




LGMD2A/Calpainopathy Registry Participant User Guide


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”.


Featuring



COALITION TO
CURE
CALPAIN 3
OVERCOMING WEAKNESS
WITH STRENGTH


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.

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. *

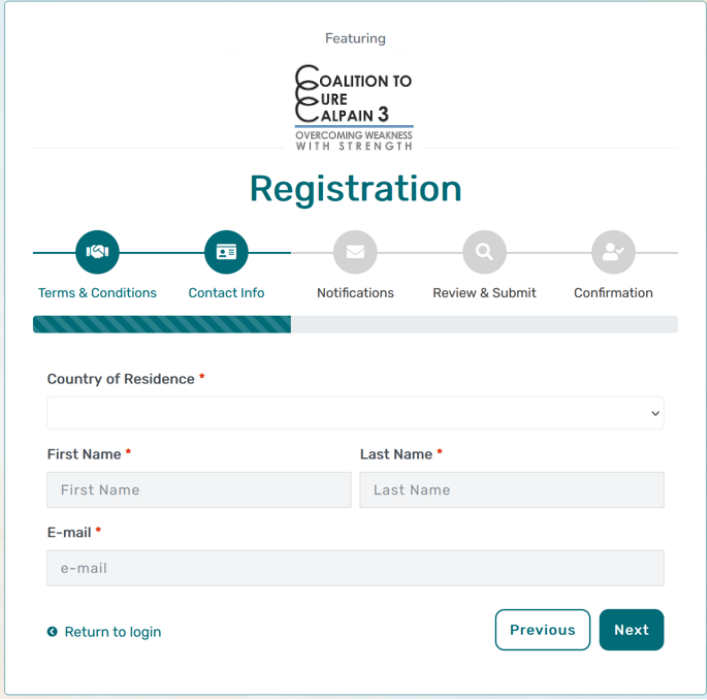
☐ You agree to support the Platform's research activities by providing truthful, appropriate information and to not do anything that will put the Services or the information in the Platform at risk. *

☐ 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](#). *

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

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



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OVERCOMING WEAKNESS WITH STRENGTH

Registration

Progress bar: Terms & Conditions (active), Contact Info, Notifications, Review & Submit, Confirmation

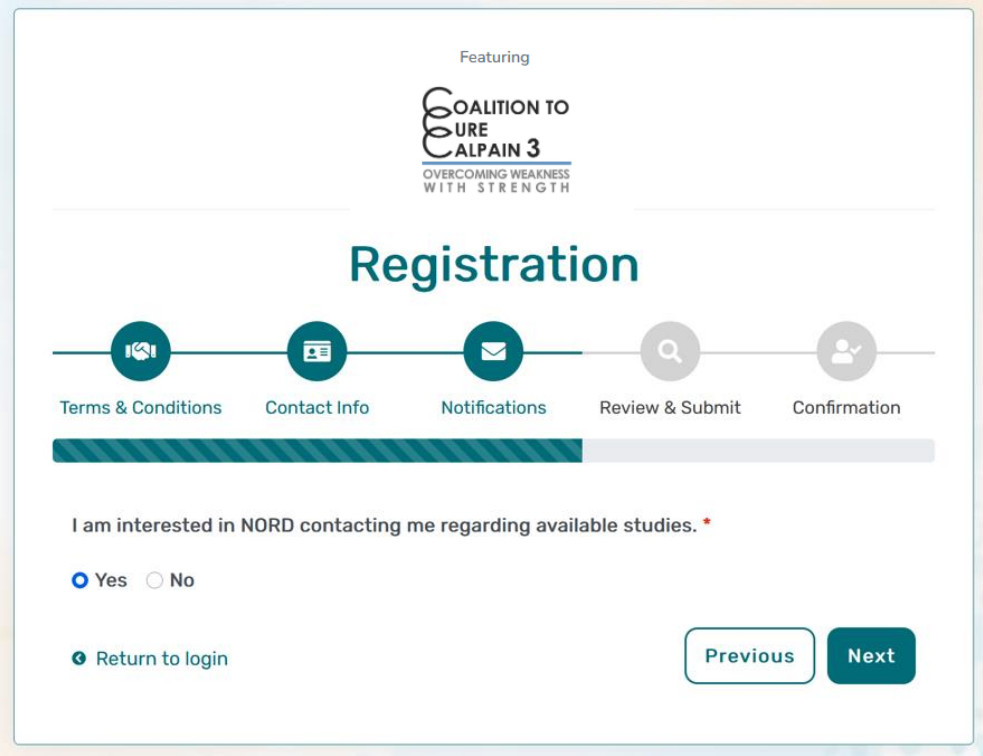
Country of Residence *

First Name * Last Name *

E-mail *

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

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



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COALITION TO CURE ALPAIN 3
OVERCOMING WEAKNESS WITH STRENGTH

Registration


Progress bar: Terms & Conditions, Contact Info, Notifications (active), Review & Submit, Confirmation

