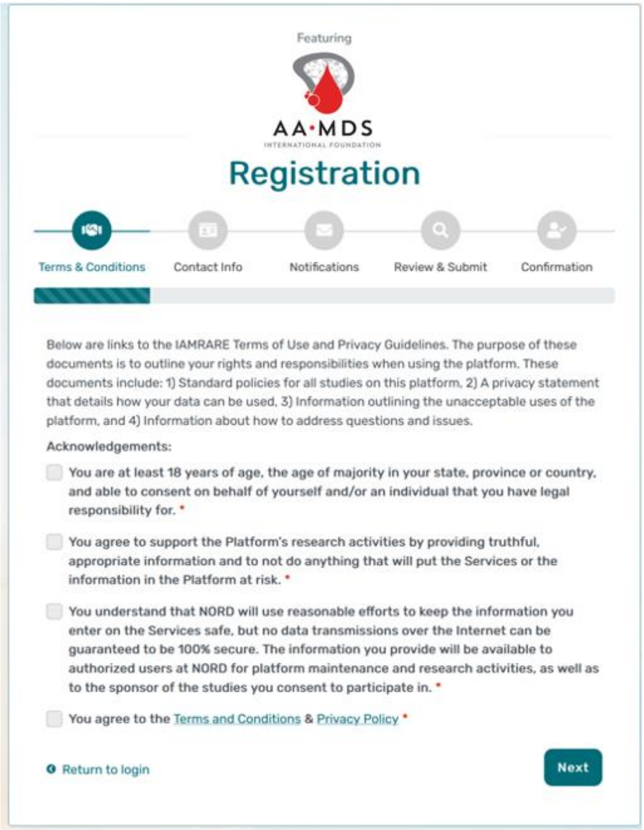
Language Selection:

- This study is available in English and Spanish.
- To change your platform language selection to Spanish: on the login/register page, select the dropdown that says “English”, choose your preferred language, and then continue with your registration or log in.
  - 

- Once logged in, you can also click “Settings” and select your Language Preference.

Register for an Account

- Step 1: Read the Terms and Conditions and Privacy Policy and attest to the statements provided. When you are finished with this page, click “Next”.



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.

Acknowledgements:

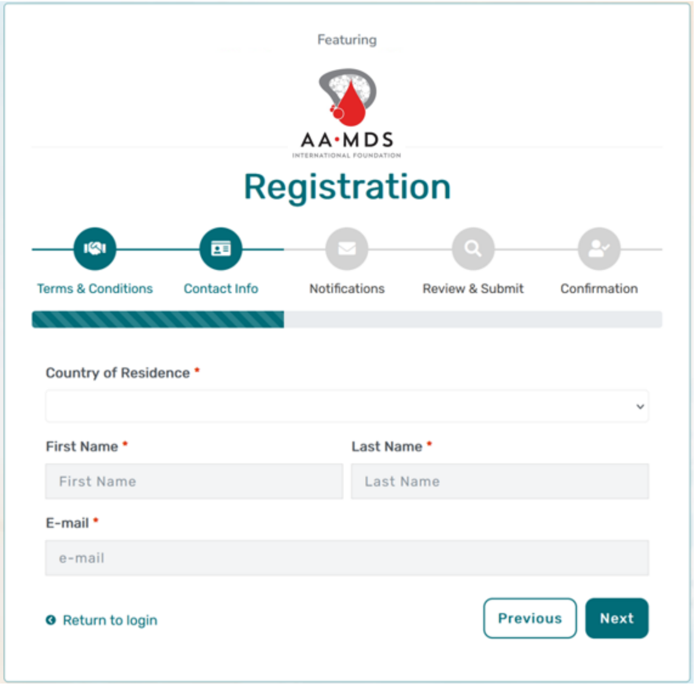
☐ You are at least 18 years of age, the age of majority in your state, province or country, and able to consent on behalf of yourself and/or an individual that you have legal responsibility for. *

☐ 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 *

- Step 2: Enter your personal information in the spaces provided. When you are finished with this page, click “Next”.



