

## Data Sharing Guide for RareX

On RareX, you have control over who and for what reasons your data is used. The decision is 100% a personal choice, and you should select whatever feels right for you! All data is de-identified.

As a parent-led organization, we hope many international scientists, clinicians, organizations, and pharmaceutical companies take an interest in studying NALCN channel-related diseases and developing treatments and cures for our children. To this effort, reducing restrictions on data sharing and making data easy to access and use can encourage scientists and companies to include NALCN channel-related diseases in their research and drug development efforts. Below are the selections we recommend and the explanations behind them:

### Sharing Data for Different Types of Research

#### 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.**

- The participant's data may **only** be used to learn more about a health condition, its cause, symptoms, progression, and treatments. (Research described in section 1.a. above)
- The participant's data will **not** be used for other kinds of studies not related to health described in section 1.b. above.

**For type of research, we recommend selecting General Research to be as broad as possible.**

This type of research focuses on understanding diseases, their causes, and how to treat or prevent them. Supporting it brings several benefits, including:

- Improving health outcomes:
- Developing personalized medicine
- Innovating in health technology

**Other Kinds** includes research on social, demographic, and genetic traits, as well as broader societal studies. Supporting these kinds of research is important for:

- Understanding health disparities
- Influencing public health policy
- Improving research methodologies
- Understanding genetic and environmental interactions

## Other Limits on Data

### Other Limits on Research - Optional

(You do **not** have to put any additional limits on how your data is used for research.)

If you would not like to add any additional limits as explained in the consent form and also below to how your data is used, please check this box and the additional limit options will be removed.

**No additional limits on research**

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's 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.

**For "Other Limits on Data," we recommend selecting "No additional limits on research" to be as broad as possible.**

Rare diseases often lack research funding because they affect a small number of people, making them less attractive to many funding sources. However, **commercial companies** (like pharmaceutical and biotech companies) often have the resources and incentive to develop targeted treatments for these diseases. Limiting data use to non-commercial purposes may exclude pharmaceutical and biotech companies from being able to access and use data on NALCN channel-related diseases and instead focus their resources and efforts on other rare diseases that they have access to data on.

### Here is why we suggest *not* limiting data to only IRB-approved studies:

- **IRB approval is not always practical:** Obtaining IRB approval for studies can often take months and cost money. Some small studies, especially pilot studies or exploratory research, may not be able to acquire IRB approval. Limiting research to IRB-approved studies could prevent new studies from accessing the data they need to generate initial findings to justify larger studies.
- **Support Collaboration:** Some researchers, especially those working in smaller or emerging fields, may face delays in getting their studies reviewed or approved by an IRB, which could limit the collaboration between researchers or prevent innovative approaches from being explored quickly.
- **Support non-health-related research:** IRBs are typically focused on protecting participants in medical or clinical studies. If the data access limitation applies only to IRB-approved studies, social scientist or non-health-related researchers might not be able to access important data.

Final questions on data sharing

**Saying Yes to Patient Communication Connections will help Channeling Hope with communication efforts.** This includes informing families of future data collection/research opportunities, sending appreciation to families for completing the surveys, and convening the patient community.

**Patient Community Connections**

Do you want to share your **contact information** with patient advocacy groups that support your diagnosis(s)? \*

- Yes
- No

Contact Information = Name and email only

**Saying Yes to including summary data will help Channeling Hope with outreach efforts.** Current dashboards include number of patients enrolled, geographic location, age groups, and ethnicity. This will greatly help Channeling Hope and RareX advocate among funders, organizations, and scientists to support our mission and efforts to better understand and development treatments for these diseases.

**Including Your Data in Summaries on the RARE-X DCP Data Dashboard**

We combine data from Participants, remove all identifiers from the combined data, summarize it and present the summary data to others on a "dashboard" that is used to display RARE-X DCP data. This summary might be made available to users of the RARE-X DCP and the general public. May we include your information in the dashboard data? \*

- Yes
- No

Below are examples of how dashboards can be used.

**MEMBERSHIP OUTREACH**  
*To encourage participation*

**SOCIAL MEDIA POSTS**  
*To share insights*

**COMMUNICATION WITH RESEARCHERS**  
*To educate & inspire*

We are pleased to announce that we now have 36 patients from our community on the RARE-X DCP platform! Thank you to everyone who has created an account and started answering surveys.

**Has your family been counted?**

**Did you know? We're GLOBAL!**

Country	Patient Count	Percent of Global Patients
Afghanistan	2	6.7%
Aland Islands	5	16.7%
Albania	1	3.3%
American Samoa	2	6.7%
United States	20	66.7%
<b>Total</b>	<b>30</b>	<b>100.0%</b>

Families from additional countries coming onboard soon.

**36 and counting.**  
No other database in the world has up-to-date phenotype information on this many patients with X disorder.

Access to this data is available to you for free! Contact \_\_\_\_\_ to get an account.

If you have additional questions about your decision, please email [channelinghopefoundation@gmail.com](mailto:channelinghopefoundation@gmail.com).