

RARE-X DATA COLLECTION PROGRAM (DCP)
PARENT OR GUARDIAN PERMISSION
FOR CHILD UNDER 7 YEARS OF AGE TO PARTICIPATION IN RESEARCH

Title: RARE-X Data Collection Program

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Please read the consent form below carefully. When you are comfortable that you understand what you have read, click “I understand” to move on to the next section. If you have questions about the RARE-X Data Collection Program (called “RARE-X DCP” or “DCP” in this Consent) or the Consent form, please contact us at (716) 427-2739 or consent@rare-x.org.

Summary of Research Program
Full Form
Sign Consent

Summary of Data Collection Program (DCP)

You are being asked to take part in the DCP because you stated that your child you have or may have a rare disease.

Taking part in the DCP is voluntary.

The purpose of the DCP is to collect and store information about patients (called “data”) with many different types of rare diseases. The DCP will make this data available to researchers worldwide for their studies. The DCP will also help improve patients’ connections with researchers and increase the number of patients taking part in research studies and clinical trials. These trials could be for medicines, medical devices, or other therapies.

Through your account on the RARE-X DCP website, you will be asked one or more sets of questions (called “surveys”) about your health, health history, treatment and care, and the effect of having a rare disease on your household. The DCP will store your data, along with other participants, in a secure server. By

making choices through your account, you will be able to tell RARE-X who you want to share your data with. Your choices may include researchers, patient groups, life science commercial companies (biotech, pharma, drug manufacturers) and others.

The data you provide may help research efforts to one day offer better treatment and prevention options for rare diseases like yours. However, you may not directly benefit from your participation in this program. *(See potential benefits and risks sections below.)*

There is a small risk that someone who is not authorized would see data that identifies you. *(See more information in the confidentiality section below.)*

If you are interested in learning more about the DCP, please keep reading on the next page.

RARE-X DATA COLLECTION PROGRAM (DCP)

FULL RESEARCH CONSENT FORM

Introduction

RARE-X is a non-profit organization that has created the RARE-X Data Collection Program (“DCP”) to support patients with rare diseases in collecting and storing their data for research and future investigation. The DCP allows patients to choose how their data is seen, shared, and used for support and research.

You are being asked to take part in the DCP because you stated that you have or may have a rare disease.

The information below may help you decide if you want to take part in the DCP and what you will have to do if you decide to take part. It also describes the risks and benefits of taking part. Any statements in the website [Terms of Use](#) that appear to limit your rights do not apply to your participation in this research program.

If you have any questions or do not understand some of the information about taking part in the DCP, you should ask the contact listed at the top of this

Consent by email or phone. It is important that you understand the DCP and your choices. You can talk about your participation in the DCP with anyone you choose to help you understand the information. Do not sign this form until your questions have been answered, and you decide that you want to be part of the DCP.

What is the purpose of the DCP?

The DCP is a program to collect and store data about patients with lots of different kinds of rare diseases for research and patient support. Another purpose of the DCP is to increase patient recruitment into research studies and clinical trials.

With your permission, your de-identified data in the DCP can be shared with researchers (including researchers at drug companies) and approved patient organizations worldwide.

Who is Funding the DCP?

RARE-X, a non-profit organization, is paying for the DCP.

RARE-X does not make money (a profit) for its work on this program. RARE-X raises funds through grants, support contracts, and sponsorships.

How long will the DCP last?

The DCP does not have an end date. In fact, overtime patients/participants and caregivers may be asked to update their data to improve rare disease research.

You can quit at any time. *(See section about quitting below.)*

Who is eligible to take part in the DCP?

All patients and families who have or may have a rare disease(s) may take part in the DCP.

What if my child does not want to take part in the DCP?

If your child does not agree to participate, they will not be included in the DCP and there will be no penalty. If your child initially agrees to be in the study, they can change their mind later without any penalty.

If you give your permission for your child to participate in the DCP by signing this form, you will be asked to read an “Assent” form to your child which will explain the DCP in plain language and will ask your child if they agree to participate.

Patients and families who may take part include:

- Any person who has been diagnosed with a rare disease, or who is looking for a diagnosis.
- A parent or guardian of a child with a rare disease may register a child who is a minor (a “minor” is a child under the age of 18, in most states).
- The legally authorized representative of an adult with a rare disease who cannot physically or mentally answer the surveys, may enroll the affected patient.

Do I have to take part in the DCP?

Taking part in the DCP is voluntary.