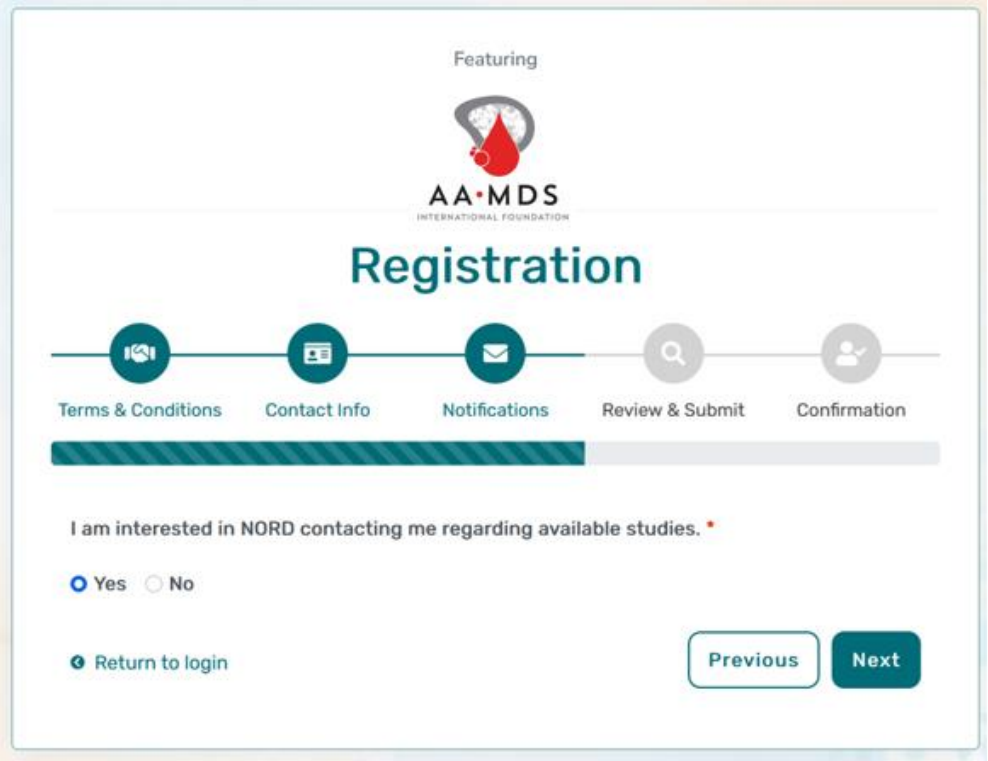
Country of Residence *

First Name * First Name

Last Name * Last Name

E-mail * e-mail

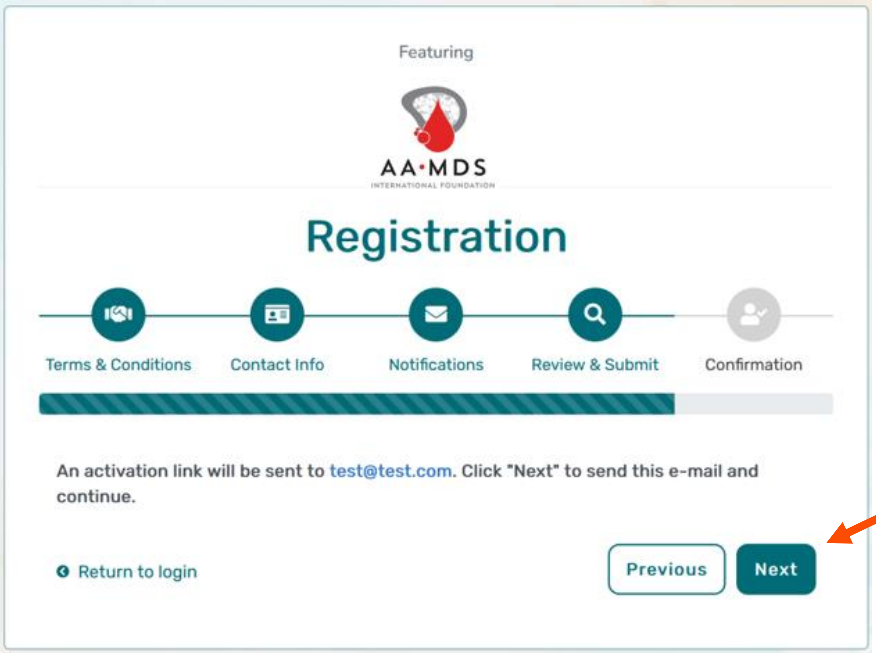
- Step 3: Select whether you are interested in being contacted by NORD regarding available studies. When you are finished with this page, click “Next”.



I am interested in NORD contacting me regarding available studies. *

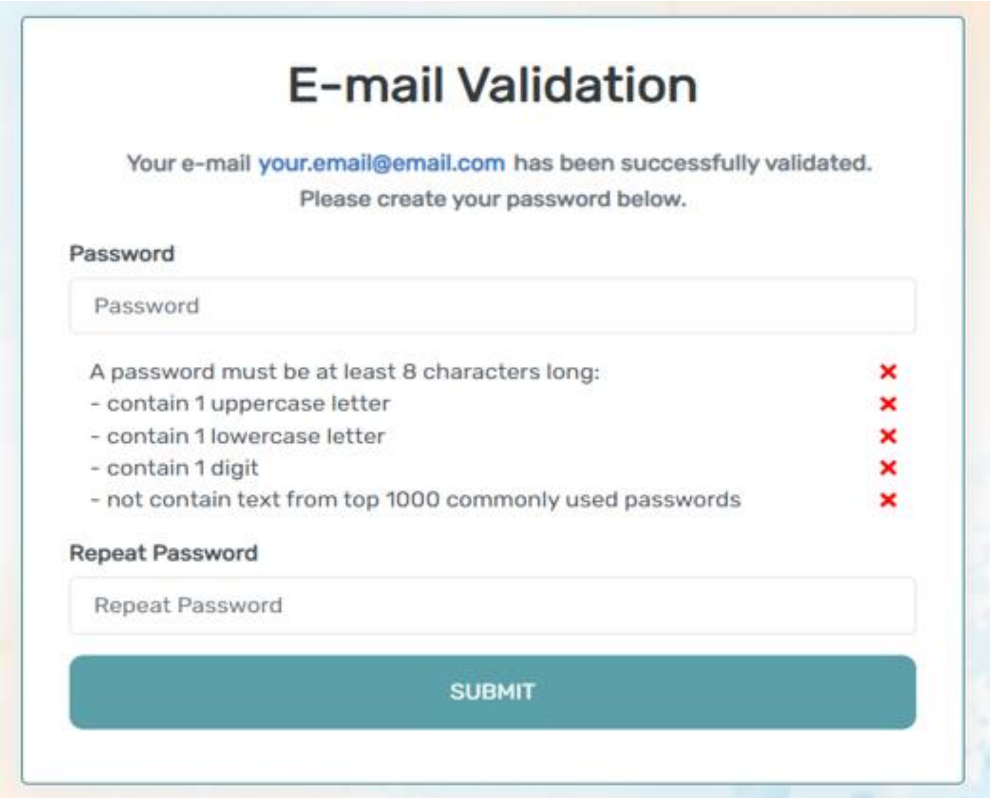
● Yes ○ No

- Step 4: Select “Next” so that an activation link is sent to your e-mail to complete registration.



An activation link will be sent to test@test.com. Click "Next" to send this e-mail and continue.

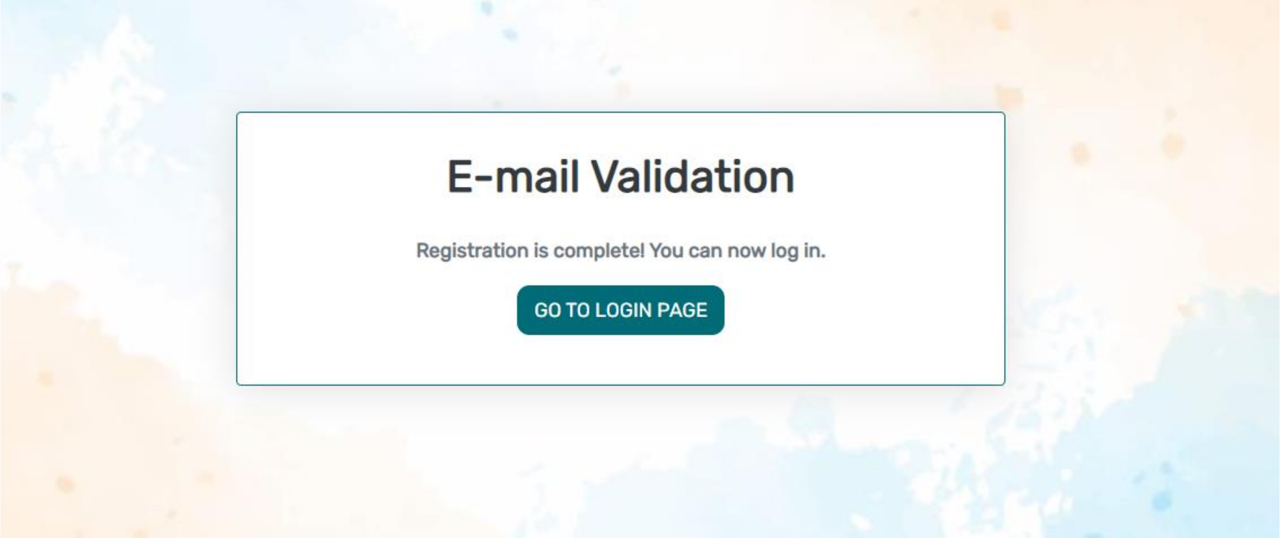
- Step 5: Click the link you are sent via e-mail. Please check your Spam folder if you do not see the e-mail. You will be taken to the following screen in a new tab within your browser. Set your password and click “Submit”.



