CHANNELING HOPE FOUNDATION

Data Collection Launch

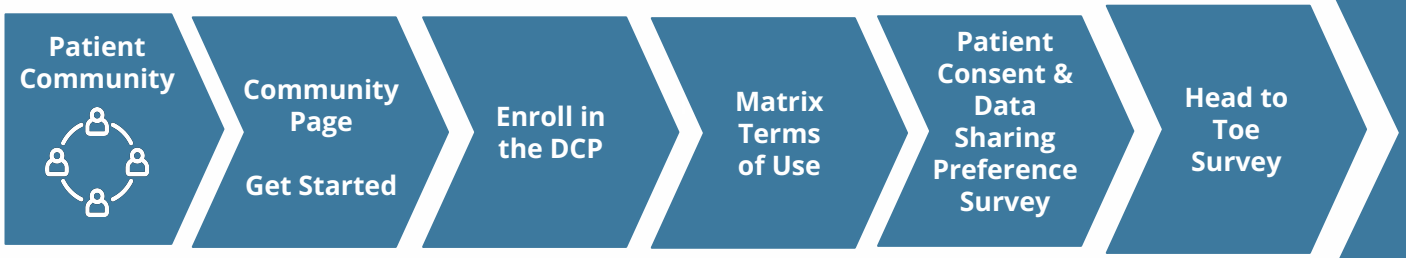
November 20, 2024

Global Genes® Allies in Rare Disease + RARE X A Research Program of Global Genes

What Is RARE-X?

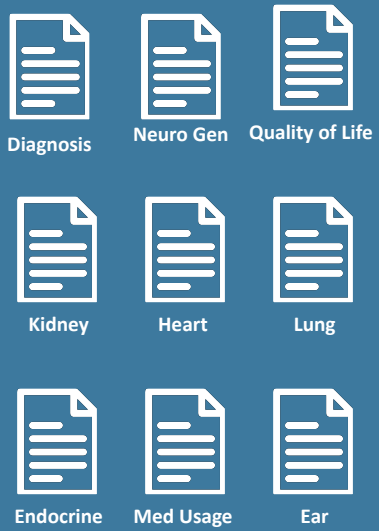
- RARE-X is a program of Global Genes created to accelerate rare disease research and treatments by removing barriers for data collection and sharing
- RARE-X is a platform to collect, connect, and share data

Patient Journey in the Data Collection Platform (DCP)



Patient Dashboard

Potential Survey Topics (Domains)



AQ CONTACT US GETTING STARTED LOGIN

Powered by: RAR
A Research Program of Global G

Home > NALCN

NALCN Channel-related disorders (including IHRPF)- Data Collection Program

NALCN Channel-related disorders (including IHRPF) patients, families, and communities are excited to participate in data collection to expand and improve medical research. By coming to this site, you can begin the first step in making your patient information available to researchers. By generating the most comprehensive NALCN Channel-related disorders (including IHRPF) Data Collection Program, we can accelerate research and the development of new drugs, devices, or other therapies. Only you hold the key to unlock future discoveries.

Start Your Journey

GET STARTED

Already Enrolled?

LOGIN

CHANNELING HOPE
FOUNDATION

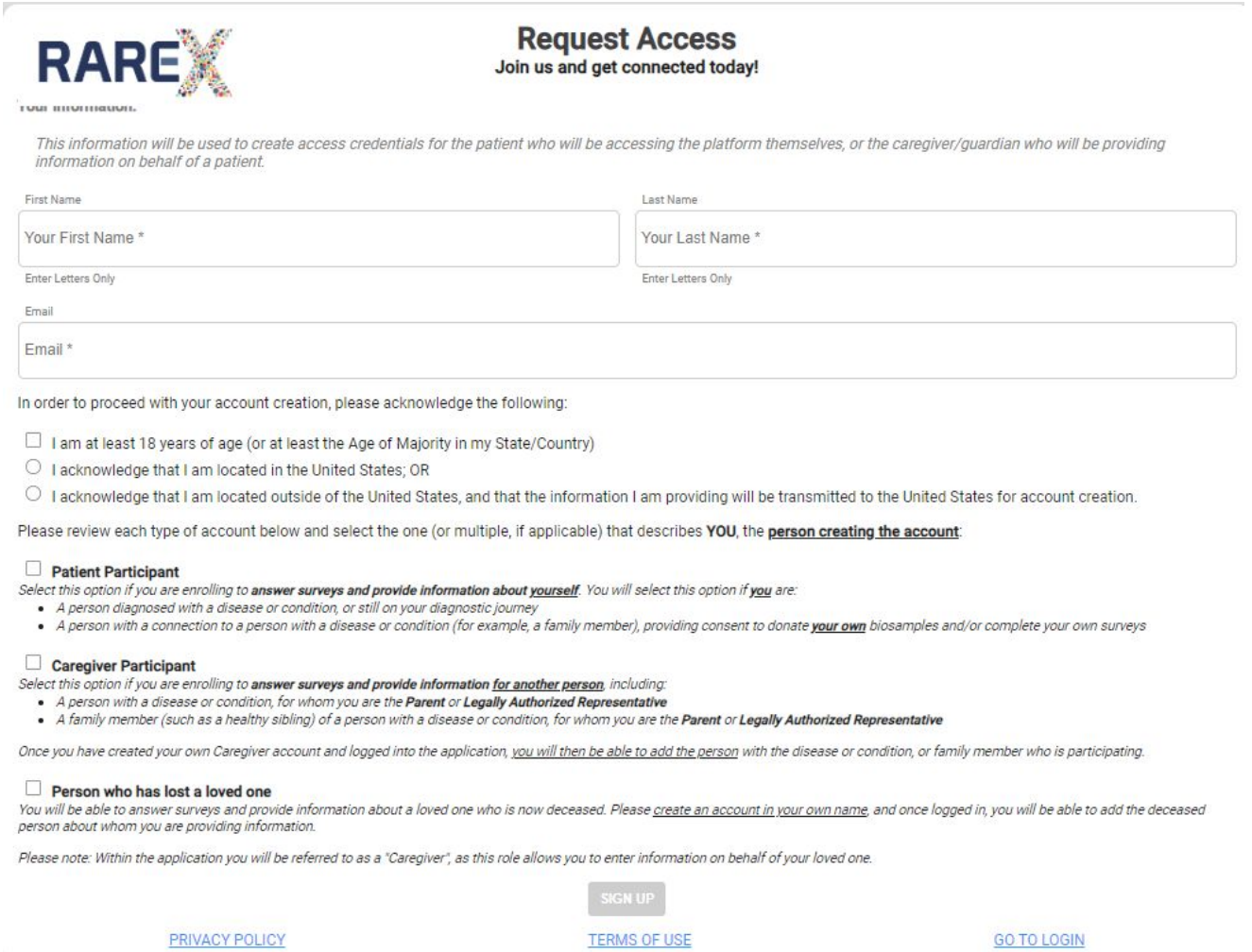
https://rare-x.org/nalcn/

What Do You Need To Get Started?

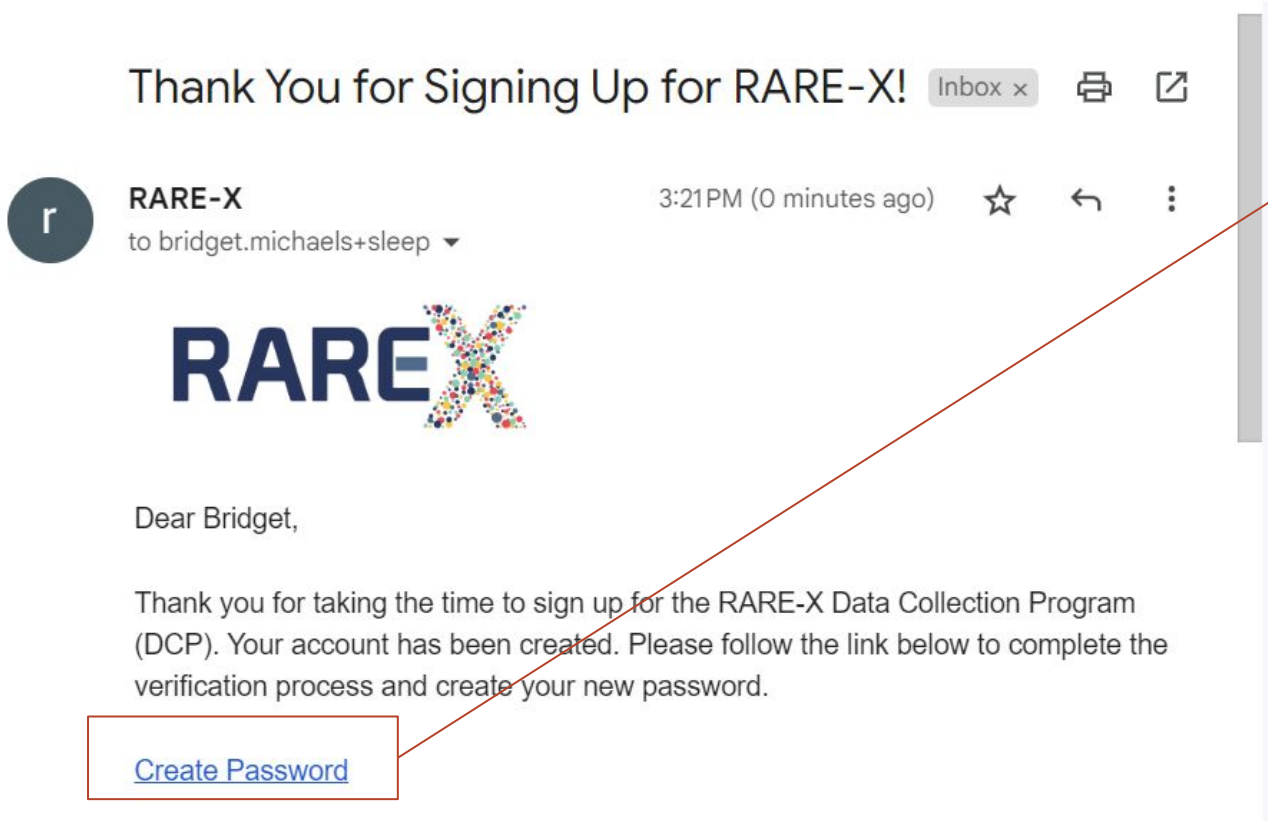
- Email address and Create a password
- An approved Browser
  - Google Chrome, or
  - Apple Safari version 14 or higher
  - Microsoft Edge
- Do not use an unapproved Browser
  - Internet Explorer
  - Mozilla
  - Firefox
- What you do NOT need to get started?
  - A Lot of Time
  - To Finish It All At Once

