



**CONSENT TO ACT AS A STUDY PARTICIPANT IN THE GLOBAL PRADER-WILLI  
SYNDROME REGISTRY  
AND TO SHARE  
DATA FOR FUTURE RESEARCH PURPOSES**

Consent for a person with a Legally Authorized Representative (LAR) who is unable to consent for him or herself

**Title: Global Prader-Willi Syndrome Registry**

**Principal Investigator: Theresa Strong, Ph.D.**

**Experimental Research Subjects Bill of Rights**

**California law, under Health & Safety Code Section 24172, requires that any person asked to take part as a subject in research involving a medical experiment, or any person asked to consent to such participation on behalf of another, is entitled to receive the following list of rights written in a language in which the person is fluent. The list includes the right to:**

- 1. Be informed of the nature and purpose of the experiment.**
- 2. Be given an explanation of the procedures to be followed in the medical experiment, and any drug or device to be utilized.**
- 3. Be given a description of any attendant discomforts and risks reasonably to be expected from the experiment.**
- 4. Be given an explanation of any benefits to the subject reasonably to be expected from the experiment, if applicable.**
- 5. Be given a disclosure of any appropriate alternative procedures, drugs or devices that might be advantageous to the subject, and their relative risks and benefits.**

- 6. Be informed of the avenues of medical treatment, if any, available to the subject after the experiment if complications should arise.**
- 7. Be given an opportunity to ask any questions concerning the experiment or the procedures involved.**
- 8. Be instructed that consent to participate in the medical experiment may be withdrawn at any time and the subject may discontinue participation in the medical experiment without prejudice.**
- 9. Be given a copy of the signed and dated written consent form.**
- 10. Be given the opportunity to decide to consent or not to consent to a medical experiment without the intervention of any element of force, fraud, deceit, duress, coercion, or undue influence on the subject's decision.**

As the guardian or authorized representative for the Study Participant, we encourage you to discuss the registry with the Study Participant to the extent compatible with their understanding. If you or the Study Participant has any questions about the registry, please feel free to contact the registry staff.

### **Definitions**

For the purpose of this Consent form, "Study Participant" refers to the person diagnosed with Prader-Willi Syndrome ("PWS"). Registry information will be collected on patients who are diagnosed with PWS. "You" refers to the person providing the information, who may be a family member or guardian who is legally responsible for the care and health of the patient. The reference to "we" in this document refers to the research organization PWS.

### **Research Data Sharing**

A patient Registry collects and stores patient medical information, family history and other relevant information for use in medical research.

On behalf of the Study Participant, you are invited to provide personal and medical information in an online questionnaire format by answering surveys and uploading medical information. This data and related information is to be stored (banked) in a research data database or Registry where it can be used for future research projects. The data collected in this Registry will be used by researchers to study PWS with the following objectives and goals:

- 1) To characterize and describe the PWS population as a whole and to gain a better understanding of the spectrum of clinical variants in individuals with PWS of all stages.

This includes but is not limited to collecting information on: diagnosis, treatment, medical history, socio-economic environment and outcomes.

- 2) To understand the changes of PWS over a lifetime as well as to gain information on clinical practice patterns and variations over the course of treatment.
- 3) To facilitate the development of best practice and management guidelines and recommendations to optimize care, improve quality of life and outcomes and standards of care.
- 4) To provide information regarding ongoing research studies and clinical trials. You, on behalf of the Study Participant, may consent to be contacted by researchers for recruitment into Institutional Review Board (IRB) approved studies.

In order to decide whether or not you, on behalf of the Study Participant, wish to allow the Study Participant's data to be used for future research, you should know about the risks and benefits to make an informed decision. This form gives you information about this Registry and how the data may be used. Once you understand the data collection and sharing process, you will be asked if you, on behalf of the Study Participant, wish to take part; if so, you will be asked to sign this form.