Your e-mail your.email@email.com has been successfully validated.
Please create your password below.

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 ✗

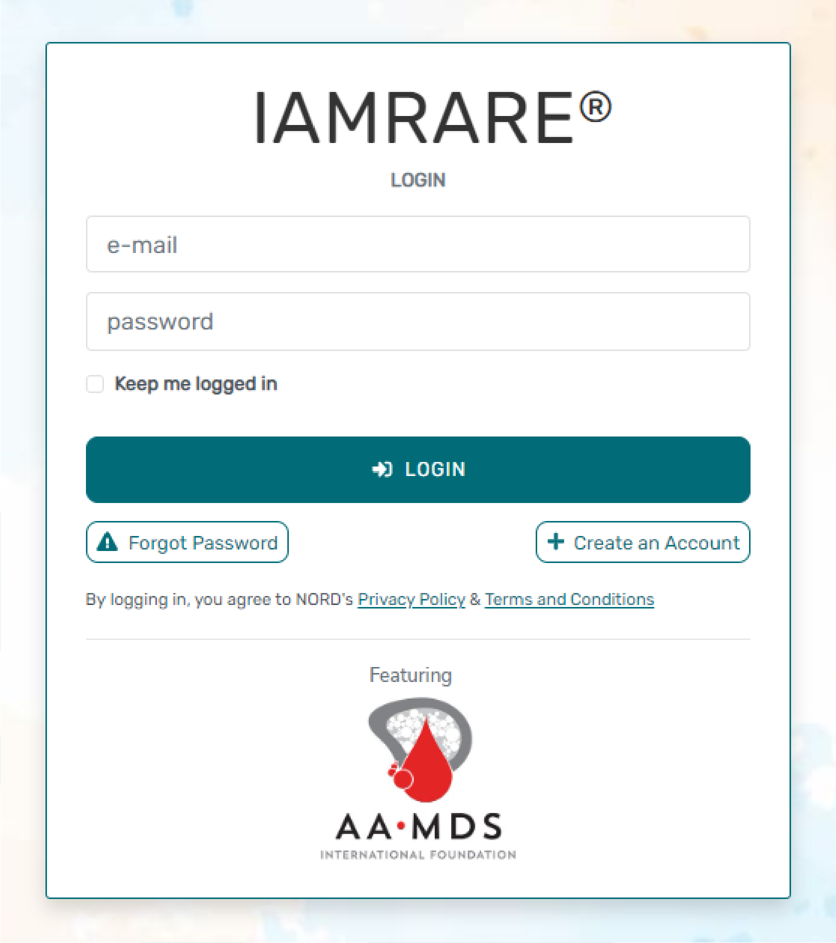
- Step 6: Your validation is now complete. Select “Go to Login Page”.



E-mail Validation

Registration is complete! You can now log in.

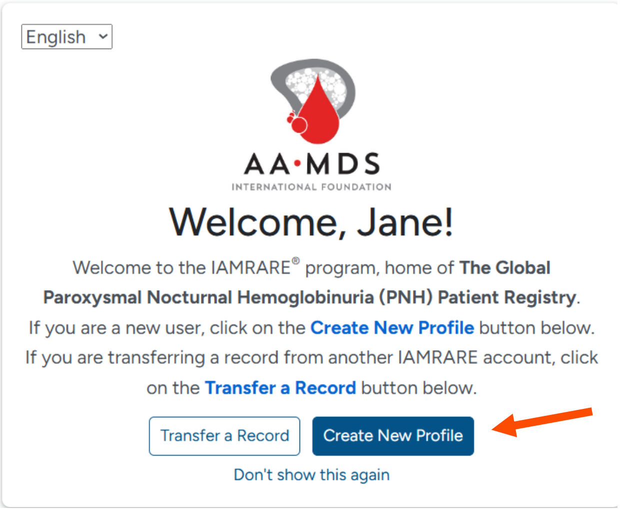
- Step 7: Log in using your new e-mail and password.



By logging in, you agree to NORD's Privacy Policy & Terms and Conditions

Create Profile

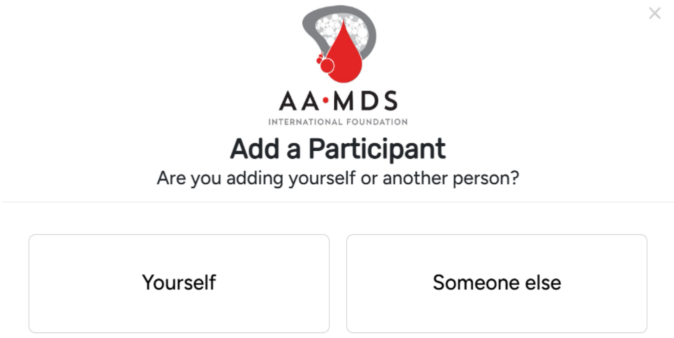
- Step 1: To start, click Create New Profile.



Welcome, Jane!

Welcome to the IAMRARE® program, home of **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**.
If you are a new user, click on the **Create New Profile** button below.
If you are transferring a record from another IAMRARE account, click on the **Transfer a Record** button below.

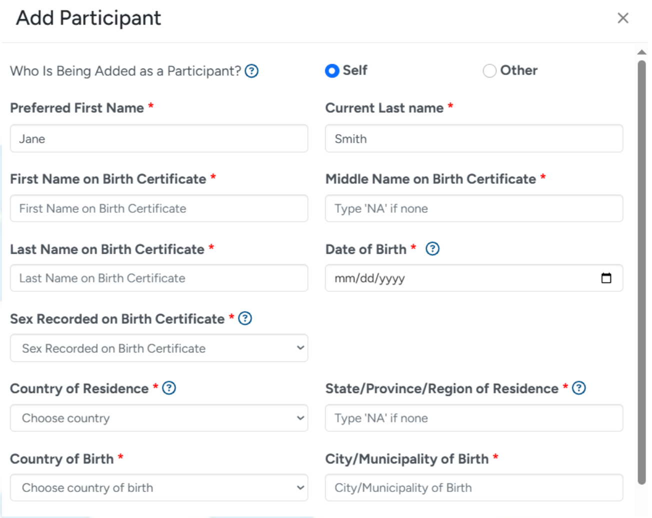
- Step 2: Select who you will be providing information about.