First-time Login Page

(Pre-Qualifications Page)



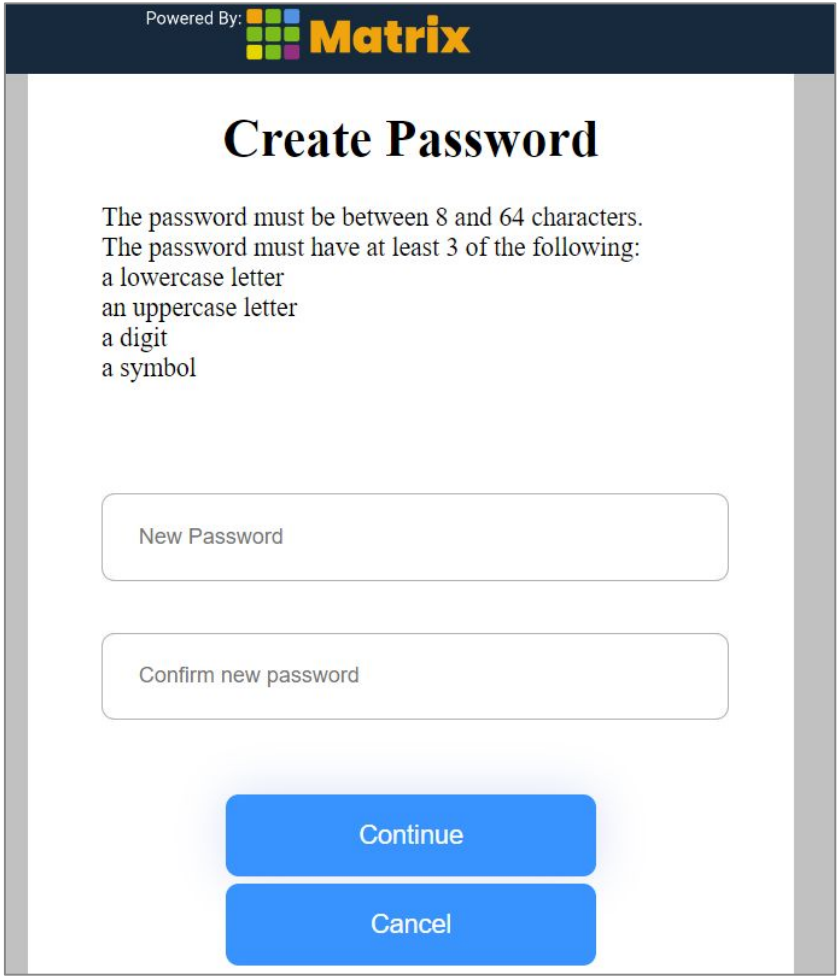
Receive Account Creation Email



Create Password

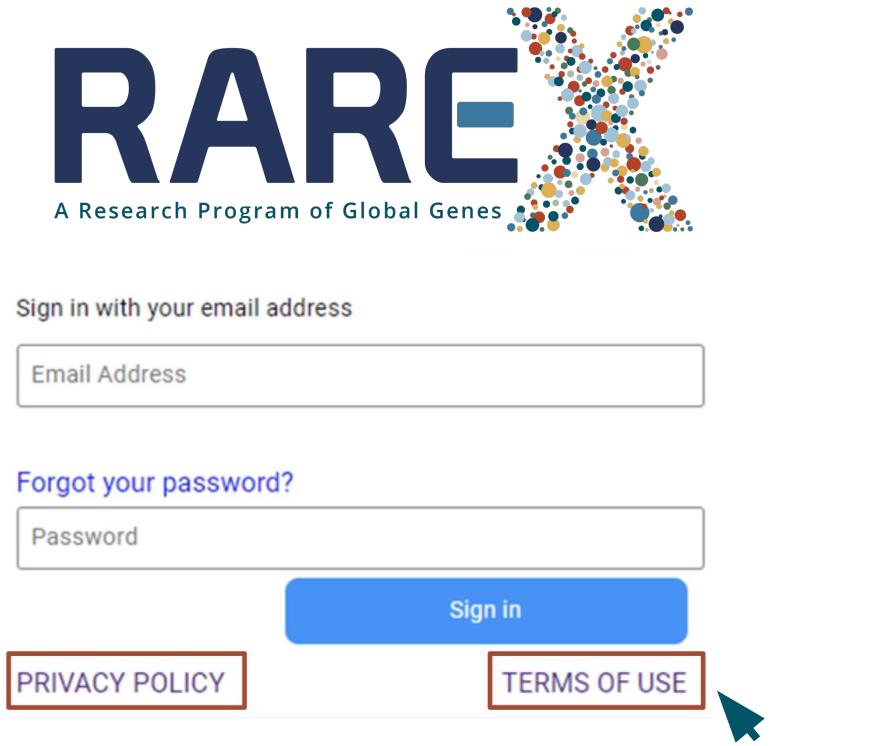
If you do not receive a response within a few minutes, check your spam/ junk folder

