RARE-X DATA COLLECTION PROGRAM (DCP)
RESEARCH CONSENT FORM
LEGALLY AUTHORIZED REPRESENTATIVES (LAR)

Title:	RARE-X Data Collection Program
Principal Investigator:	Vanessa Vogel Farley consent@rare-x.org

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 you are the legally authorized representative of a patient who has 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 the patient's health, health history, treatment and care, and the effect of having a rare disease on the patient's household. The DCP will store the patient's 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 the patient's 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 the patient's. However, neither you nor the patient

may not directly benefit from 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 the patient. *(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 have stated that you are the legally authorized representative of a patient who is not able to give consent. You are being asked as the patient's legally authorized representative to give consent on behalf of the patient to take part in the DCP because you stated the patient has or may have a rare disease.

The information below may help you decide if you want to give consent for the patient to take part in the DCP and what you will have to do if you decide that the patient will 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, the patient's 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 patients/participants, and caregivers may be asked to update their data to improve rare disease research.

You can stop the patient's participation in DCP at any time. *(See the 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.

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.

Does the patient have to take part in the DCP?
Taking part in the DCP is voluntary.

You can choose not to permit the patient take part at all. You can choose to allow the patient to 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 the patient's medical care. There will be no penalty to the patient.

What will I have to do if I permit the patient to 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 the patient's health, health history, treatment and care, and the impact of having a rare disease on their household.

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

The DCP collects and stores data. At this point, we do not expect patients who are unable to consent to take any action.

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 the patient's data be used, and for how long?
The patient's 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 the patient's 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 the patient's name, date of birth, or other personal data on it that a person could use to identify the patient easily.
- De-identified data has had the patient's name and other personal identifiers removed from their data and replaced with a code to keep their privacy.

Who can I choose to share the patient's *identifiable* data with, and what will they do with it?
You may choose to share the patient's *identifiable* data with qualified patient organizations associated with the patient's 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 the chance to share the patient's identifiable data this way because RARE-X understands the importance of patient community/organization support in rare disease.

Who can I choose to share *de-identified* data with, and what will they do with it?
You can choose to share the patient's *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 the patient to take part in any specific study they do in the future. This process allows the patient's data to be used frequently by many researchers.

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

With your permission, we will share the patient's 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 the patient. 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 the patient's data with?

RARE-X knows that you may change your mind about the people and organizations that you want to share the patient's 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 the patient's data for:

a. **Health/Medical/Biomedical Research**
Researchers can access and use the patient'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 patient's data may be used for fewer types of research studies than if you choose General Research.***

- The patient'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 patient's 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 the patient's data is used for research. You don't have to put any further limits on how the patient's data is used, but if you would like to, you can choose restrictions such as:

A. Not allowing the patient's data to be used for commercial/for-profit research purposes

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

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

<u>Who else is the patient's data shared with?</u>

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

For example, the patient's 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 the patient 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 the patient 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 the patient 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 the patient's possibly taking part in a research study and/or clinical trial.

Will the patient benefit from taking part in the DCP?
The patient is 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 the patient's rare disease.

Will participants be paid for taking part in the DCP?
No. Participants will not be paid for taking part in the DCP.

Neither you nor the patient will not be paid if new drugs, tests, devices, or commercial/for-profit products are created due to research on the patient's data.

Will I find out the results of the research?
No. You will not receive individual results from research done using the patient's 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 the patient's symptoms and care options.

How will data about the patient be kept confidential?
RARE-X will remove the patient's name and other personal identifiers and replace them with a special code number before sharing their data with others. RARE-X will encrypt (scramble) the patient's data when stored, so it is hard for people without permission to read it.

Here are some other steps we take to keep the patient's data confidential:

- Any researchers using the patient's data must sign an agreement promising that they will not try to find out who the patient is.
- Only a few members of the RARE-X staff who receive special training will have access to the patient's 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 the patient's 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 the patient.

An accidental release of the patient's data could possibly identify the patient or their family. We believe the chances that these things will happen are 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 the patient's rare disease community and/or genetics could connect de-identified data back to the patient. Although they may know the data is about the patient, they are bound by their agreement with RARE-X not to contact you or the patient or publish data that identifies the patient.

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

Will it cost the patient anything to take part in the DCP?
No. There are no costs for the patient to take part in the RARE-X DCP.

Can I access the patient's data from the DCP?
RARE-X will allow you to see and print the data you put in the DCP.

Can the patient quit the DCP?
Yes, you can stop the patient's taking part in the DCP at any time for any reason.
If you decide that the patient should quit, you will be offered some choices about whether RARE-X keeps or deletes the patient's data.

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

If you decide that the patient should quit the DCP, you can choose to:

- Allow the DCP to keep the data RARE-X has already collected about the patient. RARE-X will continue to share it with researchers for future research studies; or

- Require the DCP to delete all the patient's data, including the special code assigned to their data. However, if you choose to delete the patient's data, we cannot take back any of their 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 the patient may be a good fit for possible studies or clinical trials.
    - You may ask RARE-X to transfer the patient's data to another data collection platform before we delete it.
    - If you delete the patient's 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 the patient's rights as a participant in the DCP and you are not able to resolve your concerns or complaints with the Principal 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. A review by Advarra does not mean that the DCP is without risks.

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Consent Documentation

I understand and agree that:

- I am voluntarily providing the patient's data to the RARE-X DCP as a place to put their data for use in research.
- I may choose to stop the patient's participation at any time. If I stop the patient's participation, their 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 stop the patient's participation, it will have no impact on their medical care.
- The DCP may share the patient's de-identified data for the types of research that I choose. These research studies may happen in the future.
- The patient 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 the patient qualifies to be part of a clinical trial.

My signature below indicates:

- I understand that my obligation as a legally authorized representative is to try to determine what the patient would decide if the participant were able to make such decisions or, if the patient's wishes cannot be determined, what is in the patient's best interests.
- 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 give permission for

the patient 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 for the patient to take part in the DCP.
- I agree to allow the collection, use, and sharing of the patient's data as described above.
- By signing this form, I do not give up any of my or the patient's legal rights.
- I will get a signed copy of this Consent Form.

_____

Name of Participant

_____

Name of Authorized Personal Representative

_____ _____

Signature of Authorized Personal Representative Date

Indicate the legal representative's authority to act for the individual:

☒ Spouse

☒ Parent of a child over 18

☒ Adult child (18 years of age or over) for his or her parent

☒ Adult sibling (18 years of age or over) for his or her sibling

☒ Grandparent

☒ Adult grandchild (18 years of age or over) for his or her grandparent

☒ Individual with power of attorney

☒ Guardian appointed to make medical decisions for individuals who are incapacitated