Add a Participant

Are you adding yourself or another person?

- Step 3: Fill out the Participant Profile



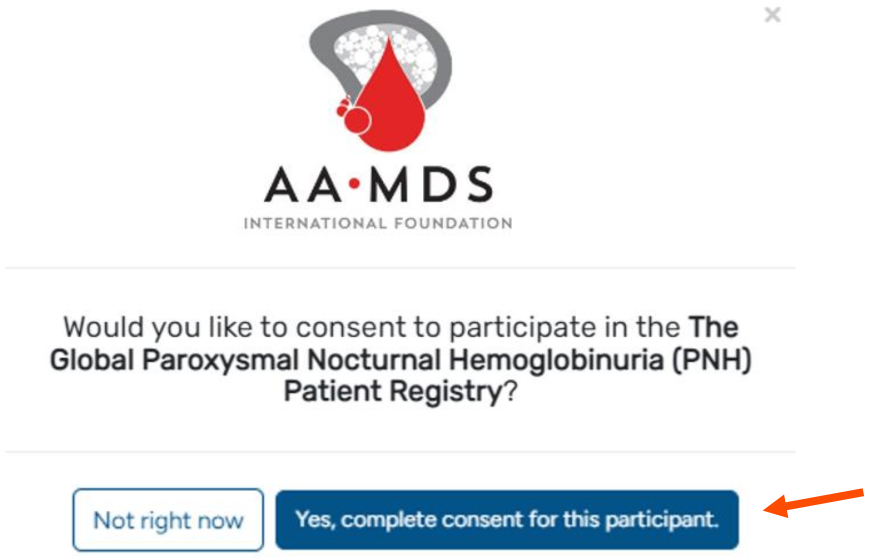
Add Participant

Who Is Being Added as a Participant? ● Self ○ Other

Preferred First Name * Jane	Current Last name * Smith
First Name on Birth Certificate * First Name on Birth Certificate	Middle Name on Birth Certificate * Type 'NA' if none
Last Name on Birth Certificate * Last Name on Birth Certificate	Date of Birth * mm/dd/yyyy
Sex Recorded on Birth Certificate * Sex Recorded on Birth Certificate	
Country of Residence * Choose country	State/Province/Region of Residence * Type 'NA' if none
Country of Birth * Choose country of birth	City/Municipality of Birth * City/Municipality of Birth

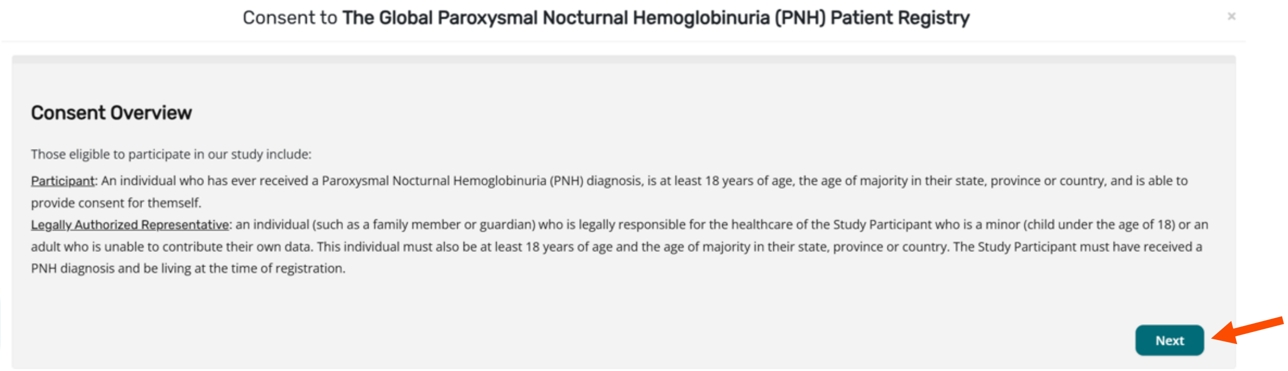
Consent to the Study

- 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 "Next."



Consent to **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**

Consent Overview

Those eligible to participate in our study include:

Participant: An individual who has ever received a Paroxysmal Nocturnal Hemoglobinuria (PNH) diagnosis, is at least 18 years of age, the age of majority in their state, province or country, and is able to provide consent for themself.

Legally Authorized Representative: an individual (such as a family member or guardian) who is legally responsible for the healthcare of the Study Participant who is a minor (child under the age of 18) or an adult who is unable to contribute their own data. This individual must also be at least 18 years of age and the age of majority in their state, province or country. The Study Participant must have received a PNH diagnosis and be living at the time of registration.

Consent to **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**

Adult Consent

Consent to Participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry and to Allow Your Data to be Shared for Future Research

Title: Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry

Principal Investigator: Alice Houk, Senior Director of Patient and Professional Programs, Aplastic Anemia and MDS International Foundation (AAMDSIF)

Phone: (800) 747-2820

Sponsor Email: pnhregistry@aamds.org

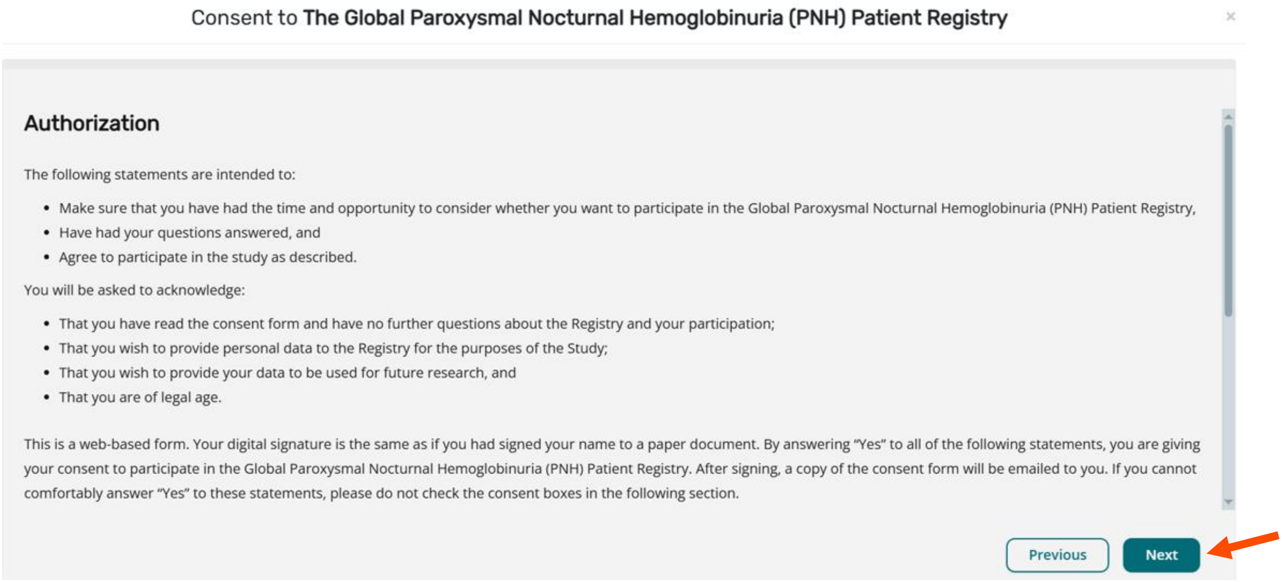
Registry Contact Email: iamrare-help@rarediseases.org