Password Creation



Privacy Policy & Terms of Use for RARE-X

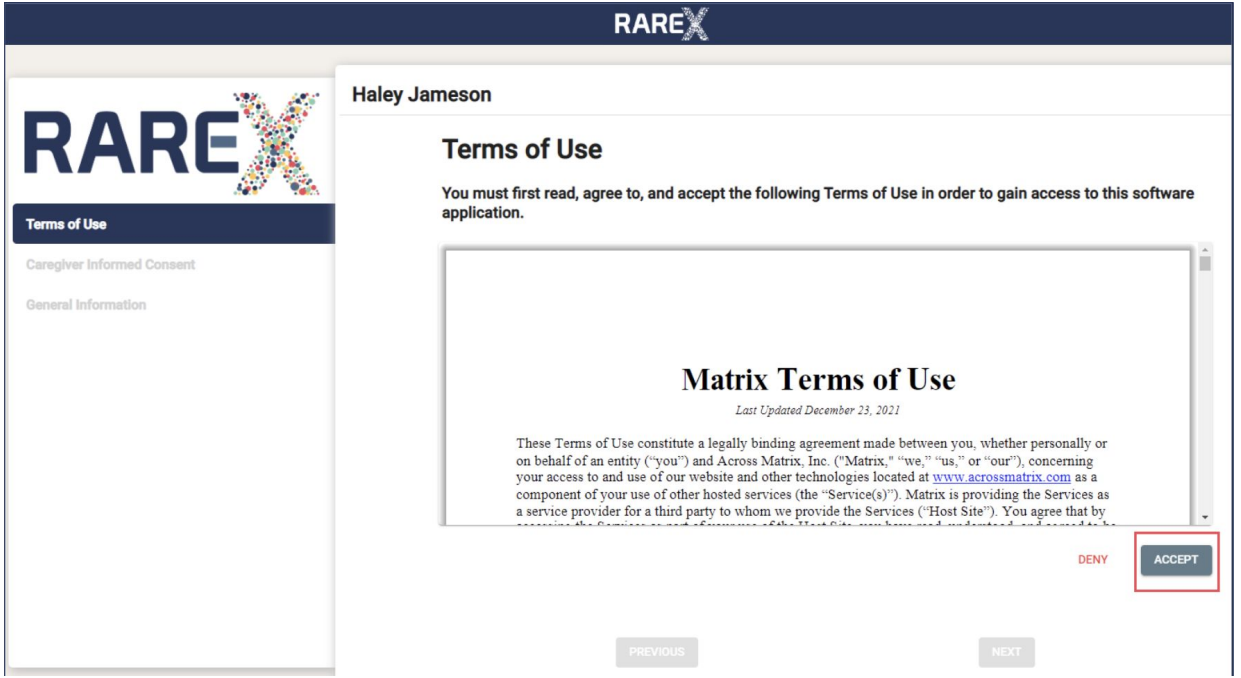
Login to the DCP



You can access these documents anytime by clicking on them

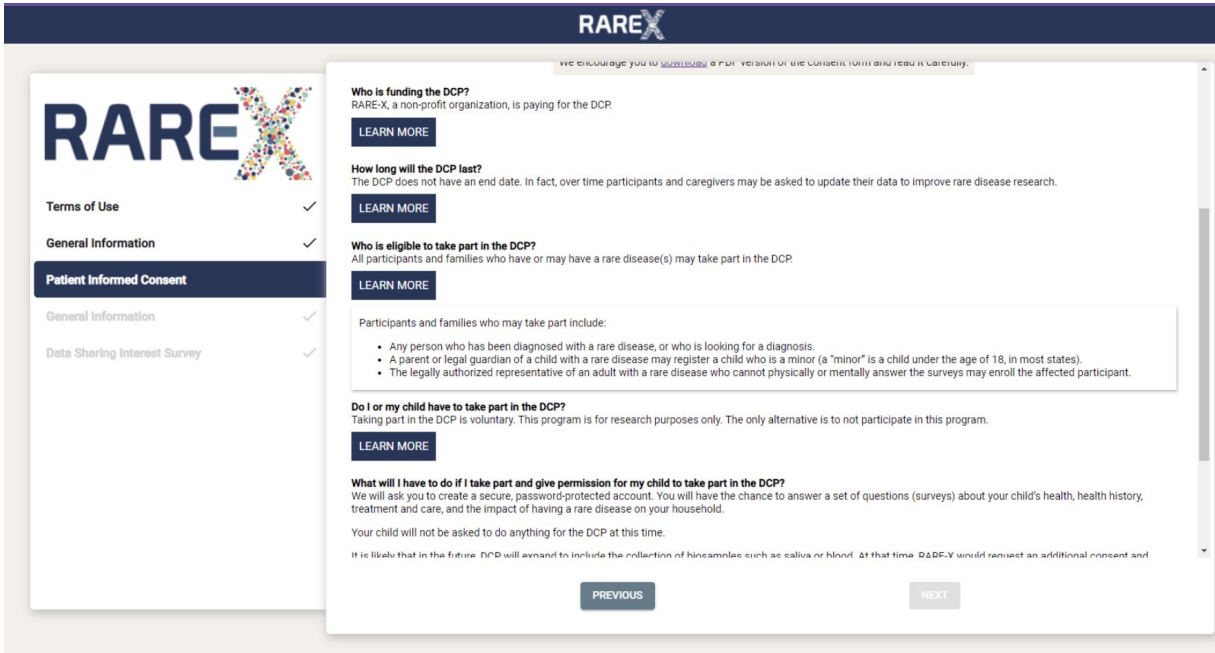
Technology Platform

Terms of Use



Informed Consent

8 Pages of Detailed Q&A to Ensure Understanding



Informed Consent

Check all that apply

I am taking part in the RARE-X DCP for one or more of the following reasons (Check all that apply) *

- [ ] You have stated that you have or may have a rare disease.
- [ ] You are the Parent or Caregiver of a person who has or may have a rare disease.
- [ ] You are the legally authorized representative of a person who has or may have a rare disease.
- [ ] You are the family member, other than a parent, caregiver or legally authorized representative of a person who has or may have a rare disease.
- [ ] You have lost a person who had or may have had rare disease.

Check the boxes below to indicate if you agree to the following options. *If you check "no" to any given option, you can still take part in the DCP.*

RARE-X may contact me with follow-up research surveys and invitations to take part in additional studies. I may choose to ignore these surveys/invitations. *

- ◯ Yes
- ◯ No

RARE-X or a qualified patient organization may contact me if a researcher thinks that I qualify to be part of a clinical trial/study. *

- ◯ Yes
- ◯ No

Informed Consent

If you are a caregiver of a patient, be sure to provide your info and the child's info in the correct places

I am taking part in the RARE-X DCP for one or more of the following reasons (Check all that apply) *

- [ ] You have stated that you have or may have a rare disease.
- [x] You are the Parent or Caregiver of a person who has or may have a rare disease.
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Check the boxes below to indicate if you agree to the following options. *If you check "no" to any given option, you can still take part in the DCP.*

My signature below indicates:

- I am 18 years or older.
- I have read this consent form. I understand the information in this form. I have had enough time to read the consent form and think about agreeing to take part in the DCP.
- I have had the opportunity to ask questions related to the DCP and do not have any unanswered questions at this time.
- I am willing to take part in the DCP.
- I agree to allow the collection, use, and sharing of my data as described above.
- By signing and dating this form, I do not give up any of my legal rights.
- I will get a signed and dated copy of this consent form.

Your complete legal first (given) name *

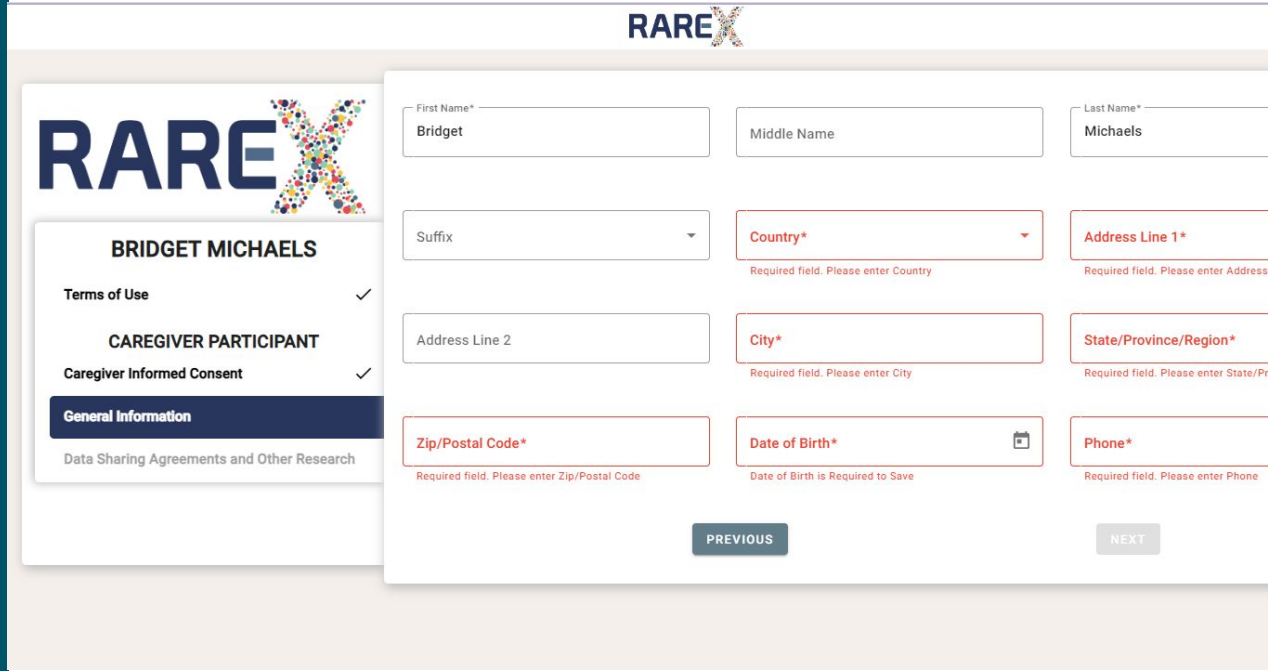
Bridget

Your complete legal last (family) name *

Michaels

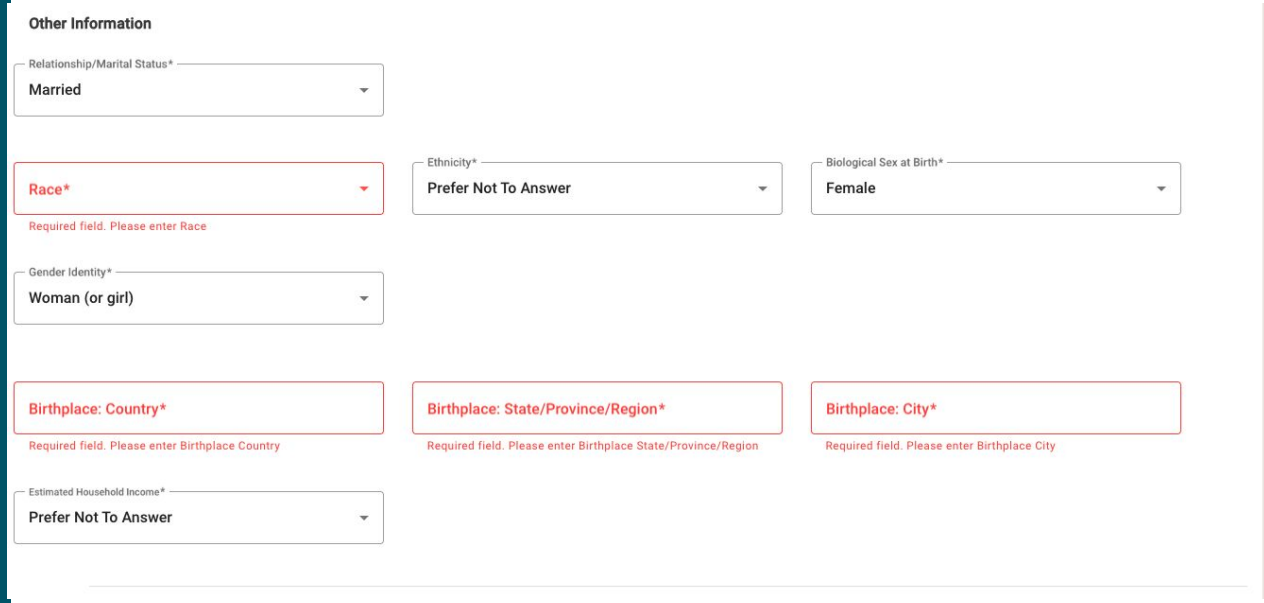
General Information *Caregiver Demographics*

