Understanding the Spectrum of ENPP1 Deficiency and Acute ABCC6 Deficiency Through the Eyes of Patients and Parents; Burden of Illness Perspectives from Patients and Parents who Speak English, French or German

Frequently Asked Questions

1. Why is this study being done?

You are being asked to be in a research study or you are being asked as the legally authorized representative, parent, or guardian to permit the subject to take part in the research. This study will try to improve understanding of the experiences of patients diagnosed with ENPP1/ABCC6 deficiency (GACI / ARHR2) and capture the burden of disease and impact of ENPP1/ABCC6 deficiency (GACI or ARHR2) on your life. Detailed data will be collected from the perspective of patients with GACI / ARHR2 or their parent/caregiver regarding their experience. This information will be used to help identify factors important to those with GACI / ARHR2.

2. What are the study procedures? What are my responsibilities?

If you agree to take part in this study, you will be responsible for filling out a short online RSVP questionnaire and participating in a telephone interview that will be conducted by a staff member of Engage Health, Inc., a health research vendor. As part of the RSVP, you will answer a few questions to help us understand who you are, your ethnicity, and if you qualify for the research. You will also be responsible for providing a document that ties your name (or your child's name) to the diagnosis of any form of ENPP1/ABCC6 deficiency (GACI or ARHR2). Examples include a report of genetic testing for ENPP1/ABCC6 deficiency (GACI /ARHR2), a doctor's note, a school report or other documentation that you have on hand. We estimate that your time commitment to the RSVP will be approximately 20 minutes. You will need access to a computer and internet browser prior to the start of the interview.

If you qualify for the research, the telephone interview will be scheduled at a time that works best for you and can be conducted in English, French, or German. The interviewer will ask questions about the experiences surrounding burdens of the disease and how it has impacted your/your child's life. As you participate in the interview please know that there are no right or wrong answers. The interview is anonymous, and you will only be identified by a unique study number. If you do not qualify to participate in the research you will be notified.

We estimate that your time commitment for the RSVP questionnaire and the telephone interview will take about 60 minutes. The study will initially recruit 60 patients with 20 from each of three patient groups but there will be a minimum of 30 and a maximum of 90 participants.

3. What are the risks or inconveniences of the study?

Participating in this research study will not result in any clinical benefit to you. You will contribute to the understanding of this disease from your own experience and will help efforts to develop a treatment. This research study does not involve any risks. The time it takes to complete the RSVP survey and telephone interview may be a possible inconvenience.

We estimate that your overall time commitment will be approximately 60 minutes (20 minutes for the RSVP/survey and 40 minutes for the telephone interview). You will be told about any new information that might change your decision to be in this study.

Engage Health, the health research vendor conducting the study, will make every reasonable effort to protect the study data. Submitting personal data over the internet always involves some risk, however. We cannot guarantee that the RSVP site and servers are 100% safe from illegal tampering or "hacking". Once Engage receives study data and enters it into the database, said data has the same protection that Engage Health extends to its own confidential information.

4. Are there costs to participate?

There are no costs to participate in this study.

5. What are the benefits of the study?

You may not receive a direct benefit if you agree to participate. However, we hope your participation in this study may provide a better understanding of how ENPP1/ABCC6 deficiency (GACI or ARHR2) impacts your child's life/your life in meaningful ways to you and possibly help in developing a treatment for this condition. People in the future may benefit from the information obtained from this research.

Your alternative is to not participate in this study.

6. How will my personal information be protected?

The interview data collected by the research vendor, Engage Health, will be stored in a locked / secure location and stored on secure, encrypted, and wholly-owned servers. Your records will be "pseudonymized" which means that your name and any information that could identify you will be removed and replaced with a unique code. The answers that you provide will be combined with those of others participating in the study and summarized in a final report. The final report will be shared with others but will not identify you because the survey is anonymous during analysis of the data. At the end of this study, the researchers may publish the results of this research. We will keep your name and other identifying information confidential, however.

To review the privacy and data storage policies of Engage Health, Inc. for the U.S. and areas outside of the U.S. please visit **https://www.engagehealth.com/privacy-policy/**.

7. Who can answer my questions about this research?

Take as much time as you like before you decide to participate in this study. We will be happy to answer any questions you have about this study.

Contact Pedro Huertas, MD, PhD, Chief Medical Officer at Inozyme, at **(978) 394-5700** for questions, concerns or complaints about the research or if you think you have been harmed as a result of joining this research.

Contact the Western Institutional Review Board (WIRB) if you have questions about your rights as a research subject, concerns, complaints or input: **1-800-562-4789**. WIRB is a group of people who perform an independent review of research.

8. What happens to the information collected for this research?

The study staff at Engage Health, Inc. may share the records generated from this research with other staff at Engage Health on an as-needed basis, and the IRB. This information is shared so the research can be conducted and properly monitored. The people receiving this information are required to protect it and your information may not be redisclosed without your permission. If you do not provide permission to use your information you cannot be in the study. As noted above, the answers that you provide will be combined with those of others participating in the study and summarized in a final report. The final report will be shared with others but will not identify you because the survey is anonymous for the data analysis. At the end of this study, the researchers may publish the results of this research. However, we will keep your name and other identifying information confidential.

This permission will not end unless you cancel it. You may cancel it by sending written notice to Engage Health, the research vendor at **pengel@engagehealth.com** or **Attn: P. Engel, Engage Health Inc., 3265 Lexington Ave. So, Eagan MN 55121**. Alternatively, you can contact Engage Health's Data Protection Officer at **DataProtectionOfficer@engagehealth.com** to remove your information. Any information collected before you withdraw may still be used.

A description of this clinical trial will be available on **http://www.ClinicalTrials.gov,** as required by U.S. Law. This Web site will not include information that can identify you. At most, the Web site will include a summary of the results. You can search this Web site at any time.

9. Can I stop being in the study?

Your decision to be in this study is voluntary. You will not be penalized or lose benefits if you decide not to participate or if you decide to stop participating.

10. Can I be removed from this research without my approval?

Your part in this study may be stopped at any time by Engage Health, Inc., the health research vendor, or the sponsor without your approval for any reason, including:

- If it is in your best interest;
- you do not consent to potential changes made in the study plan (if applicable); or
- you do not keep your scheduled appointment for the telephone interview.

11. Will I be paid for taking part in this research?

If you qualify and participate in the RSVP survey and the interview, you will be paid a $100 honorarium as compensation for your time. You will have two choices regarding how you will be paid. You can either be paid in US dollars by a check that will be mailed to you by Engage Health at the completion of your interview, or you can choose to be paid by an electronic "e-gift card" to Amazon.com, that will be emailed to you by Engage Health at the completion of your interview.

If the telephone interview is not completed for any reason, you will not receive the honorarium.