Sponsor: Aplastic Anemia and MDS International Foundation (AAMDSIF)

401 N. Washington Street, Suite 430
Rockville, Maryland 20850 USA

Key Information

You are invited to take part in a research study for individuals with Paroxysmal Nocturnal Hemoglobinuria (PNH). We hope that this form will help you decide whether to participate, but you can also call or e-mail the study staff at the contacts above if you have any other questions.



Consent to **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**

Authorization

The following statements are intended to:

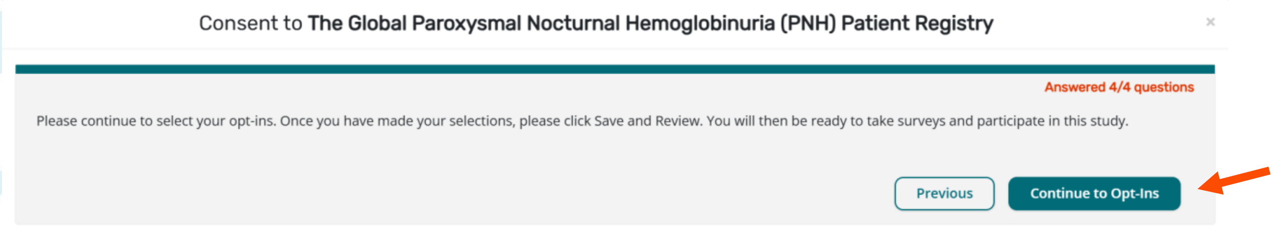
- Make sure that you have had the time and opportunity to consider whether you want to participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry,
- Have had your questions answered, and
- Agree to participate in the study as described.

You will be asked to acknowledge:

- That you have read the consent form and 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;
- That you wish to provide your data to be used for future research, and
- That you are of legal age.

This is a web-based form. Your digital signature is the same as if you had signed your name to a paper document. 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. After signing, a copy of the consent form will be emailed to you. If you cannot comfortably answer "Yes" to these statements, please do not check the consent boxes in the following section.

- Step 3: Once you click "Next" and reach the Thank You page, click "Continue to Opt-Ins".

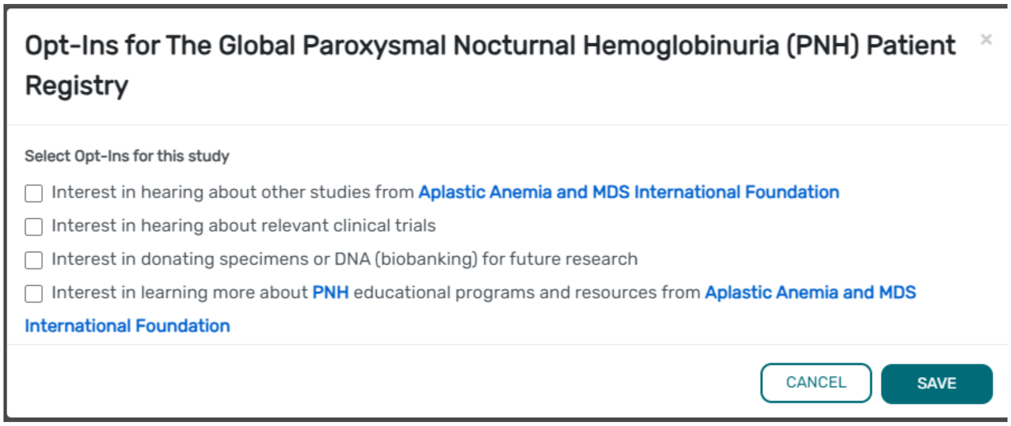


Consent to **The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry**

Answered 4/4 questions

Please continue to select your opt-ins. Once you have made your selections, please click Save and Review. You will then be ready to take surveys and participate in this study.

- Step 4: Select your opt-ins, then click “Save and Review.”

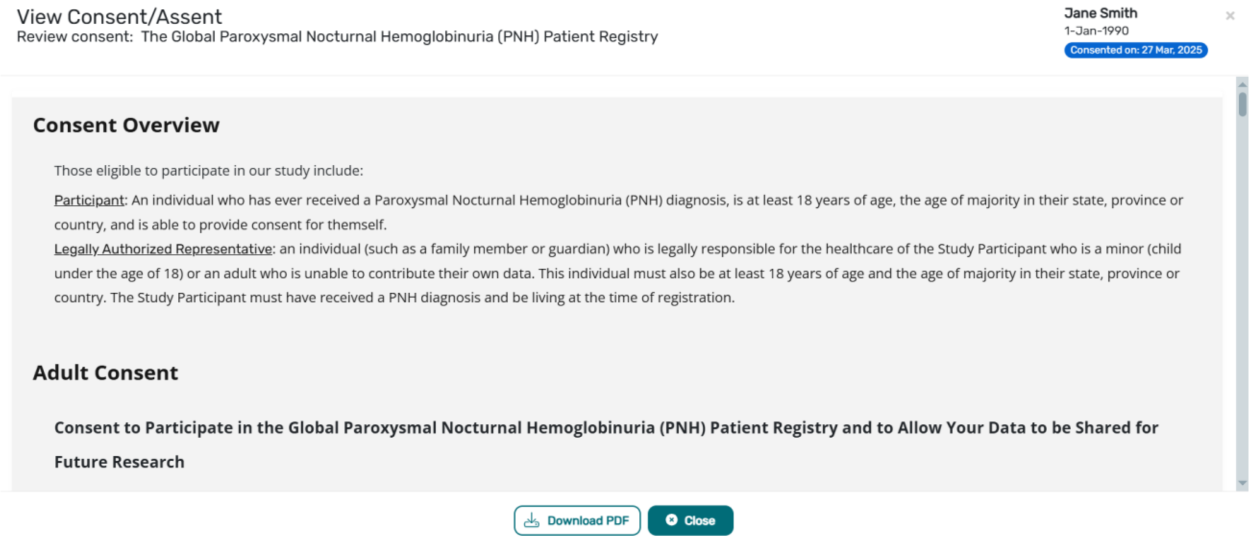


Opt-Ins for The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry

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

- Step 5: Download a copy of your consent or click “Close” to continue.