Demographic information collected on both patients and caregivers



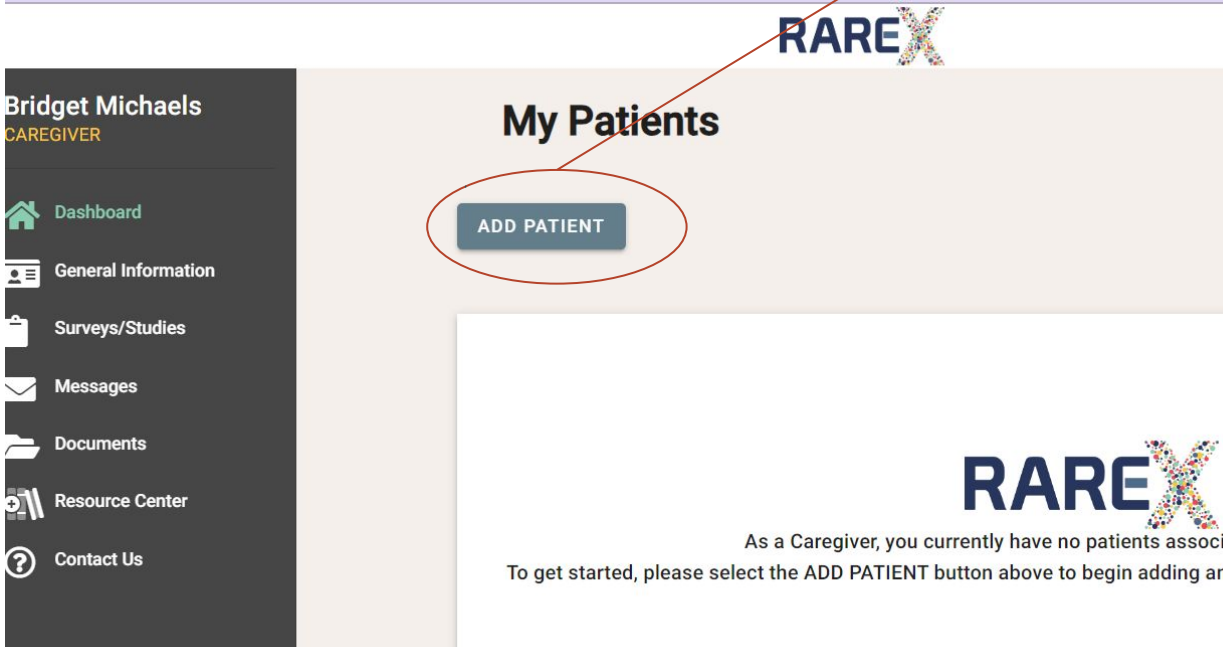
General Information *Demographics*

Demographic information collected on both patients and caregivers



Caregiver Dashboard
Adding a Patient

Click ADD PATIENT on your Caregiver Dashboard to add your patient participant



Informed Consent

This time, you will complete the consent form on behalf of the patient.

I am taking part in the RARE-X DCP for one or more of the following reasons (Check all that apply) *

- [ ] You have stated that you have or may have a rare disease.
- [x] You are the Parent or Caregiver of a person who has or may have a rare disease.
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Your complete legal first (given) name *

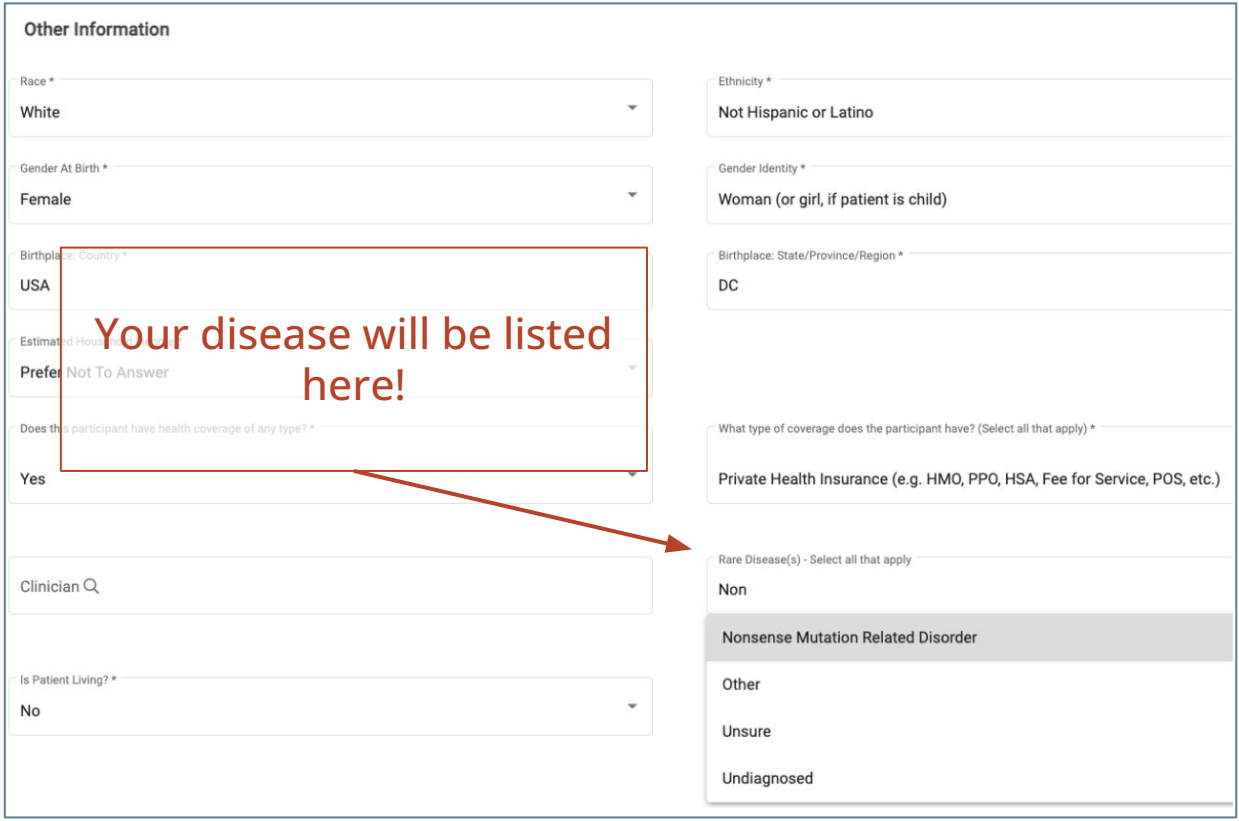
Bridget

Your complete legal last (family) name *

Michaels

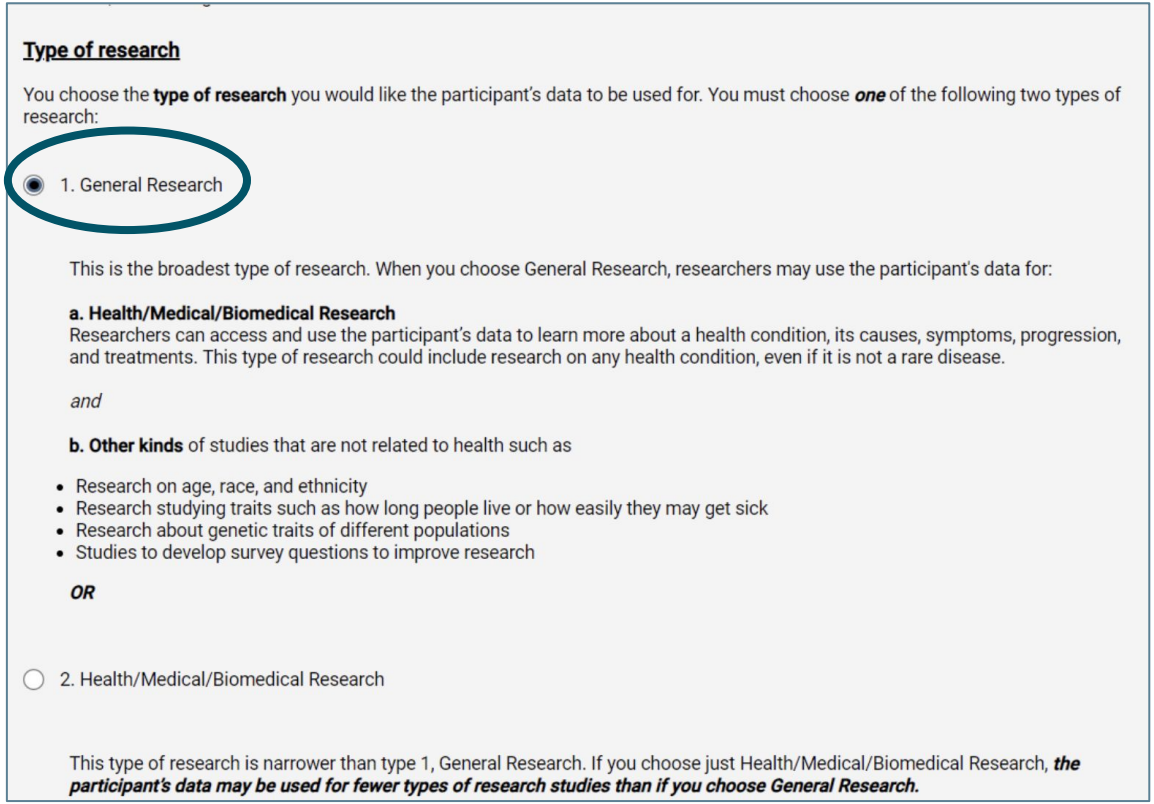
General Information *Patient Demographics*