You can choose not to take part at all. You can take part in some or all of the DCP.

You can change your mind at any time.

No matter what you decide, now or in the future, it will not affect your medical care. There will be no penalty to your child.

What will I have to do if I take part in the DCP?

We will ask you to create a secure, password-protected account. You will have the chance to answer a set of questions (surveys) about your health, health history, treatment and care, and the impact of having a rare disease on your household.

How will my data be used, and for how long?

Your data will be safely stored on a secure server and made available to researchers and trained patient organizations that you choose. There is no set time limit on how long the DCP will store your data for future research.

What kind of data can I choose to share?

There are two kinds of data that you can choose to share:

- Identifiable data may have your name, date of birth, or other personal data on it that a person could use to identify you easily.

- De-identified data has had your name and other personal identifiers removed from your data and replaced with a code to keep your privacy.

Who can I choose to share my *identifiable* data with, and what will they do with it?

You may choose to share your *identifiable* data with qualified patient organizations associated with your disease.

A qualified patient organization will have training in research rules about privacy and security. They may contact you to offer community support. They will also be able to help you with completing your DCP surveys if you need help. RARE-X offers you to share your data this way because RARE-X understands the importance of patient community/organization support in rare disease.

Who can I choose to share my *de-identified* data with, and what will they do with it?

You can choose to share your *de-identified* data with researchers for their studies. These studies may happen in the future. The researcher will not have to ask you again if you want to take part in any specific study they do in the future. The researchers will be able to include your data in current and future studies related to your disease or to other patients with similar issues or, if you choose, other subjects. This process allows your data to be used frequently by many researchers.

This also means that researchers may do a study in the future using your data for a study that you may disagree with.

With your permission, we will share your de-identified data with many different types of researchers. For example, researchers might be associated with universities, patient organizations, or drug companies.

Some of these studies may be about diseases *other* than the disease that affects you. Sometimes researchers discover that unrelated diseases may have connections. Some of these researchers may study ways to collect data to better measure health issues and how health issues might change with treatment or care. For example, the researchers might study how to collect data in a standard way, so the data is easier to study. Some of these studies may be for for-profit purposes, such as developing new drugs.

When researchers request data for analysis, they are required to tell RARE-X what they will use the data for and agree only to use it for that reason.

How can I choose who to share my data with?

RARE-X knows that you may change your mind about the people and organizations that you want to share your data with over time. You can change your preferences at any time through the online Data Sharing Preference Survey in the RARE-X DCP.

You will have to 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 your data for:

a. Health/Medical/Biomedical Research

Researchers can access and use your 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, your data may be used for fewer types of research studies than if you choose General Research.

If you choose just Health/Medical/Biomedical Research, your 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)

Your data will ***not*** be used for other kinds of studies not related to health described in section 1.b above.

Other Limits on Sharing for Research

You will also be able to choose to further ***limit or restrict*** how your data is used for research. You don't have to put any further limits on how your data is used, but if you would like to, you can choose restrictions such as:

A. Not allowing your data to be used for commercial/for-profit research purposes

B. Not allowing your data to be used for research projects unless an Institutional Review Board (IRB) has approved them.

An Institutional Review Board, 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.

Whether you say yes or no to these choices, you can still take part in this Program.

Who else is my data shared with?

As part of this Consent you understand that RARE-X may need to see and use your data to help improve the quality of the DCP or as required by law.

For example, your data may be seen/used by:

- A limited number of RARE-X staff and contractors who need the data to do their jobs; or
- When required, with federal regulatory or oversight authorities; or
- Our Institutional Review Board (IRB) so that they can make sure we are following your consent choices and the ways that we have said we would run this Program; or

- Officials if otherwise required by law (such as if we find or suspect child abuse).

Will I be contacted if it looks like I might be able to take part in a research study or clinical trial (drugs, devices, or other treatments) based upon the data I provided?

It is your choice if you would like to be contacted by RARE-X or a patient organization if a researcher thinks you might be a good fit for a research study and/or clinical trial. You will be given a choice at the end of this form to indicate whether or not you wish to be contacted.

If you say yes, a staff member from RARE-X or a qualified patient organization will contact you to tell you about the study to see if you might be interested in taking part in a study or trial and how to contact the researcher. You will NOT be contacted directly by the researcher. It will be up to you whether you want to contact the researcher to further discuss possibly taking part in a research study and/or clinical trial.

Will I benefit from taking part in the DCP?

You are not likely to directly benefit from participating in this Program.