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

Jane Smith
1-Jan-1990
Consented on: 27 Mar, 2025

Consent Overview

Those eligible to participate in our study include:

Participant: An individual who has ever received a Paroxysmal Nocturnal Hemoglobinuria (PNH) diagnosis, is at least 18 years of age, the age of majority in their state, province or country, and is able to provide consent for themself.
Legally Authorized Representative: an individual (such as a family member or guardian) who is legally responsible for the healthcare of the Study Participant who is a minor (child under the age of 18) or an adult who is unable to contribute their own data. This individual must also be at least 18 years of age and the age of majority in their state, province or country. The Study Participant must have received a PNH diagnosis and be living at the time of registration.

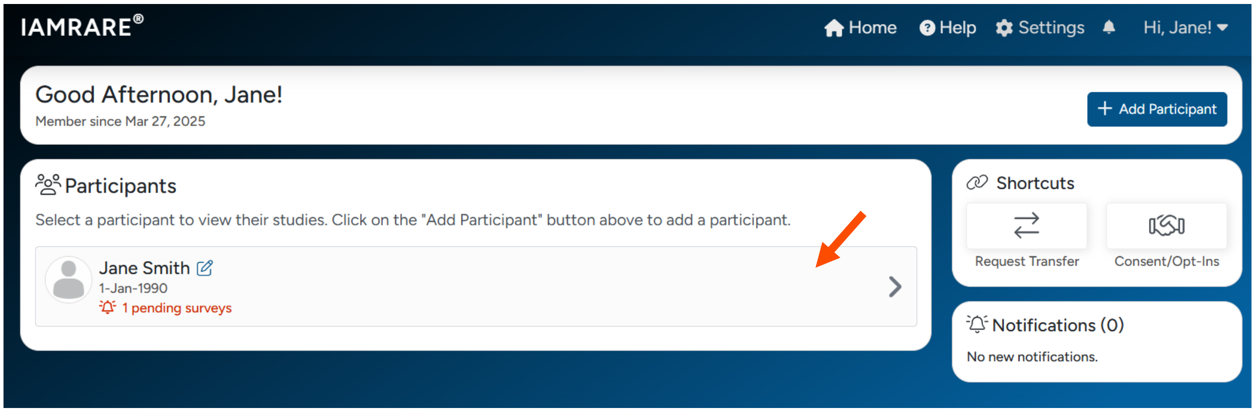
Adult Consent

Consent to Participate in the Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry and to Allow Your Data to be Shared for Future Research

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

Taking Surveys

- Step 1: Click on your Participant.



Good Afternoon, Jane!
Member since Mar 27, 2025

Participants

Select a participant to view their studies. Click on the "Add Participant" button above to add a participant.

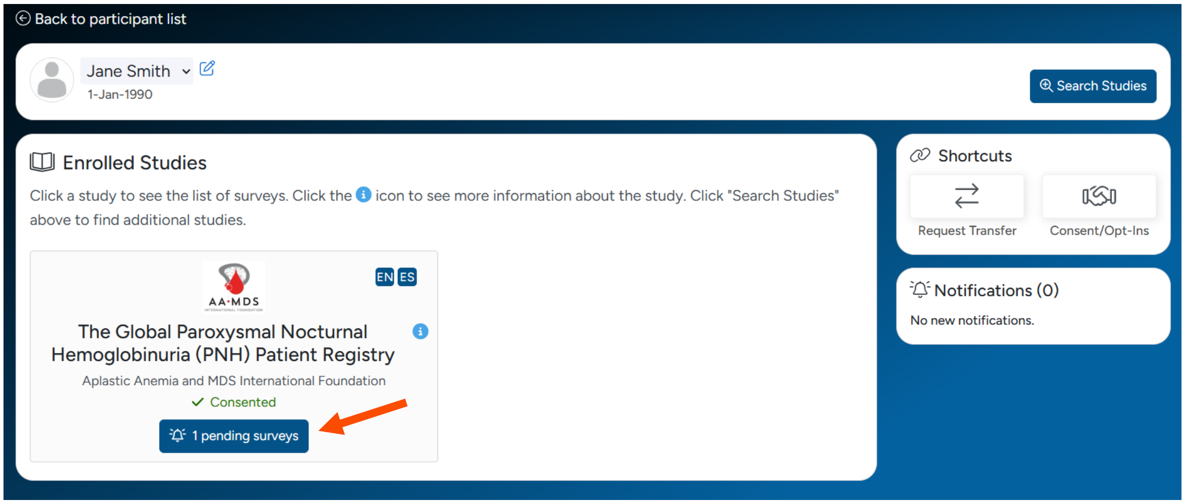
Jane Smith
1-Jan-1990
1 pending surveys

Shortcuts

Notifications (0)

No new notifications.

- Step 2: Click on the appropriate study.



Jane Smith
1-Jan-1990

Enrolled Studies

Click a study to see the list of surveys. Click the ℹ icon to see more information about the study. Click "Search Studies" above to find additional studies.

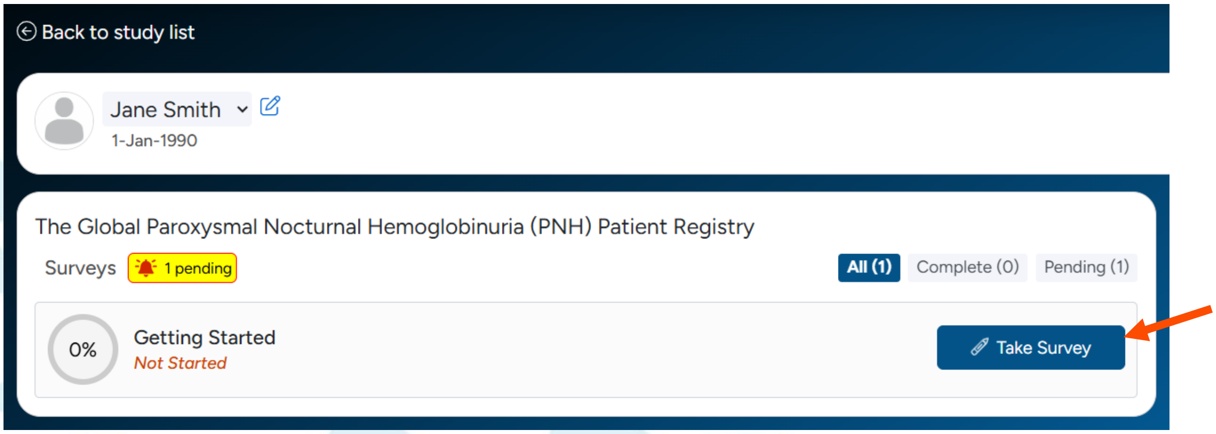
The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry
Aplastic Anemia and MDS International Foundation
✓ Consented

Shortcuts

Notifications (0)

No new notifications.