### **How Study Participant Data Gets Into this Registry**

The data obtained from you, on behalf of the Study Participant, for this Registry will be sent to the data bank along with the following information about the Study Participant, which will be entered into a computer (the database) that is used for research purposes:

Data entered into this Registry includes, but is not limited to:

Name, date of birth, diagnosis, grades in school and graduation information, treatment, past and proposed, general medical information, blood level results.

### **How Study Participant Data Is Stored and Used for Future Research**

The goal of the Registry is to share medical and other PWS-relevant information with scientists and other researchers, while protecting the Study Participant's privacy.

When the Study Participant's information is stored, we are careful to try to protect the Study Participant's identity from discovery by others. Strict security safeguards are in place to reduce the chance of misuse or unplanned release of information.

Whenever possible, the researchers will use the Study Participant's information in a de-identified manner. De-identified means that the researchers will use the Study Participant's information without knowing his/her identity. In some cases, they may use some identifying information about the Study Participant for research purposes, subject to an approval process through the Global Prader-Willi Syndrome Registry Advisory Committee. At times, the researchers will use the information with a code, instead of the Study Participant's name; the code would allow results of the research to be linked back to the Study Participant.

## **Risks and Inconveniences**

There are no physical risks to you or the Study Participant for allowing the Study Participant's data to be stored or used in future research studies. In the unlikely event that someone outside the research team views the Study Participant's information however, there is a risk of breach of confidentiality (see the Confidentiality section for an explanation of how the information is protected). In the event that there is a breach in this Registry's computer system, you will be notified.

There is a risk that the Study Participant's information could be misused. The chance of this happening is very small. We have protections in place to lower this risk. There can also be a risk in uncovering genetic information. New health information about inherited traits that might affect the Study Participant or his/her blood relatives could be found during a research study. Very rarely, employers, health insurance companies, and others could misuse health or genetic information. There is a federal law called the Genetic Information Nondiscrimination Act (GINA) that, in general, makes it illegal for health insurance companies, group health plans, and most employers, except those with fewer than 15 employees, to discriminate against the Study Participant based on his/her genetic information. However, it does not protect the Study Participant against discrimination by companies that sell life insurance, disability insurance, or long-term care insurance. There may be other risks we are not aware of at this time.

## **Benefits**

Participation in this Registry is voluntary and may not result in direct benefit to you (or the Study Participant) personally, medically or financially. However, we hope the research results will help people with PWS in the future. We hope that the information we learn in future research studies will increase our knowledge of human health and that this information will lead to better prevention, diagnoses and treatments in the future.

## **Alternative**

On behalf of the Study Participant, you do not have to participate in this study so your alternative is to say "no."

## **Economic Considerations**

Neither you nor the Study Participant will receive any payments for allowing the Study Participant's information to be stored in this Registry.

The Study Participant's information will only be used for research. Should one of the researchers use the Study Participant's medical information to develop a commercial product, neither you nor the Study Participant should expect to receive any financial gain from these efforts.

## **Confidentiality**

All identifiable information that is obtained in connection with this Registry will remain confidential. This Registry aims to share detailed medical and other information with researchers while protecting the Study Participant's privacy. One way the Registry protects the Study Participant's information is to remove his/her name, address and other "identifying" information from our medical information before providing it to researchers. This information is "de-identified" because it has had all the personal identifiers removed including the Study Participant's name, address, or other information that identifies the Study Participant or the Study Participant's family. The Study Participant's Registry information will be labeled with a code number and stored on secured computers and servers and protected with encryption and passwords. Only authorized people who work in this Registry will have access to the key to the code and will be able to identify you or the Study Participant if needed. When the results of future research are published or discussed in conferences, no information will be included that would reveal your or the Study Participant's identity unless your specific consent is obtained. This Registry will not share your or the Study Participant's identifiable information with anyone outside this Registry (unless you give permission to share it). Approved researchers and clinicians will be allowed to see only the de-identified information. Approved researchers and clinicians may use de-identified information to conduct research, including research unrelated to PWS. They may also search the de-identified information to find patients for their studies. If a patient looks like a good match for a researcher's study, the Global Prader-Willi Syndrome Registry or Registry's agent will contact you if you have given us permission to contact you about research studies.