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

☒ Yes ☐ No

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

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



Featuring

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URE
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OVERCOMING WEAKNESS
WITH STRENGTH

Registration

Terms & Conditions Contact Info Notifications Review & Submit Confirmation

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

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

- 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”.

E-mail Validation

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

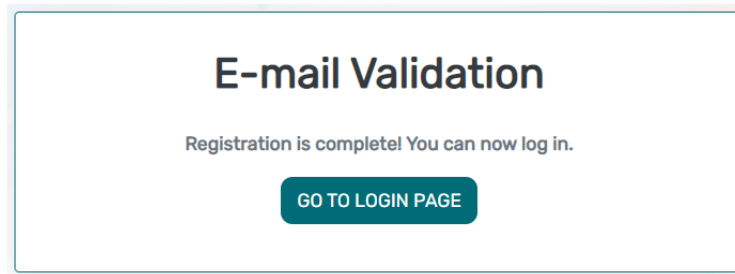
Password

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 ✗

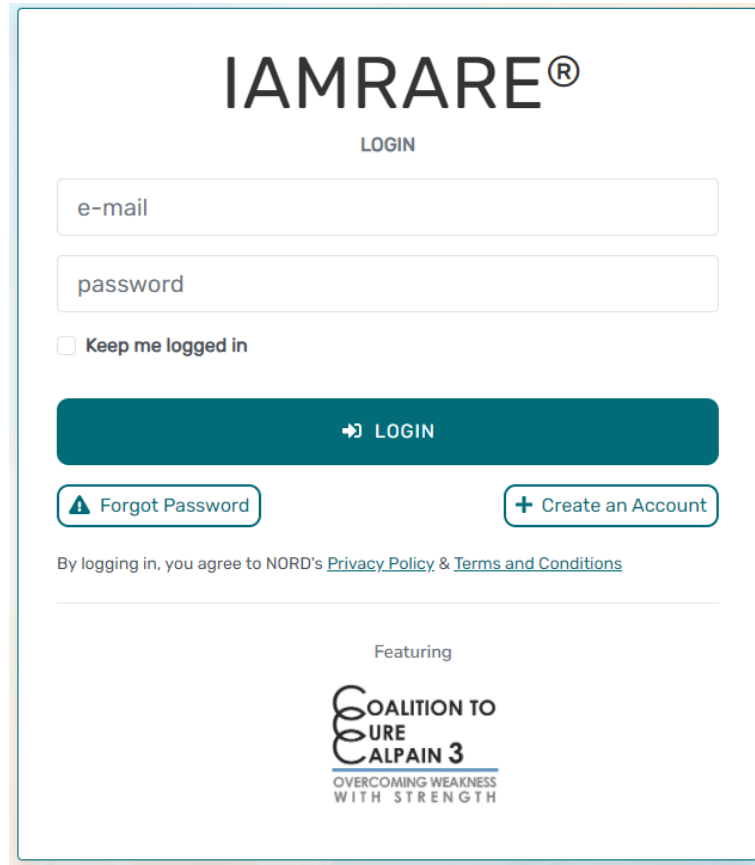
Repeat Password

[SUBMIT](#)

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




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

The login page for IAMRARE. At the top is the "IAMRARE®" logo. Below it is the word "LOGIN". There are two input fields: "e-mail" and "password". Below these is a checkbox labeled "Keep me logged in". A large teal button with a right arrow icon and the text "LOGIN" is centered. Below the button are two links: "Forgot Password" (with a warning icon) and "Create an Account" (with a plus icon). A line of text states: "By logging in, you agree to NORD's [Privacy Policy](#) & [Terms and Conditions](#)". At the bottom, it says "Featuring" followed by the "COALITION TO CURE ALPAIN 3" logo and the tagline "OVERCOMING WEAKNESS WITH STRENGTH".