- Step 3: Click “Take Survey” for each available survey.



Jane Smith
1-Jan-1990

The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry

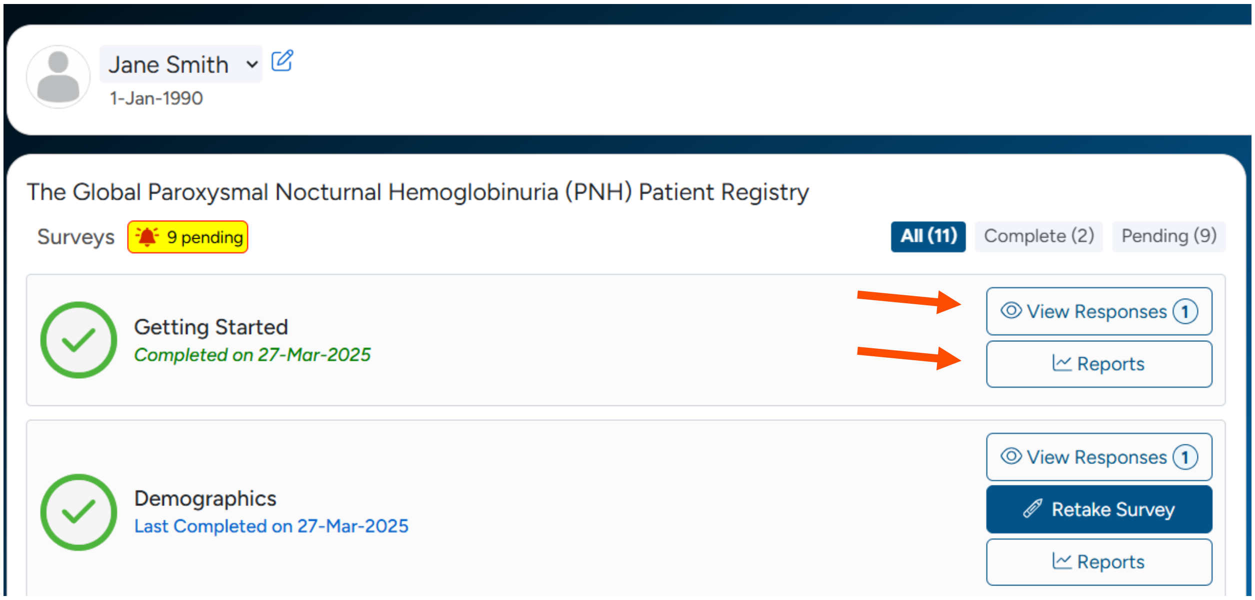
Surveys 1 pending

0% Getting Started
Not Started

- You can click the “Pin as Home Page” button if you want to be brought directly to this page the next time you log in.

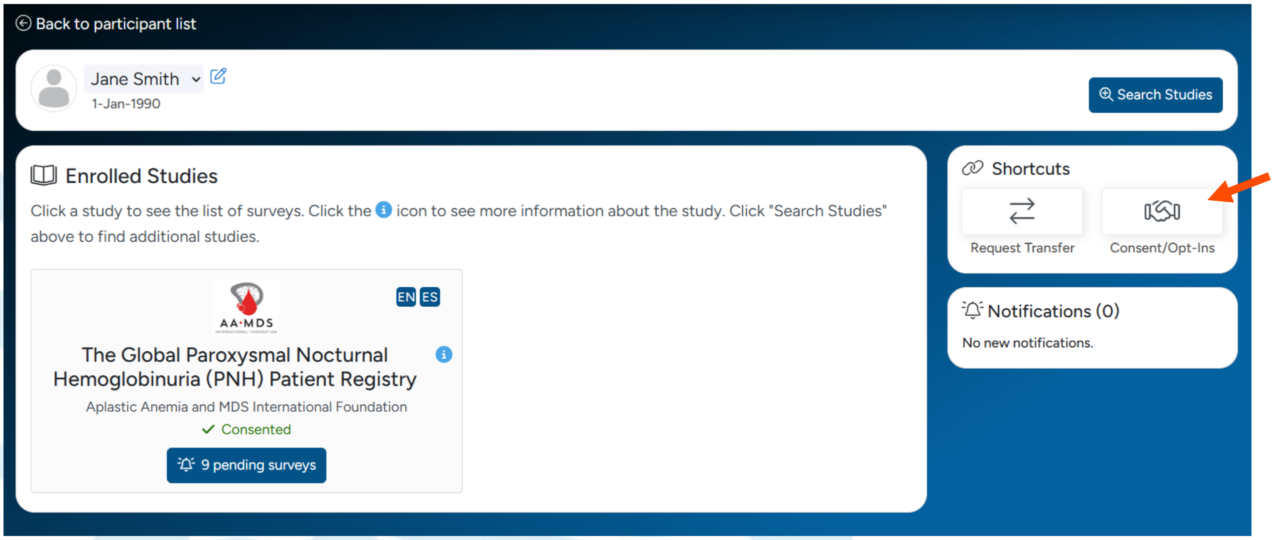
View Responses and Reports

- 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.



