Indiana University Informed Consent Statement and Authorization for Research

Evaluating impact of CFTR modulators on pancreatic function
Protocol#24152

This Consent and Authorization Form will give you information about this study to help you decide whether you want your child to participate. It is your choice whether you want your child to be in this research study. Please read this form and ask any questions before agreeing to be in this study.

If you are reading this consent to allow your child to participate in this study, when reading this form, please read "you/your" as "your child."

Why is This Study Being Done?

Cystic fibrosis (CF) is a genetic disorder that primarily affects the lungs but also impacts the liver, pancreas, and digestive system. Recent reports indicate that CF medications, known as CFTR modulators, have been shown to improve or even restore pancreatic function in some people. To date, about 80 people have reported recovery of pancreatic function after starting CFTR modulators. This study aims to explore the recovery of pancreatic function in more detail. We will monitor levels of CFTR modulators to better understand how they affect pancreatic function and their absorption into the body. We will analyze these results alongside other medical information about you. Our goal is to use these findings to better understand why some people regain pancreatic function. If pancreatic function is restored, you may no longer need to take pancreatic enzyme replacement therapy (will be referred to as "enzymes" in this form). Pancreatic enzyme replacement therapy is currently an FDA approved therapy for loss of pancreatic function. Should you stop taking your enzymes, we will monitor for symptoms of malabsorption following the discontinuation of enzymes.

We are asking if you would like to participate in this study because you have pancreatic insufficiency secondary to cystic fibrosis and are currently taking a CFTR modulator medication.

The study is being conducted by Audra Rougraff, MD, Molly Bozic, MD, Don B. Sanders, MD, Emma Tillamn, PharmD, PhD, and Karen Maguiness, RD who are affiliated with Riley Hospital for Children at Indiana University Health.

What Will Happen During the Study?

If you agree to participate in the study, you will need to:

- **Consent:** A researcher will explain the study to you, and you can ask questions before agreeing to participate.

- **Survey:** You will complete an online survey to confirm your history and assess symptoms of your GI tract and nutrition. This survey should ideally be completed every 3 months, preferably during your clinic appointment. This will need to be completed 1 or 3 times during the study.
- **Blood Work:** You will need to submit a blood sample to measure CFTR modulator levels. Please avoid taking your CFTR modulator before the blood draw, as this can affect the lab results. Additionally, blood work will be collected to assess vitamin levels and liver function. We will aim to coordinate all blood work with your clinic appointment. This will need to be completed 1 or 2 times during the study.
- **Stool Sample:** You will need to submit a stool sample to evaluate pancreatic function. This will need to be completed 1 or 2 times during the study.
    - If your stool sample reveals a **fecal elastase amount ≥ 200**, you will stop your pancreatic enzyme replacement therapy.
        - After you stop your enzymes, you will need to repeat the online survey after 3 months. After 6 months, you will need to submit another stool sample, blood work, and complete the online survey. This will be the last step of the study.
        - If you experience symptoms of malabsorption (diarrhea, bloating, cramping, gas, nausea, vomiting, tiredness), at any point before the 6th month specimen collection, you will be asked to restart the enzymes and your participation in the study will be completed.
    - If your stool sample reveals a **fecal elastase amount ≤ 199**, you will continue your pancreatic enzyme replacement therapy. This will be the last step of the study.
- **Medical Records Review:** We will review your medical records to gather information about your cystic fibrosis, associated diagnoses, and laboratory test results.
- **Study Duration:** You will remain enrolled in this study for up to 6 months. After the study is completed, we recommend every 3-month follow-up with your pediatric pulmonologist if you are to continue off of enzymes.

What Are the Risks of Taking Part in the Study?

- You may experience minor discomfort or soreness at the site where blood is drawn. We will limit the blood work for this study to about 2 times per year.
- You may become uncomfortable while filling out the online survey. You can withdraw from the study at any time.
- If you stop taking your enzymes, there is a risk that you may experience malabsorption symptoms and/or lose weight. We will check for malabsorption every 3 months, but please inform us if you notice any signs or symptoms before your next appointment. If we suspect you are experiencing malabsorption symptoms, you will be asked to restart your enzymes and your participation in the study will end.
- There is a potential risk to your privacy when participating in a research study, but all measures will be taken to minimize this risk as described above.

- Some data obtained for research use will be labeled with your name; however, this information will stay only at Riley Hospital for Children and stored as password-protected files or in a secure location.
- As with any research study, there may be additional risks that are unknown or unexpected.

Who Will Pay for my Treatment if I am Injured?

If you experience an injury or illness from participating in the study, necessary medical treatment will be provided and billed as part of your medical expenses. Any costs not covered by your medical insurance will be your responsibility. However, signing this form does not waive any of your legal rights if you are injured.

What Are the Benefits of Taking Part in the Study?

By participating in this study, you will learn information about the function of your pancreas. Additionally, you may find out that you have regained pancreatic function. If your pancreas does regain function, you will stop taking your enzymes.

Will I be Paid for Participating?

You will receive $25 for submitting research study specimens. You will be paid $12.50 for each blood sample and $12.50 for each stool sample that you submit. This payment will be added to a reloadable credit card. You can earn up to $50 during the study.

Will it Cost Me Anything to Participate?