Select the Rare Disease Community in the patient demographics section



Data Sharing *Preference Agreement*

By selecting General Research, your participant's data will reach the most researchers (recommended)



Type of research

You choose the **type of research** you would like the participant's data to be used for. You must choose ***one*** of the following two types of research:

(●) 1. General Research

This is the broadest type of research. When you choose General Research, researchers may use the participant's data for:

a. Health/Medical/Biomedical Research
Researchers can access and use the participant's data to learn more about a health condition, its causes, symptoms, progression, and treatments. This type of research could include research on any health condition, even if it is not a rare disease.

and

b. Other kinds of studies that are not related to health such as

- Research on age, race, and ethnicity
- Research studying traits such as how long people live or how easily they may get sick
- Research about genetic traits of different populations
- Studies to develop survey questions to improve research

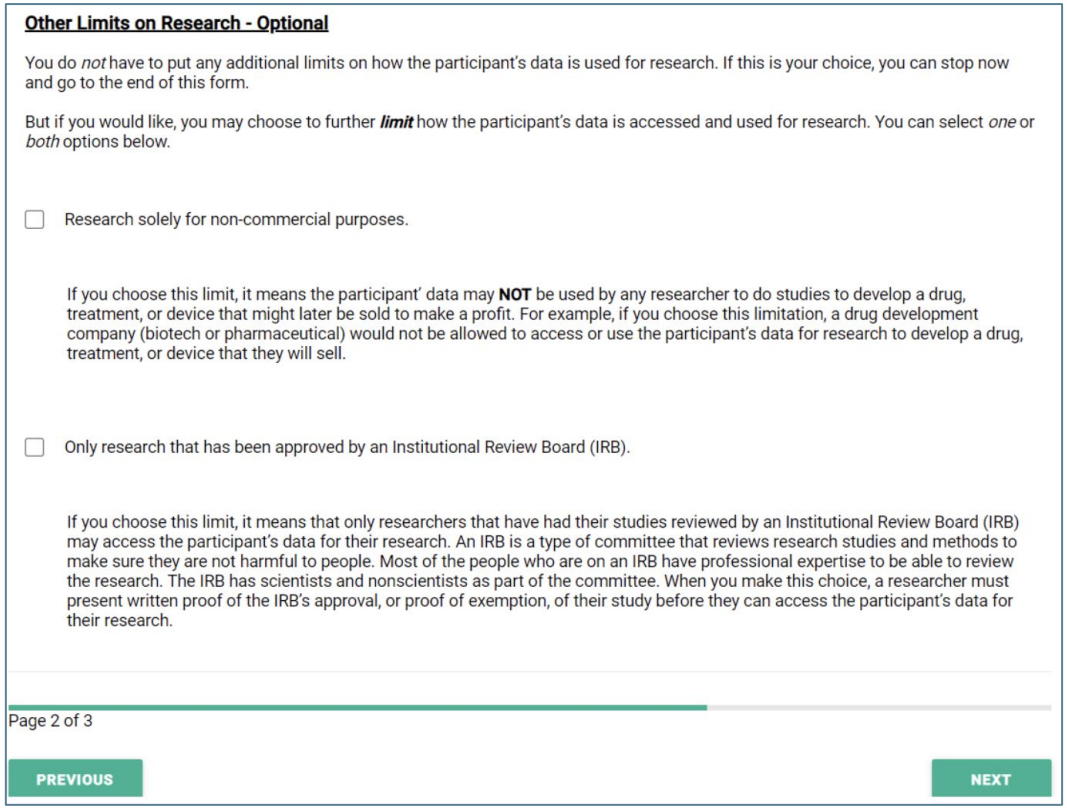
OR

( ) 2. Health/Medical/Biomedical Research

This type of research is narrower than type 1, General Research. If you choose just Health/Medical/Biomedical Research, ***the participant's data may be used for fewer types of research studies than if you choose General Research.***

Data Sharing *Preference Agreement*

Optional: Setting Restrictions



Other Limits on Research - Optional

You do *not* have to put any additional limits on how the participant's data is used for research. If this is your choice, you can stop now and go to the end of this form.

But if you would like, you may choose to further ***limit*** how the participant's data is accessed and used for research. You can select *one* or *both* options below.

☐ Research solely for non-commercial purposes.

If you choose this limit, it means the participant' data may **NOT** be used by any researcher to do studies to develop a drug, treatment, or device that might later be sold to make a profit. For example, if you choose this limitation, a drug development company (biotech or pharmaceutical) would not be allowed to access or use the participant's data for research to develop a drug, treatment, or device that they will sell.

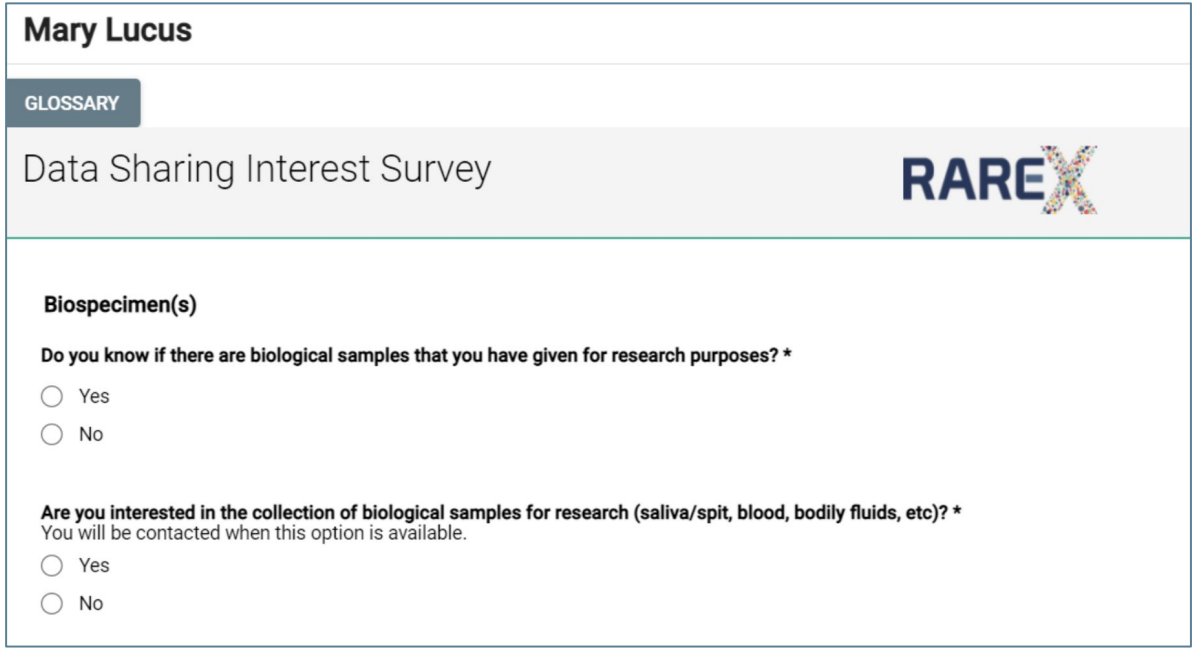
☐ Only research that has been approved by an Institutional Review Board (IRB).

If you choose this limit, it means that only researchers that have had their studies reviewed by an Institutional Review Board (IRB) may access the participant's data for their research. An IRB is a type of committee that reviews research studies and methods to make sure they are not harmful to people. Most of the people who are on an IRB have professional expertise to be able to review the research. The IRB has scientists and nonscientists as part of the committee. When you make this choice, a researcher must present written proof of the IRB's approval, or proof of exemption, of their study before they can access the participant's data for their research.

