**UNIVERSITY OF WASHINGTON CONSENT FORM**

**Glycosaminoglycan Levels in Newborn Dried Blood Spots from MPS Patients**

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*If you are a parent providing permission for a child, “you” in this form means your child*

**Researchers’ statement**

We are asking you to be in a research study. The purpose of this consent form is to give you the information you will need to help you decide whether or not to be in the study. Please read the form carefully. You may ask questions about the purpose of the research, what we would ask you to do, the possible risks and benefits, your rights as a volunteer, and anything else about the research or this form that is not clear. When all your questions have been answered, you can decide if you want to be in the study or not. This process is called “informed consent.” We will give you a copy of this form for your records. You can direct your questions to the staff member of the National MPS Society who sent you this consent form.

**PURPOSE OF THE STUDY**

Screening of newborn infants for diseases that can be treated very early in life is now done routinely in many countries, including the United States. Screening does not diagnose an infant. Screening identifies those infants at increased risk (those more likely) to be affected based on substances found in their blood shortly after birth. By identifying those infants at increased risk, testing to confirm or rule out the diagnosis can be done. Then, if the child is affected, treatment can be started before major health problems happen.

Diseases can occur when the body is missing an enzyme (a type of protein), and the body cannot break down certain substances correctly. As a result, these substances can be stored throughout the body. One group of disorders that occur due to missing or non-working enzymes are called “lysosomal storage diseases” or “LSDs.” (The lysosome is a part of the cell which contains many enzymes. If one is not working, then material is stored in the lysosome and the body).

New treatments are becoming available for LSDs, so it is important to diagnose individuals with these diseases as early as possible. At the University of Washington, new tests are being developed to use for newborn screening for LSDs.

Newborn screening for a subset of LSDs called Mucopolysaccharidoses is carried out by measuring the amount of residual lysosomal enzymatic activity in newborn dried blood spots. When the enzyme is below the cutoff for the screen, additional tests may be performed to determine how likely it is for the newborn to develop one of the Mucopolysaccharidoses syndromes. In our new study we are exploring whether the level of a biomarker called glycosaminoglycan is elevated in patients that went on to develop a Mucopolysaccharidosis syndrome. We need to evaluate the biomarker level in newborn dried blood spots since that is what will be available in a newborn screening program. We are thus asking you to participate in our study by requesting a stored dried blood spot from your state's newborn screening lab. The lab will send the dried blood spot to Professor Gelb's lab at the Univ of Washington so that his lab can measure the level of biomarkers. We also want to see if there is a correlation between the level of the biomarker and the age of onset of symptoms for the Mucopolysaccharidosis syndrome. This correlation may be useful someday in predicting the severity of the disease in newborns who test postive in newborn screening. We want to focus on the newborn dried blood spot since that is what is most relevant for newborn screening.



**STUDY PROCEDURES**

If you choose to take part in this study, you would complete your state's form for request of a stored dried blood spot from you the patient or your affected child. We are providing you with a partially completed form from your state's newborn screening lab. You would also complete the patient questionaire form, and send this along with the completed dried blood spot release form and the signed consent form to the staff member of the National MPS Society.

Professor Gelb will never know the identity of the patient, all dried blood spots that his lab will receive will have a code number. The Natioanal MPS Society will have a list of patient names linked to their code and also linked to the questionaire that you will fill out to provide important information about the patient. This questionaire information will be provided to Professor Gelb so that he can interpret the data in his research study. Again, Professor Gelb's research team will never know the identity of the patient.

The only test the Gelb lab will perform on the dried blood spot is the measurement of the glycosaminoglycan biomarker.

**RISKS, STRESS, OR DISCOMFORT**

You may find that participating in a research study is an invasion of your privacy. We will make every effort to keep all of the information we collect for this study about you safe. More information of confidentiality is described under the CONFIDENTIALITY OF RESEARCH INFORMATION section of this form.

**ALTERNATIVES TO TAKING PART IN THIS STUDY**

Taking part in this study is voluntary. You do not have to take part if you do not want to.

**BENEFITS OF THE STUDY**

While you will not directly benefit from this study, we hope that the results of this study will provide important new information that can be used to improve the knowledge gained from newborn screening related to lysosomal storage diseases.

**SOURCE OF FUNDING**

The study team and/or the University of Washington is receiving financial support from the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), National Institutes of Health (NIH).

**FINANCIAL INTEREST**

None.

**CONFIDENTIALITY OF RESEARCH INFORMATION**

The only individuals who will know the identity of the patients are the administrative staff of the National MPS Society. They will keep these names and code numbers in a secure place.

We have a Certificate of Confidentiality from the National Institutes of Health. This helps us protect your privacy. The Certificate means that we do not have to give out identifying information about you even if we are asked to by a court of law. We will use the Certificate to resist any demands for identifying information.

We can’t use the Certificate to withhold your research information if you give your written consent to give it to an insurer, employer, or other person. Also, you or a member of your family can share information about yourself or your part in this research if you wish.

There are some limits to this protection. We will voluntarily provide the information to: • a member of the federal government who needs it in order to audit or evaluate the research;

• individuals at the University of Washington, the funding agency, and other groups involved in the research, if they need the information to make sure the research is being done correctly;
• the federal Food and Drug Administration (FDA), if required by the FDA;

• Local authorities, if we learn of child abuse, elder abuse, or the intent to harm yourself or others.

If we publish the results of the study in scientific journals or present them at scientific meetings, we will not include any information that could identify you.

**OTHER INFORMATION**

You may refuse to participate in any or all portions of this study.You are also free to withdraw from this study at any time without penalty or loss of benefits to which you are otherwise entitled.

You will not be charged for study-related procedures. You will not be paid for taking part in this study.

If you have any questions about the study, contact the staff person of the National MPS Society. Do not contact Professor Gelb, since he is not to learn the identities of the families.

I consent to the research study described in this consent form.

Printed name:\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

Signature:\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

Date:\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_