Add a Participant

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

English ▾



**Coalition to
Cure
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Welcome, Jane!

Welcome to the IAMRARE® program, home of
LGMD2A/Calpainopathy 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.

Transfer a Record

Create New Profile

[Don't show this again](#)

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



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WITH STRENGTH

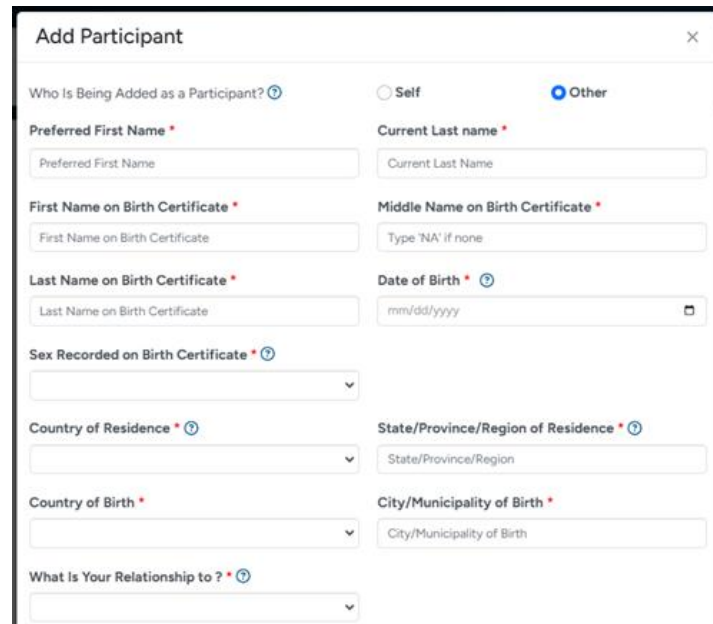
Add a Participant

Are you adding yourself or another person?

Yourself

Someone else

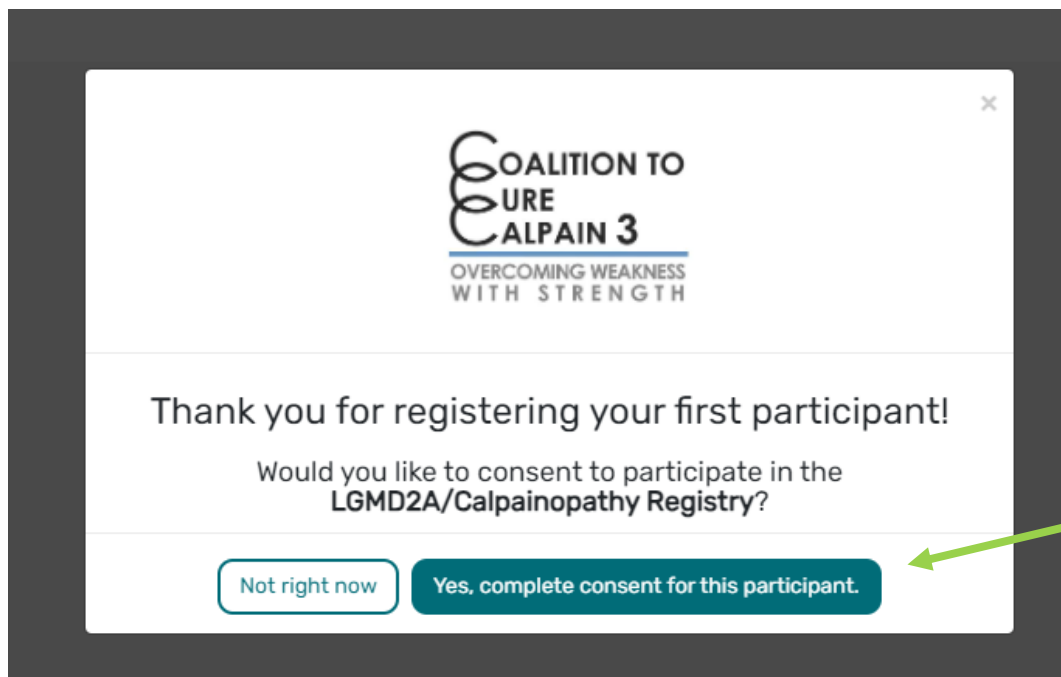
- Step 3: Fill out the Participant's information.