Page 2 of 3

PREVIOUS NEXT

Data Sharing *Preference Agreement*



Mary Lucus

GLOSSARY

Data Sharing Interest Survey

RAREX

Biospecimen(s)

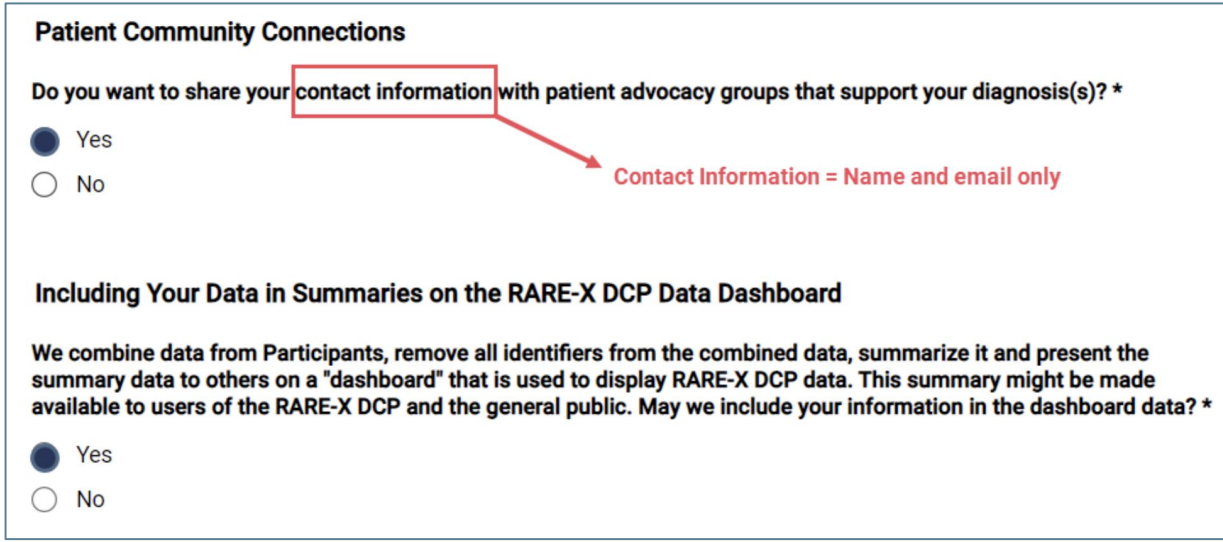
Do you know if there are biological samples that you have given for research purposes? *

- ◯ Yes
- ◯ No

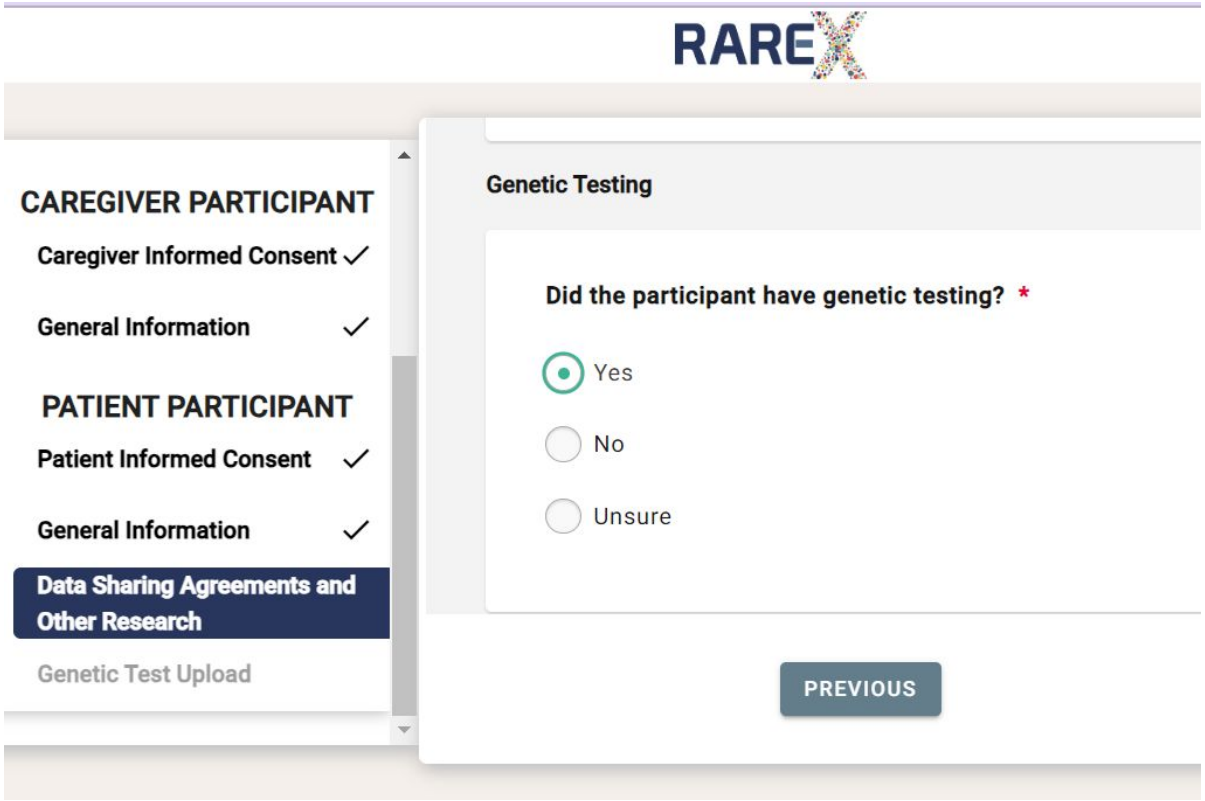
Are you interested in the collection of biological samples for research (saliva/spit, blood, bodily fluids, etc)? *
You will be contacted when this option is available.