However, the data you share may help research efforts to one day provide better treatment and prevention options for your rare disease.

Will I be paid for taking part in the DCP?

No. You will not be paid for taking part in the DCP.

You will not be paid if new drugs, tests, devices, or commercial/for-profit products are created due to research on your data.

Will I find out the results of the research?

No. You will not receive individual results from research done using your data. However, the DCP will provide summary results about research and significant discoveries to all participants.

We will do this by regularly updating the DCP website that you used to learn about this program. Having access to this summary data may help you better understand your symptoms and care options.

How will data about me be kept confidential?

RARE-X will remove your name and other personal identifiers and replace them with a special code number before sharing your data with others. RARE-X will encrypt (scramble) your data when stored, so it is hard for people without permission to read it.

Here are some other steps we take to keep your information confidential:

- Any researchers using your data must sign an agreement promising that they will not try to find out who you are.
- Only a few members of the RARE-X staff who receive special training will have access to your identified data and only for authorized purposes.
- All RARE-X staff and contractors with access to identifiable data must sign an agreement with RARE-X to keep data confidential. If you have questions or concerns, ask the Principal Investigator, whose contact information is on the top of the page, for more information.
- We limit access to your data to only those organizations and people who are authorized to see it.

What are the risks of taking part in the DCP?

As with all online data collection, there is a small risk that someone who does not have permission could see the data that RARE-X has stored about you.

An accidental release of your data could possibly identify you or your family. We believe the chances that these things will happen is very small, but we cannot guarantee absolute confidentiality.

Because of the small number of people who have some rare diseases, there is a risk that a researcher familiar with your rare disease community and/or genetics could connect de-identified data back to you. Although they may know the data is about you, they are bound by their agreement with RARE-X not to contact you or publish data that identifies you.

It is possible that someone who has access to your identifiable data could use or share it in a way that could make it harder for you or your family to get or keep a job or insurance. There are laws against the misuse of genetic data, but they may not protect your data in all cases.

Will it cost me anything to take part in the DCP?

No. There are no costs for you to take part in the RARE-X DCP.

Can I access my own data from the DCP?

RARE-X will allow you to see and print the data you put in the DCP.

Can I quit this Program?

Yes, you can stop taking part in the DCP at any time for any reason.

If you decide to quit, you will be offered some choices about whether RARE-X keeps or deletes your data.

To stop participating, you must do so in writing by contacting the Principal Investigator listed on the top of this form.

If you quit, you can choose to:

- Allow the DCP to keep the data RARE-X has already collected about you. RARE-X will continue to share it with researchers for future research studies; or
- Require the DCP to delete all your data, including the special code assigned to your data. However, if you choose to delete your data, we cannot take back any of your data that has already been shared with researchers or approved patient organizations. We will not be able to contact you for any reason in the future, including telling you that you may be a good fit for possible studies or clinical trials.
 - You may ask RARE-X to transfer your data to another data collection platform before we delete it.
 - If you delete your data and choose to re-join the DCP in the future, you will have to start over again from the beginning.

What if I have questions, concerns, or complaints about the DCP?

If you have questions, concerns, or complaints about the DCP or this Consent, please contact the Principal Investigator listed at the beginning of this Consent.

If you have questions or concerns about your rights as a participant in the DCP and you are not able to resolve your concerns or complaints with the Principle Investigator you may contact:

Advarra IRB

Advarra IRB reviewed this study. Advarra is a group of people who review research studies to protect research participants' rights and welfare. You can ask Advarra general questions about what it means to be in a research program. Review by Advarra does not mean that the DCP is without risks.

Consent Documentation

I understand and agree that:

- I am voluntarily providing my personal data to the RARE-X DCP as a place for me to put my data for use in research.
- I may choose to stop participating at any time. If I quit, my data may be deleted at my request. I also understand that any data that has already been shared with researchers and used in a specific study cannot be taken back. If I quit, it will have no impact on my medical care.
- The DCP may share my de-identified data for the types of research that I choose. These research studies may happen in the future.
- I may not personally benefit from participating in the DCP or from the use of my de-identified data in any research study.
- I give permission to the RARE-X DCP's staff to contact me to ask me to update my health status, or my contact information, to request that I upload a particular attachment or to complete forms associated with my participation in the DCP.

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.*

YES NO

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.

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

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 this form, I do not give up any of my legal rights.
- I will get a signed copy of this Consent form.

Printed Name of Participant

Signature of Participant

Date of Signature