The 'Add Participant' form is a web-based interface for adding a new participant to a study. It features a title bar with a close button (X) and a question 'Who Is Being Added as a Participant?' with two radio button options: 'Self' and 'Other' (which is selected). The form is divided into several sections with input fields and dropdown menus. The first section contains 'Preferred First Name' and 'Current Last name'. The second section contains 'First Name on Birth Certificate' and 'Middle Name on Birth Certificate'. The third section contains 'Last Name on Birth Certificate' and 'Date of Birth' (with a calendar icon). The fourth section contains 'Sex Recorded on Birth Certificate' (a dropdown menu). The fifth section contains 'Country of Residence' and 'State/Province/Region of Residence' (both dropdown menus). The sixth section contains 'Country of Birth' and 'City/Municipality of Birth' (both dropdown menus). The final section contains 'What Is Your Relationship to ?' (a dropdown menu). All fields are marked with a red asterisk indicating they are required.

Consent to the Study

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



The consent dialog box is a white rectangular window with a close button (X) in the top right corner. It features the logo for 'COALITION TO CURE CALPAIN 3' with the tagline 'OVERCOMING WEAKNESS WITH STRENGTH'. Below the logo, the text reads 'Thank you for registering your first participant!' followed by the question 'Would you like to consent to participate in the LGMD2A/Calpainopathy Registry?'. At the bottom, there are two buttons: 'Not right now' and 'Yes, complete consent for this participant.'. A green arrow points to the 'Yes, complete consent for this participant.' button.

- Step 2: Scroll down and read through the consent form thoroughly. Once you finish each page, click the “Next” button. Once you reach the Authorization form, 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 LGMD2A/Calpainopathy Registry

Consent for a Person with a Legally Authorized Representative (Caregiver)

Title: LGMD2A/Calpainopathy Registry

Principal Investigator: Jennifer Levy, PhD, Scientific Director, Coalition to Cure Calpain 3

Phone: (203) 293-8864

Email: Registry@CureCalpain3.org

Key Information

You are invited to take part in a research study for individuals with Calpainopathy on behalf of the person in your care. We hope that this form will help you decide whether or not to participate, but you can also call or e-mail the study staff at the contacts above if you have any other questions.

Things you should know:

- The purpose of the study is to improve the understanding of the symptoms of Calpainopathy and how they change over time. The study will also allow participants, if they choose, to be contacted about research studies and clinical trials.
- If you choose to participate on behalf of the participant, you will be asked to complete web-based surveys when you enroll and at least once a year. This will take approximately 30 minutes to complete.
- There are no anticipated risks of physical harm to participating in the study. The surveys ask questions about the impact of Calpainopathy on the life of the participant, which you may find unpleasant.
- There are no direct benefits to your participation in the LGMD2A/Calpainopathy Registry. Learnings from the study may increase our understanding of the disease and availability of therapies for Calpainopathy patients.
- It is up to you whether to participate in this study, 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 on behalf of the person in your care. 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. Detailed information about your participation in this study follows.

Purpose of this Informed Consent Document

This document will give you the information for you to decide if you want to join this study on behalf of the participant or not. This consent document is structured to follow the framework provided by federal regulations. While we hope the information we provide will answer most of your questions, it may not answer them all. If you have any remaining questions, please contact the Principal Investigator at the phone or email listed above.

Definitions

For the purpose of enrolling in this registry, a legal adult is defined as a person who is at least 18 years of age, the age of majority in their state, province or country, and able to consent for themselves or on behalf of a Study Participant. On this form, “Study Participant” refers to the person with Calpainopathy (including LGMD2A, LGMDR1 Calpain 3-related, LMGD11, and LGMDD4 Calpain 3-related) who is not of legal age or is an adult who requires someone to act on their behalf. Registry information will be collected on individuals who have Calpainopathy. “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, Coalition to Cure Calpain 3.

Institutional Review Board (IRB) is an independent group that reviews research proposals to make sure they properly protect participants.

Next

Consent to LGMD2A/Calpainopathy Registry

Authorization

The following statements are intended to:

- Make sure that you have had the time and opportunity to consider whether you and the Study Participant want to participate in this 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 the Study Participant's participation;
- That you wish to provide the Study Participant's personal data to the registry for the purposes of the Study;
- That you allow for this data to be used for future research;
- That you have explained the study to the Study Participant to the extent they are able to understand; 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 LGMD2A/Calpainopathy Registry on behalf of the Study Participant. All four items must be checked “Yes” to participate in the LGMD2A/Calpainopathy 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.