Your and the Study Participant's de-identified PWS Registry information may be shared with other databases such as the Global Rare Disease Patient Registry Data Repository (GRDR). This will allow more researchers to use the information to do research. The de-identified information collected and compiled by this Registry belongs to the PWS community. The Global Prader-Willi Syndrome Registry is the guardian of the information contained within this Registry.

Third parties may seek access to data in the Global Prader-Willi Syndrome Registry. Third parties may include, but are not limited to, researchers or companies conducting retrospective studies or conducting research and/or clinical trials on new therapies. Third parties will only be granted access to registry information upon review and approval of the Advisory Board. Such approvals shall be obtained prior to providing access to registry information; shall be based upon considerations of scientific quality and validity; shall be granted for research studies related to PWS; and shall be documented. Third parties seeking access to registry information for retrospective studies will only have access to anonymous information identifiable only by the assigned unique identifier. Third parties seeking access to registry information for the purpose of determining eligibility for participation in a research study or clinical trial must demonstrate evidence of IRB approval of the research study for which access is being requested

You will be asked to update your and the Study Participant's Registry information at least once per year by completing questionnaires, called surveys. You will be asked to update some surveys more frequently. Based upon your preference for how you would prefer to be contacted, this Registry will contact you to remind you to update your and the Study Participant's data. The

Registry may also ask you to upload the Study Participant's genetic test results and other blood work or test results. Your Registry account can be updated whenever there is a change in the Study Participant's health, change in treatment, or new symptom.

Research results will not be returned to you or the Study Participant's doctor. If research results are published, your and the Study Participant's names and other personal information will not be given.

Representatives from the National Institutes of Health (NIH), U.S. Food and Drug Administration (FDA) or National Organization for Rare Disorders (NORD) and from Hummingbird IRB may inspect study records during auditing procedures to be sure that this Registry is being protected according to regulations and policies. However, these organizations and the individuals acting on their behalf are required to keep all donor information confidential.

### **Voluntary Participation and Withdrawal**

Participating in this Registry is voluntary. Refusing to participate will involve no penalty or loss of benefits to which you/the Study Participant are otherwise entitled, (such as health care outside the study, the payment for health care, and health care benefits). On behalf of the Study Participant, you are free to choose not to donate the Study Participant's data to research and if the Study Participant does become a donor you are free to change your mind at any time, on behalf of the Study Participant, but the researchers may still use the information collected before you changed your mind in order to complete the research that has already started. Information that has already been shared with the GRDR or sent to a researcher for a specific study prior to your request for removal cannot be retrieved or removed. The researchers will anonymize the data by removing and destroying all identifiers and links to identifiers so that it cannot be associated with you or the Study Participant, but the researchers will not destroy the data.

If you choose not to allow the Study Participant's information to be stored and used, or if you withdraw your permission, this will involve no penalty or loss of benefits to which you and/or the Study Participant are otherwise entitled. This will not harm your or the Study Participant's relationship with the Study Participant's doctors who may be involved with this Registry or PWS.

This form and your permission will never expire unless you change your mind and withdraw it. You may withdraw your permission by telling the Registry staff in writing to:

Jessica Bohonowych  
Foundation for Prader-Willi Research  
5455 Wilshire Blvd., Suite 2020  
Los Angeles, CA 90036  
registry@fpwr.org

### **Participation of minors and adults unable to consent**

Registry information will be collected on patients who are diagnosed with a form of PWS. Patients over the age of 18 who understand the consent form and legally provide their own consent (and thus do not have a legal guardian) are eligible to join this Registry on their own. Otherwise, the legal guardian or parent of the patient must sign the consent form for the patient to join. When a minor Study Participant becomes 18 (and if they are able), consent will be obtained directly from them for continued participation.

