

**INFORMED CONSENT FORM  
FOR ADULT PARTICIPANTS**

**Sponsor / Study Title:** RARE-X / "RARE-X Data Collection Program"

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**Summary of Data Collection Program (DCP)**

You are being asked to take part in a research study (the DCP) because you stated that 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 (called "data") about participants 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 participants' connections with researchers and increase the number of participants taking part in research studies and clinical trials. These studies/trials could be for study drugs, study devices, or other study therapies. The total number of participants who will take part in this study will not have a cap.

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.

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**Introduction**

RARE-X is a non-profit organization that has created the RARE-X Data Collection Program ("DCP") to support participants with rare diseases in collecting and storing their data for research and future investigation. The DCP allows participants 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.

If you have any questions or do not understand some of the information about taking part in the DCP, you should ask the principal investigator contact listed at the top of this consent by phone or email. 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 and date 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 participants with lots of different kinds of rare diseases for research and participant support. Another purpose of the DCP is to increase participant 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, over time 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 participants and families who have or may have a rare disease(s) may take part in the DCP.

Participants 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 legal 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 participant.

**Do I have to take part in the DCP?**

Taking part in the DCP is voluntary. This program is for research purposes only. The only alternative is to not participate in this program.

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. You may choose to not participate or you may withdraw from the program for any reason without penalty or loss of benefits to you.

**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.

It is likely that in the future, DCP will expand to include the collection of biosamples such as saliva or blood. At that time, RARE-X would request an additional consent to take part in that process.

**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 people with similar issues or, if you choose, other study participants. 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 studies unless an Institutional Review Board (IRB) has reviewed them.

An Institutional Review Board, is a type of committee that reviews research studies and methods to help protect the rights and welfare of study participants. 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 study 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 (study drugs, study devices, or other study 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 study 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.

Any new important information that is discovered during the program and which may influence your willingness to continue participation in the program will be provided to you.

**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 data 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 study staff who receive special training will have access to your identified data and only for authorized purposes.
- All RARE-X study 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.

*This research is covered by a Certificate of Confidentiality from the National Institutes of Health.*

*Certificates of Confidentiality protect the privacy of research participants by prohibiting the release (disclosure) of identifiable, sensitive information (data) to anyone not connected to the research except when the subject consents or in a few other specific situations.*

What that means is that the researchers with this Certificate **may not disclose or use** information, documents, or biospecimens that may identify you/the participant in any civil, criminal, administrative, legislative, or other action, suit, or proceeding, or be used as evidence, for example, if there is a court subpoena (i.e. divorce proceedings or criminal investigations, etc), unless you have consented for this use.

Information, documents, or biospecimens protected by this Certificate cannot be disclosed to anyone else who is not connected with the research except, if any of the following:

1. there is a federal, state, or local law that requires disclosure, such as those laws that require reporting of child abuse or neglect, harm to self or others, and communicable diseases, for instance.
2. you have consented to the disclosure, including for the participant's medical treatment
3. it is used for other scientific research, as allowed by federal regulations protecting research participants.

The Certificate **cannot be used** to refuse a request for information from any governmental agency sponsoring/funding the project that is needed for auditing or program evaluation. Neither can the Certificate be used to refuse a request for information that must be disclosed in order to meet the requirements of the federal Food and Drug Administration (FDA).

You should understand that a Certificate of Confidentiality does not prevent you from voluntarily releasing information about yourself/the participant or your/the participant's involvement in this research. If you want your/the participant's research information released to an insurer, medical care provider, or any other person not connected with the research, you must provide consent to allow the researchers to release it.

The Certificate of Confidentiality will not be used to prevent disclosure for any purpose you have consented to in this informed consent document or agreed to as part of data collection (see "How can I choose who to share the participant's data with?" part of this consent form).

### **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.



As part of this research, you may be required to use one or more of the following to collect research data: a phone or web app/ site, online/electronic questionnaires/surveys, or a device that tracks information about you.

While using these electronic tools, information about you may be collected and shared with the researchers or people outside of the study. This data might include personal health information, location, call logs, text message history, web browsing history, or social media use. A complete description of the data collection and data sharing for an app, online/electronic questionnaires/surveys, or device can commonly be found in the Terms of Use, End User License Agreement, or Privacy Policy. If you would like to read these documents, request a copy or instructions about how to access this information from the study staff. While the Terms of Use, End User License Agreement, or Privacy Policy may include statements limiting your rights if you are harmed as a result of your use of the app, online/electronic questionnaires/surveys, or device in this study, you do not release the investigator, sponsor, institution, or agents for responsibilities from mistakes. You also do not waive any of your rights as a research subject.

Some people feel uncomfortable when answering questions about the quality of their life. Though it is always better to have fully completed questionnaires, you do not need to answer any questions that make you feel uncomfortable.

There may be risks that are unknown.

**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 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.

The principal investigator or the sponsor can stop your participation at any time without your consent for the following reasons:

- If you fail to follow directions for participating in the study;
- If it is discovered that you do not meet the study requirements;
- If the study is canceled; or
- For administrative reasons.

### **Who would I contact about this study?**

During the study, if you have questions, concerns or complaints about the study, please contact the principal investigator at the telephone number listed on the first page of this consent document.

An institutional review board (IRB) is an independent committee established to help protect the rights of research participants. If you have any questions about your rights as a research participant, and/or concerns or complaints regarding this research study, contact:

- By mail:  
Study Subject Adviser  
Advarra IRB  
6100 Merriweather Dr., Suite 600  
Columbia, MD 21044
- or call **toll free:** 877-992-4724
- or by **email:** [adviser@advarra.com](mailto:adviser@advarra.com)

Please reference the following number when contacting the Study Subject Adviser:  
Pro00049000.

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 study 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/study.

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.

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Printed Name of Participant

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Signature of Participant

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Date of Signature

**WITNESS SIGNATURE FOR PARTICIPANTS WHO CANNOT READ**

The study participant has indicated that he/she is unable to read. The consent document has been read to the participant by a member of the study staff, discussed with the participant by a member of the study staff, and the participant has been given an opportunity to ask questions of the study staff.

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Printed Name of Impartial Witness

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Signature of Impartial Witness

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Date of Signature