☒ I have read 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 LGMD2A/Calpainopathy 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 LGMD2A/Calpainopathy Registry for the purposes described above under Study Aims.

☒ I wish to provide the Study Participant's research data to the LGMD2A/Calpainopathy Registry for future research within recognized ethical standards for scientific research, as described under How We Use Your Data.

Previous

Next

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

Jane Smith

Consent to LGMD2A/Calpainopathy Registry

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.

Previous Continue to Opt-Ins

- Step 4: Once you click “Continue to Opt-Ins” read through the opt-ins thoroughly. If you would like to receive information about the topic, check the box, and click “Save and Review”.

Opt-Ins for LGMD2A/Calpainopathy Registry

Select Opt-Ins for this study

- ☐ Interest in hearing about relevant clinical trials
- ☐ Interest in donating specimens or DNA (biobanking) for future research
- ☐ Interest in genetic testing
- ☐ If eligible, I have interest in receiving **Coalition to Cure Calpain 3** merchandise that would be sent via electronic or postal mail
- ☐ Interest in hearing about news and other studies from **Coalition to Cure Calpain 3**.
- ☐ Interest in learning about upcoming events such as webinars and conferences

Save and Review

- Step 5: Once you’ve reviewed your consent, click “Close”. You will then have access to start taking surveys.

Taking Surveys

- Step 1: Click on your Participant.

IAMRARE®

Home Help Settings Hi, Jane!

Good Afternoon, Jane!
Member since Mar 29, 2025

+ Add Participant

Participants

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

Jane Smith
5-May-2000
1 pending surveys

Shortcuts

Request Transfer Consent/Opt-Ins


Notifications (0)
No new notifications.

- Step 2: Click on the appropriate study.

← Back to participant list

Jane Smith 5-May-2000 [Search Studies](#)

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.

 English

LGMD2A/Calpainopathy Registry [i](#)

Coalition to Cure Calpain 3

✓ Consented

🔔 1 pending surveys

Shortcuts

[Request Transfer](#) [Consent/Opt-Ins](#)


Notifications (0)
No new notifications.

- Step 3: Click "Take Survey" for an available survey.

← Back to study list

Jane Smith 5-May-2000

LGMD2A/Calpainopathy Registry

Surveys  1 pending **All (1)** Complete (0) Pending (1)

0% **Getting Started**
Not Started

[Take Survey](#)

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.

The screenshot shows the user profile for Jane Smith (5-May-2000) at the top. Below, the study title 'LGMD2A/Calpainopathy Registry' is displayed. A 'Surveys' section indicates '6 pending' with a clock icon. To the right, filters show 'All (7)', 'Complete (1)', and 'Pending (6)'. A 'Getting Started' card shows a green checkmark and 'Completed on 28-Mar-2025'. On the right side, two buttons are visible: 'View Responses (1)' and 'Reports', both highlighted with green arrows.

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

The screenshot shows the 'Enrolled Studies' page for Jane Smith. A green arrow points to the user profile at the top left. The main content area has a heading 'Enrolled Studies' and a description: 'Click a study to see the list of surveys. Click the ⓘ icon to see more information about the study. Click "Search Studies" above to find additional studies.' On the right, there is a 'Shortcuts' section with two buttons: 'Request Transfer' and 'Consent/Opt-Ins'. A green arrow points to the 'Search Studies' button at the top right.

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

The screenshot shows the 'Consents/Opt-Ins' table for Jane Smith. The table has four columns: 'Study Name', 'Consent Status', 'Consented On', and 'Actions'. There is one row for 'LGMD2A/Calpainopathy Registry' with a status of 'Consented' and a date of '28-Mar-2025'. The 'Actions' column contains three buttons: 'View Consent', 'Revoke', and 'Opt-Ins'. Green arrows point to the 'Revoke' and 'Opt-Ins' buttons.