### **Privacy Rights**

The health-related information that we gather about the Study Participant in this study is personal. The researchers are required by law to protect the privacy of information known as protected health information (PHI). All reasonable efforts will be made to protect the confidentiality of the Study Participant's PHI, which may be shared with others to support this research, to conduct public health reporting, and to comply with the law as required. Despite these protections, there is a possibility that information about you or the Study Participant could be used or disclosed in a way that it will no longer be protected.

By signing this form, you give permission on behalf of the Study Participant for this Registry to store and use and/or disclose the information in this Registry database. You have a right to refuse to sign this form. The Study Participant's health care outside the study, the payment for his/her health care, and his/her health care benefits will not be affected if you do not sign this form. You do not give up any of your or the Study Participant's legal rights by signing this form.

### **User Submitted Content**

NORD and the Global Prader-Willi Syndrome have the right to edit and delete any portion of the Website.

### **Use of the Data/Information Contained on the Website**

All data collected by NORD and Global Prader-Willi Syndrome may be combined and shared with other NORD organizations or outside researchers. You are responsible for the accuracy of the data and its content. NORD and its affiliated organization will protect the data to the extent possible and as described in this consent.

### **Consent from International Users of the Platform**

For persons living outside the United States who choose to share information about themselves (or about a person for whom they serve as a Legally Authorized Representative), the same protections for privacy and confidentiality are offered as in the United States. By signing this consent, you acknowledge that you are disclosing information that would otherwise be private. Privacy laws in your country may have different protections than those provided in the United States.

### **GETTING ANSWERS TO YOUR QUESTIONS ABOUT THE STUDY**

We have used some technical terms in this form. Please feel free to ask about anything you don't understand and to consider sharing your/the Study Participant's information and the consent form carefully – as long as you feel is necessary – before you make a decision. Please contact the Registry staff as listed above.

An Institutional Review Board, for the purpose of protecting your/the Study Participant's rights, has reviewed this Registry. An institutional review board is a group of people who are responsible for protecting the rights and welfare of people who participate in studies. For questions about the rights of the Study Participant in this Registry or to discuss other study related concerns or complaints with someone who is not part of this Registry team, you may contact Hummingbird IRB, One Broadway, 14<sup>th</sup> Floor, Cambridge, MA 02142 at 1-855-447-2123 (toll free). Review and approval of this Registry by Hummingbird IRB is not an endorsement of this Registry or its outcome.

Do not sign this form unless you have had a chance to ask questions and have received satisfactory answers to all of your questions.

Because of the way data is collected and saved, to participate in this study, the participant or person agreeing must be willing to agree to permitting data to be shared as described. If you cannot agree to this, please do not participate in the study.

Please reference the following sections of the consent form for details on how data collected in this study will be used.

- How Study Participant Data gets into the registry
- How Study Participant Data is stored and used for future research
- Confidentiality
- Voluntary Participation and Withdrawal
- Privacy Rights

### **Authorization**

I have read this Consent and Authorization Form to donate data for future research purposes and have decided to donate the Study Participant's data to the Global Prader-Willi Syndrome Registry. The general purposes of registry participation, details of my and the Study Participant's involvement and possible hazards and inconveniences have been explained to my satisfaction. I understand that I will receive an electronic copy of this consent/authorization form.

If appropriate, select the category that best describes the Study Subject's Legally Authorized Representative who signed above:

- Court-appointed guardian
- Durable power of attorney
- Health care proxy
- Family member / next of kin (describe relationship below):



Relationship to Study Subject: \_\_\_\_\_

**CHECK all that apply (Yes or No)**

I give permission on behalf of the Study Participant to provide research data to the Global Prader-Willi Syndrome Registry only for the purposes described above. Yes \_\_\_ No \_\_\_

I give permission on behalf of the Study Participant to provide research data **that has been de-identified** to the Global Prader-Willi Syndrome Registry for future, undefined research. Yes \_\_\_  
No \_\_\_