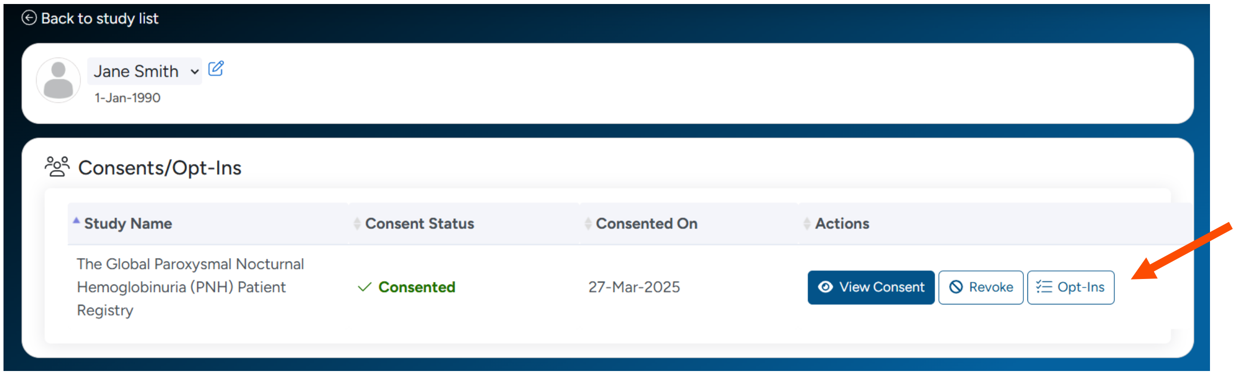
View Consent and Opt-Ins

- Step 1: Once you have consented to the study, you are able to view your consent at any time. Navigate to the Enrolled Studies page. Then, click “Consents/Opt-Ins” to see your consent and opt-ins.



Click a study to see the list of surveys. Click the ℹ icon to see more information about the study. Click "Search Studies" above to find additional studies.

- Step 2: You may revoke your consent at any time by clicking “Revoke”. You may also edit your Opt-Ins by clicking “Opt-Ins”.



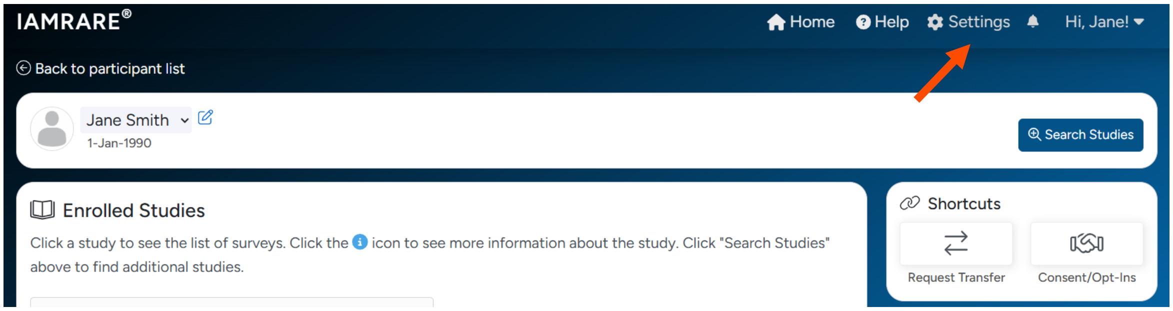
Jane Smith
1-Jan-1990

Consents/Opt-Ins

Study Name	Consent Status	Consented On	Actions
The Global Paroxysmal Nocturnal Hemoglobinuria (PNH) Patient Registry	✓ Consented	27-Mar-2025	View Consent Revoke Opt-Ins

Display Settings

- Step 1: You can change the platform display settings. First, click Settings.

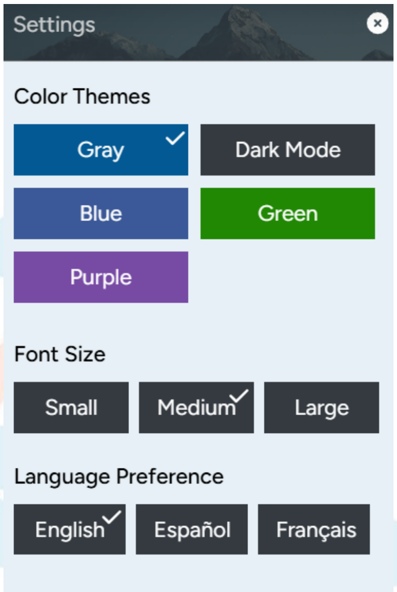


Jane Smith
1-Jan-1990

Enrolled Studies

Click a study to see the list of surveys. Click the ℹ icon to see more information about the study. Click "Search Studies" above to find additional studies.

- Step 2: Select a color theme, font size, or language preference.


Settings

Color Themes

Gray ✓
Dark Mode
Blue
Green
Purple

Font Size

Small
Medium ✓
Large

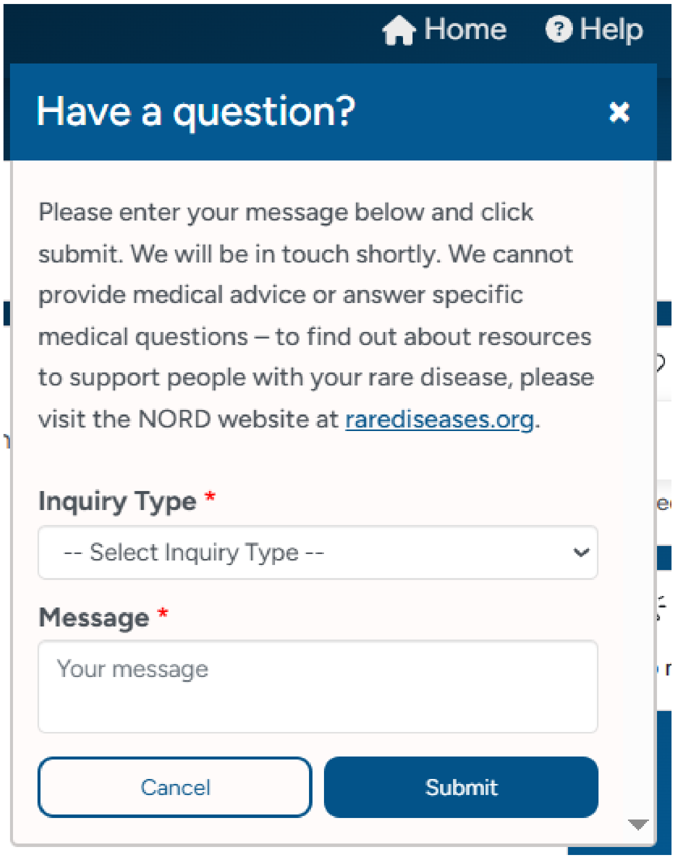
Language Preference

English ✓
Español
Français

- Step 3: Exit the Settings menu, and your selection will be saved.

Need Assistance?

- Step 1: If you need help while using the platform, click Help.
- Step 2: Select an Inquiry Type and type a message.



Please enter your message below and click submit. We will be in touch shortly. We cannot provide medical advice or answer specific medical questions – to find out about resources to support people with your rare disease, please visit the NORD website at rarediseases.org.

- Step 3: Click Submit.
- You may also contact the study staff directly by using the contact information shown on your dashboard or the study website, pnh.iamrare.org.