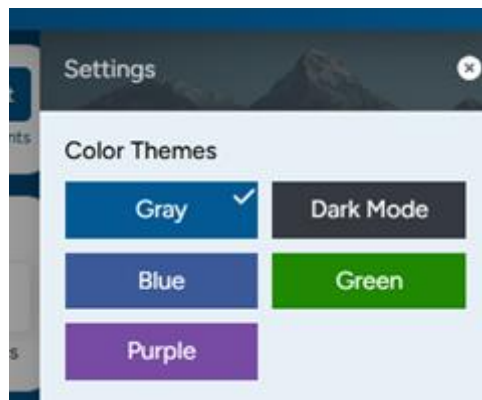
Study Name	Consent Status	Consented On	Actions
LGMD2A/Calpainopathy Registry	✓ Consented	28-Mar-2025	View Consent Revoke Opt-Ins

Dark Mode Settings

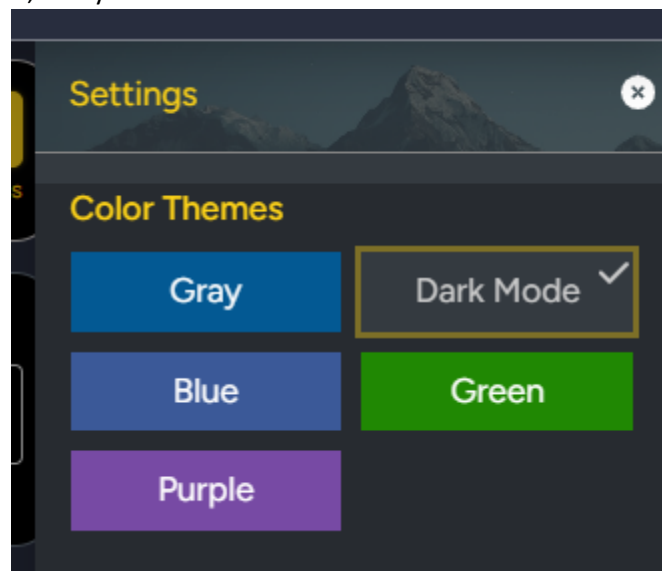
- Step 1: You can view the platform in Dark Mode. First, click Settings.



- Step 2: Select Dark Mode.