Participating in this study will not lead to any costs associated with this research study. You will not be billed for the cost of the stool studies and bloodwork required for this study. You should check your medical bills to be sure you are billed correctly.

How Will My Information be Used?

The study team will collect information about you from your medical records. This may include identifiable information such as your name, contact information, and medical record number. Information from your medical records will be used to ensure you meet the criteria for participation in the study, gather details about your medical history for inclusion in the research data, review results of prior and new medical tests, monitor your health in the future to help answer our research questions, and inspect and/or copy your research records for quality assurance and data analysis. The information released and used for this research will include:

- Demographic information: age, gender, race, ethnicity
- Medical history: physician progress notes from inpatient and outpatient encounters
- Medication history
- Growth chart: weight, length, and/or height
- Laboratory and diagnostic tests: blood work and stool samples
- Radiology reports
- Operative reports from any previous surgeries

If you agree to participate, you authorize the following to disclose your medical record information:

- Indiana University Health
- Indiana University Health Physicians

The following individuals and organizations may receive or use your identifiable health information:

- The researchers and research staff conducting the study
- The Institutional Review Boards (IRB) or its designees that review this study
- Indiana University
- Data safety monitoring boards and others authorized to monitor the conduct of the study
- US or foreign governments or agencies as required by law
- State or Federal agencies with research oversight responsibilities, including but not limited to:
    - The United States Food and Drug Administration (FDA)

After your medical record information is released for purposes of this research study, your information may no longer be protected under federal privacy laws, such as HIPAA. However, your identifiable information will still be stored securely and used only as described in this consent form.

Information collected for this study may be used for other research studies or shared with other researchers conducting their own research studies. This may include sharing with researchers outside Indiana University and sharing with private companies. It may also include making the information available in public and private databases of research data so that other researchers can use the information to answer research questions.

If we share your information in this way, we will remove information that could identify you, such as your name and contact information, before any information is shared. Since identifying information will be removed, we will not ask for your additional consent for this sharing.

How Will My Information be Protected?

Confidentiality will be taken seriously throughout the study. We will do our best to keep your personal information private, but we cannot guarantee complete confidentiality. We will not share any information that we think could be used to identify you in publications about this study. However, your personal information may be shared outside the research study as described above and/or if required by law.

Who Should I Call with Questions or Problems?

For questions about the study or a research-related injury, contact the researchers Audra Rougraff MD or Molly Bozic MD at 317-944-3774. If you cannot reach the researcher or your doctor during regular business hours (i.e., 8 a.m. to 5 p.m.), please contact the Pediatric Gastroenterology doctor on-call by calling the Riley Hospital operator at 317-944-5000.

For questions about your rights as a research participant, to discuss problems, complaints, or concerns about a research study, or to obtain information or to offer input, please contact the IU Human Research Protection Program office at 800-696-2949 or at irb@iu.edu.

What if I Do Not Want to Participate or Change my Mind?

After reviewing this form and having your questions answered, you may decide to participate in the study. Alternatively, you may choose not to participate in the study. This decision is up to you. If you choose not to participate in this study or change your mind after signing this document, it will not affect your relationship with Indiana University or the medical care you receive from Riley Hospital for Children at Indiana University Health.

If you change your mind and decide to leave the study in the future, the study team will help you withdraw from the study. If you decide to withdraw, please let a member of the study team know.

If you choose to withdraw your authorization for use and disclosure of your protected health information, you must do so in writing by notifying Dr. Rougraff located at 705 Riley Hospital Dr, ROC 4210, Indianapolis, IN 46202 or at 317-944-3774. If you withdraw your authorization, you will not be able to continue in this study. However, even if you cancel this authorization, the research team, research sponsors, and/or the research organizations may still use information about you that was collected as part of the research project between the date you signed this document and the date you cancelled this authorization. This is to protect the quality of the research results. Otherwise, this authorization remains valid until the research ends and required monitoring of the study has been completed.

You will be told about new information that may affect your health, welfare, or willingness to stay in the study.

Agreement to be Contacted by Text and/or Email

We would like to communicate with you about this study by text message and/or email. We might use text or email to send you reminders about upcoming visits or appointments.

Text messaging and email are not secure methods of communication. The information sent over text or email, which may include sensitive or personal information, such as protected health information, could be accessed or read by someone other than you. If you would like us to communicate with you via text or email, please initial the lines below and provide the phone number(s) and/or email address(es) you would like us to use.

_____ I authorize the researchers to send me emails related to this research study

Email address for this communication: _____

_____ I authorize the researchers to send me text messages related to this research study

Phone number for this communication: _____

You can still participate in this study even if you do not want us to contact you by text or email.

Participant's Consent and Authorization

I have read through the consent form provided, and I agree to participate in this research study.

Printed Name of Participant: _____

Participant's Address: _____

City: _____ State: _____ Zip Code: _____

Participant's Signature: _____

<u>If consenting on behalf of your child participant:</u>

Printed Name of Parent: _____

Signature of Parent: _____Date: _____

<u>For children aged 15 – 17:</u>

Signature of Child: _____Date: _____

For research team:
Printed Name of Person Obtaining Consent: _____

Signature of Person Obtaining Consent: _____ Date: _____