- ◯ Yes
- ◯ No

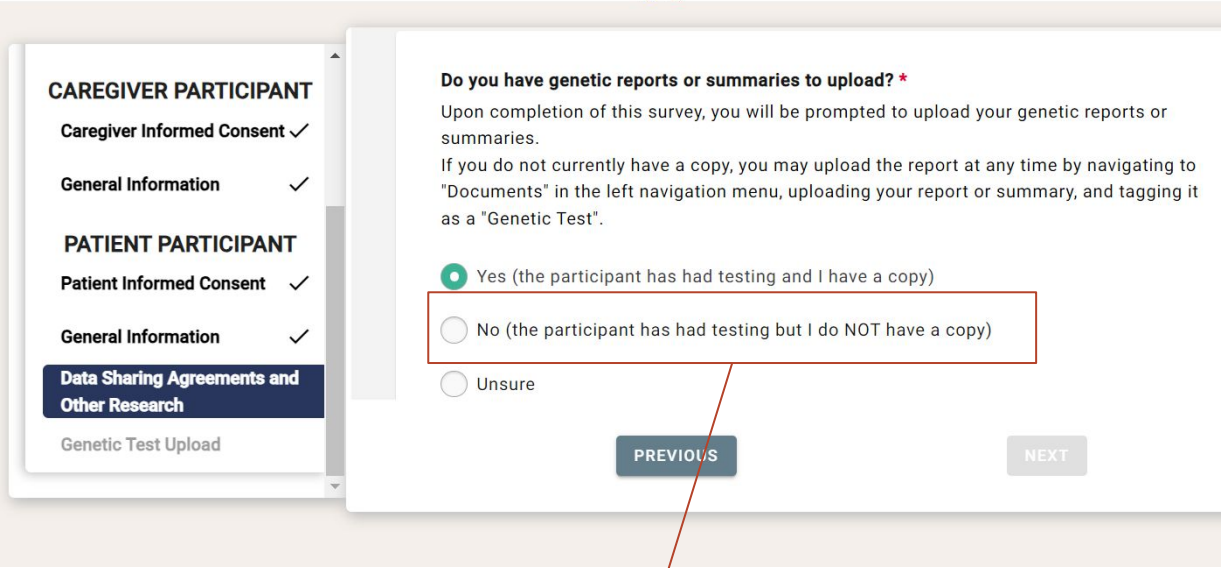
Data Sharing *Preference Agreement*



Genetic Testing Information



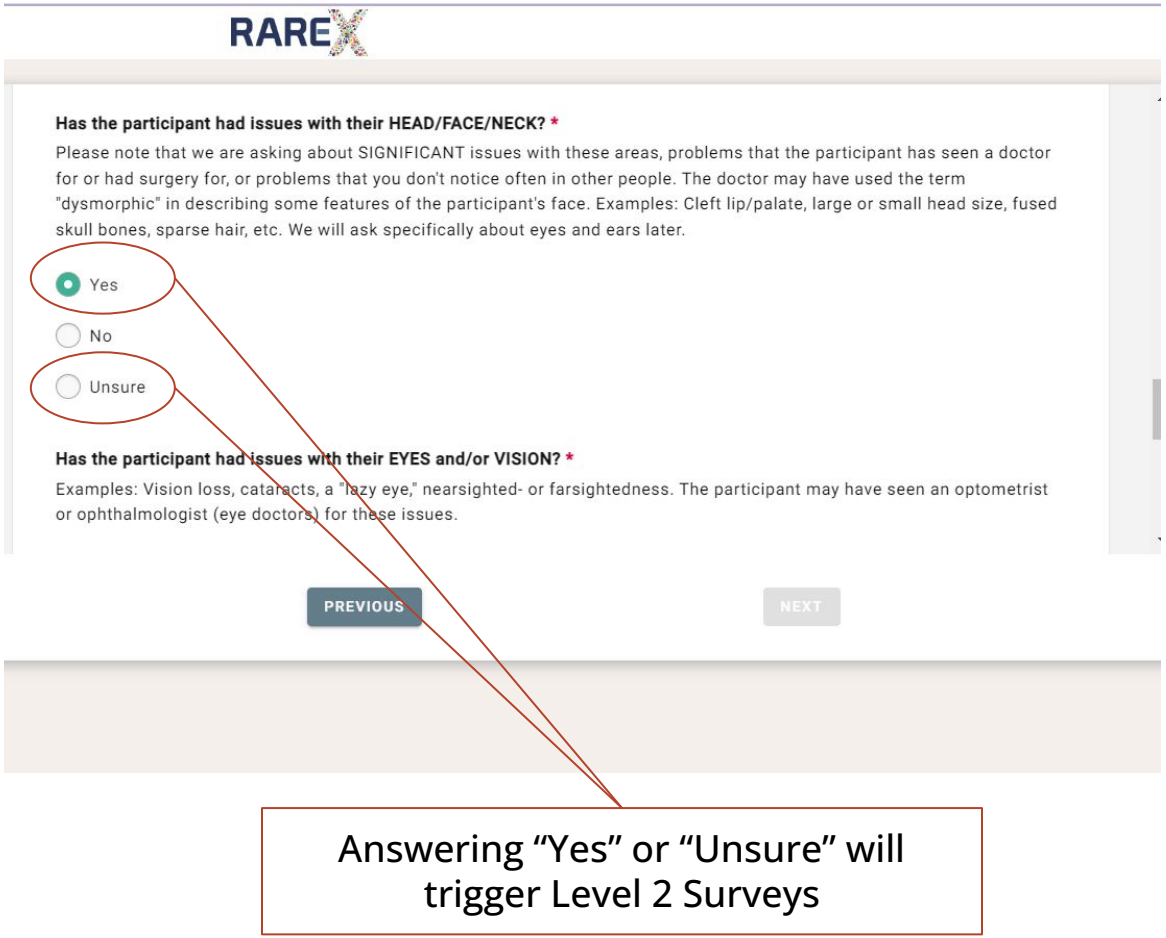
Genetic Report Upload



If you select “No” you will still be able to upload your report later!

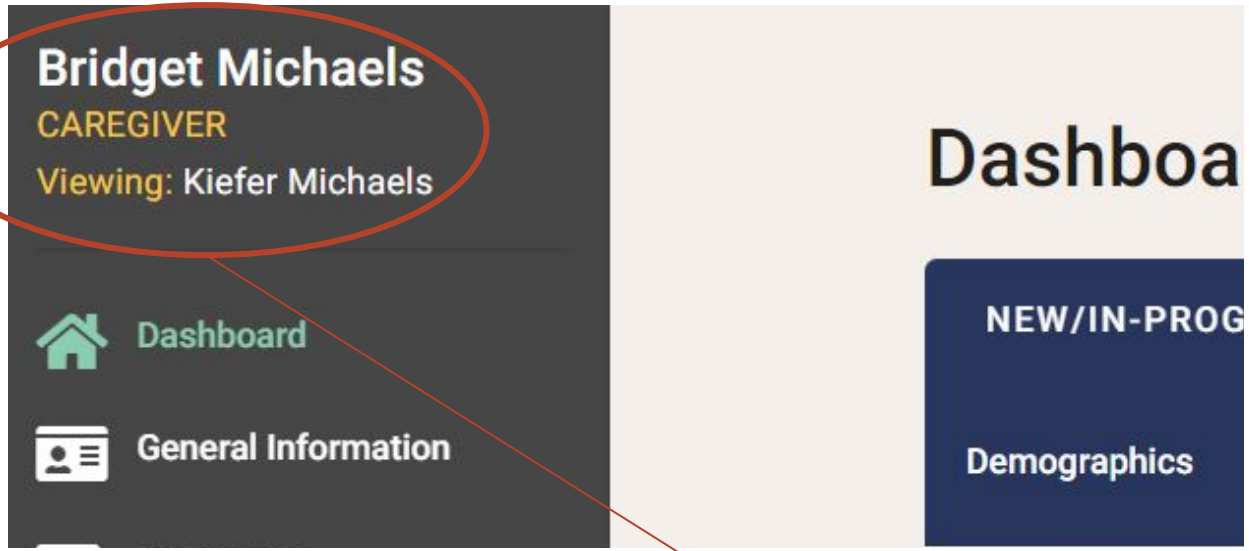
Head to Toe Survey

All Level 2 surveys "branch" from the Head to Toe



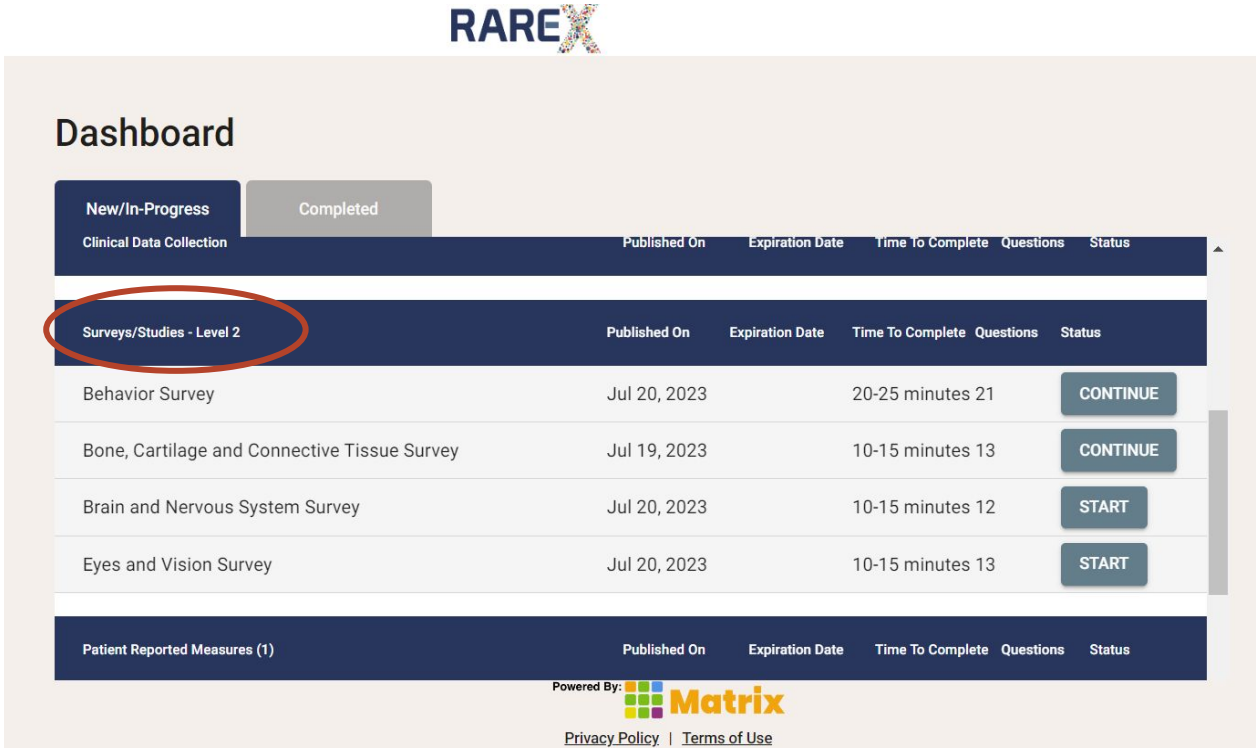
Caregiver Dashboard

Navigating Between Caregiver & Patient

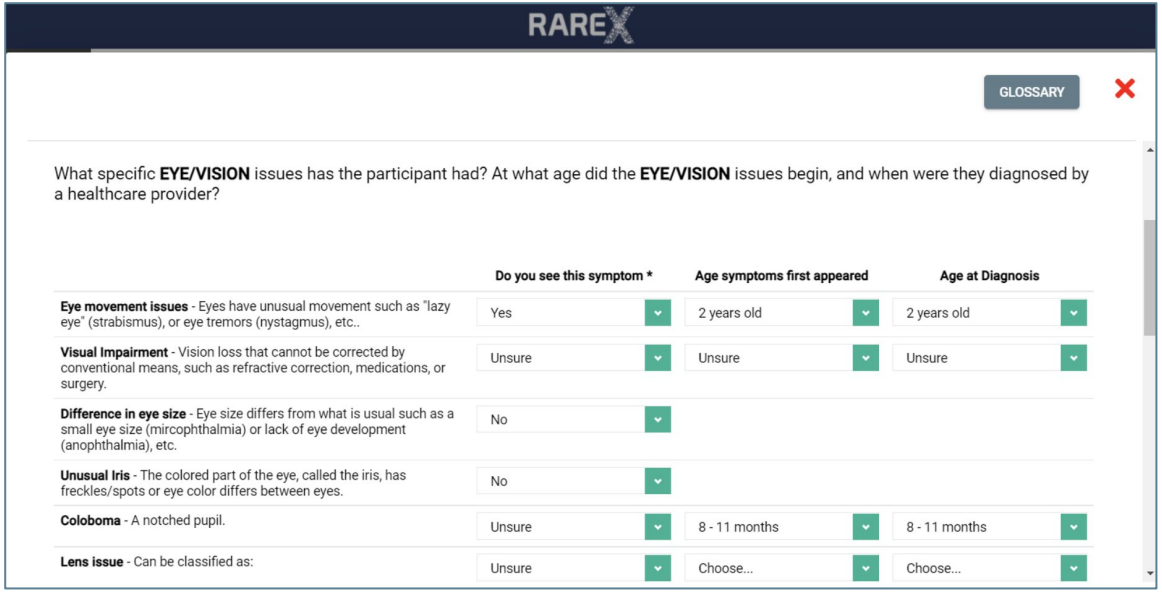


The top left corner will always indicate whose dashboard you are viewing!