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

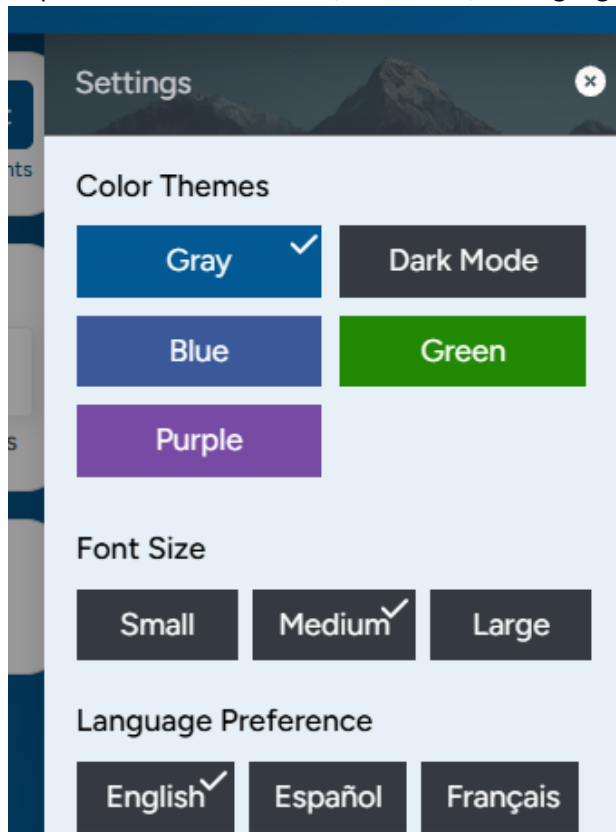


Display Settings

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



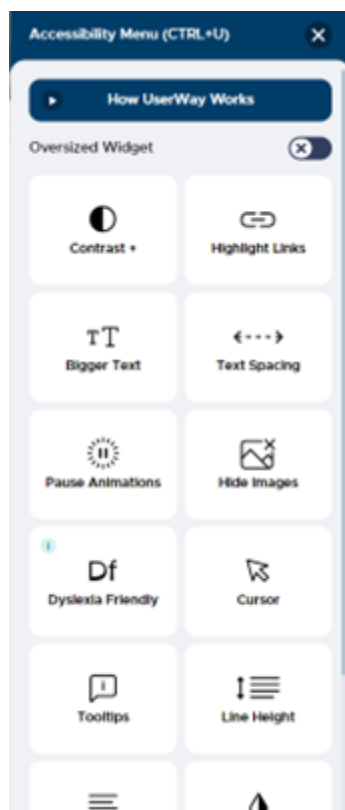
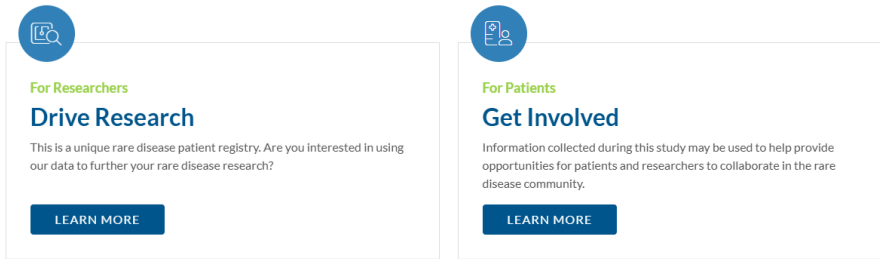
- Step 2: Select a color theme, a font size, or language preference.



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

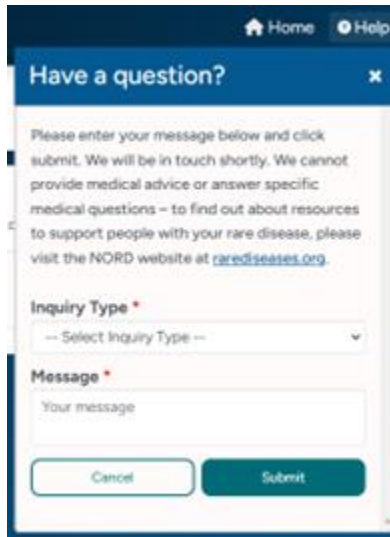
Microsite Visibility

- Step 1: You can change how you view the microsite (lgmd2a.iamrare.org) using an Accessibility menu. Click the icon of a person at the bottom of the screen. You are able to change the settings such as the contrast, text sizing, and text spacing.



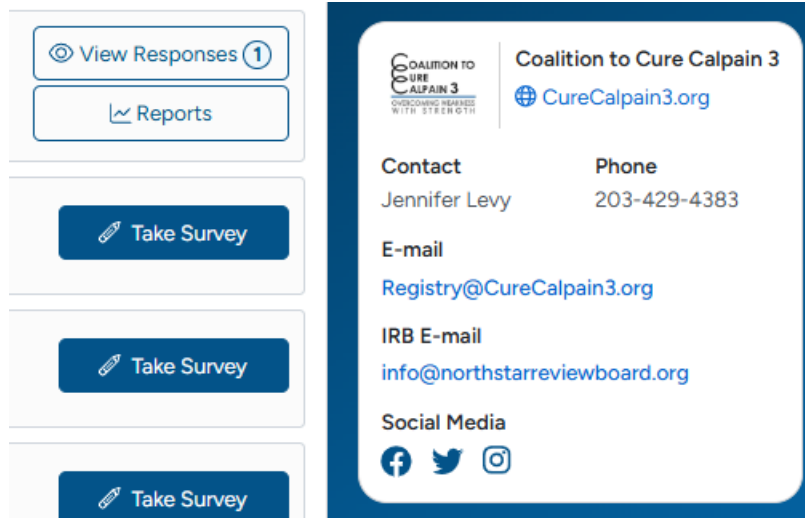
Need Assistance?

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



The screenshot shows a mobile app interface with a dark blue header containing 'Home' and 'Help' icons. Below the header is a white modal box titled 'Have a question?' with a close button (X). The modal contains the following text: '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.' Below this text is a dropdown menu labeled 'Inquiry Type *' with the placeholder text '-- Select Inquiry Type --'. Underneath the dropdown is a text input field labeled 'Message *' with the placeholder text 'Your message'. At the bottom of the modal are two buttons: 'Cancel' and 'Submit'.

- Step 3: Click Submit.
- You may also contact the study sponsor directly by using the contact information shown on your dashboard or the study website.



The screenshot shows a dashboard on the left and a contact information card on the right. The dashboard has four buttons: 'View Responses 1', 'Reports', and two 'Take Survey' buttons. The contact information card is for the 'Coalition to Cure Calpain 3' and includes the following details:

Contact	Phone
Jennifer Levy	203-429-4383

E-mail
Registry@CureCalpain3.org

IRB E-mail
info@northstarreviewboard.org

Social Media