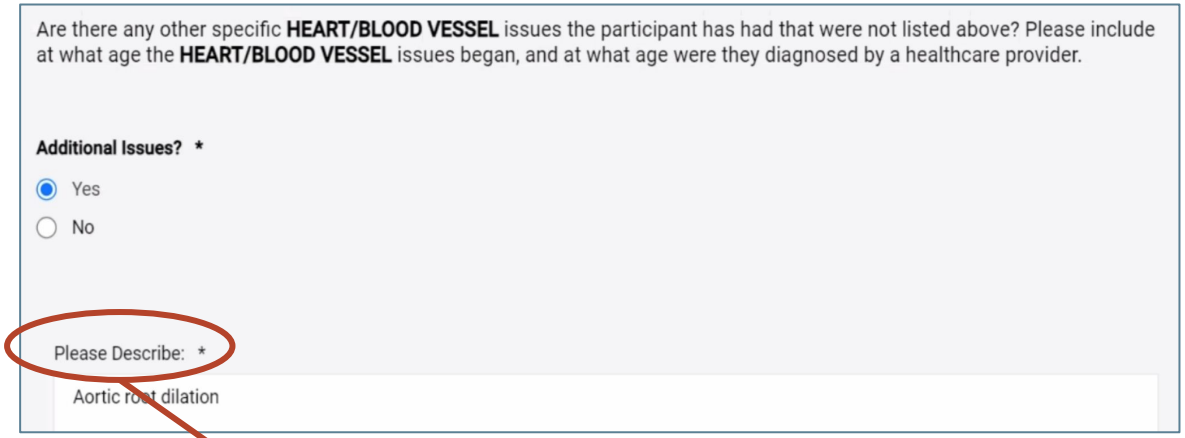
Surveys
Level 2 Surveys



Surveys
Answering Level 2 Surveys

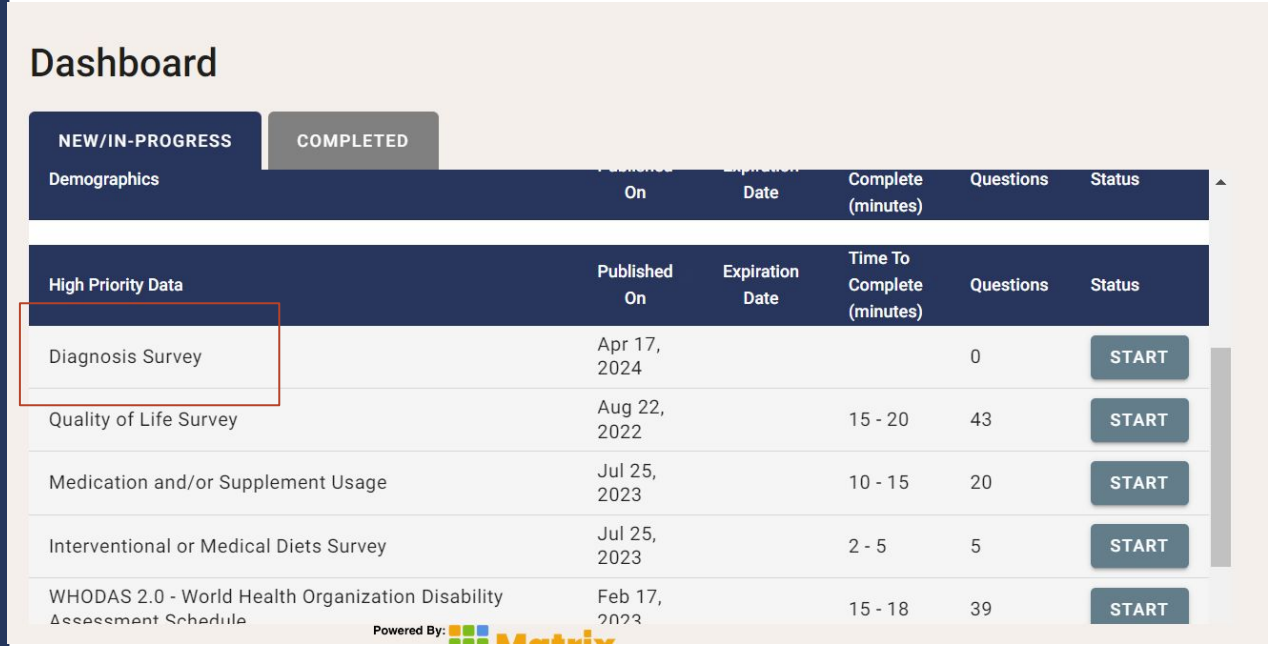


Surveys
Adding Symptoms Not Mentioned in Level 2 Surveys



Try not to include any identifying information in the free text boxes!

Surveys
Diagnosis Survey



Diagnosis Survey

You chose the following condition as the rare disease diagnosis you affiliate with: **NALCN channel-related disorders (including IHRPF)**
Please note: If you chose more than one diagnosis when you set up your account, you will be asked these questions about each diagnosis separately.

There are multiple specific conditions that can be diagnosed in individuals in this disease community. Does one of these more specific conditions apply to you? *

- [ ] CLIFAHDD syndrome, NALCN-related
- [ ] IHPRF1 (Infantile Hypotonia syndrome), NALCN-related
- [ ] IHPRF2 (Infantile Hypotonia syndrome), UNC80-related
- [ ] NALCN Channel-related disorder, unknown type

The Diagnosis survey will ask about any sub-diagnosis you may have!

Please note that throughout this survey, "you" refers to the participant with the rare disease. If you are filling this survey out as a caregiver, please respond based on your observations of the person you care for.

Diagnosis Survey

Do you know the genetic cause of your disease? *

If you are not sure of the genetic cause, please say no.

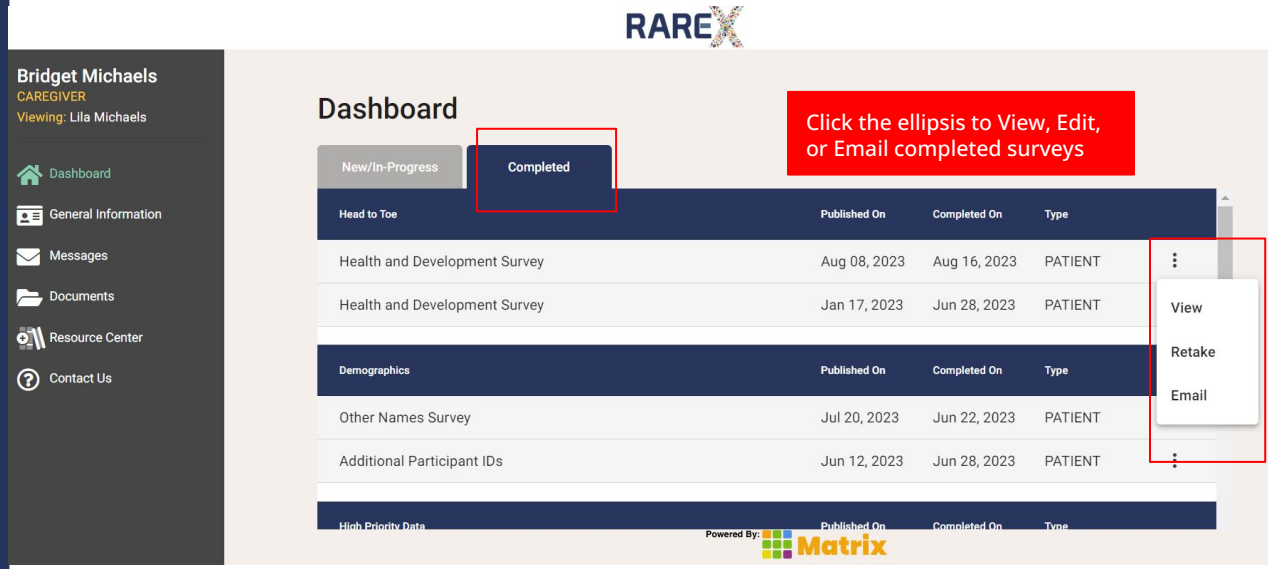
- ○ Yes
- ○ No

Family History

Please remember that these questions pertain ONLY to this diagnosis: Sleep-related disorders.

The Diagnosis survey will also ask about genetic testing, newborn screening, and family history

Dashboard
Completed Tab



If you have questions or technical issues…

Email:

- rarexsupport@globalgenes.org

Questions?