

## QUESTIONS FOR THE DOCTOR

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*Learn More About*

Long-Term Registry in Patients  
with Urea Cycle Disorders



# THRIVE





## WHAT IS THRIVE?

THRIVE is an observational study that will collect information on up to 500 patients with urea cycle disorders (UCDs). The information collected will help doctors better understand how different treatments affect individuals with UCDs. THRIVE will follow enrolled patients for up to 10 years. As an observational study, enrolled patients will not be required to make any additional office visits or take any medicine outside of normal care.

## WHAT IS A UCD?

A urea cycle disorder, also known as UCD, is a rare genetic condition in which a mutation causes a deficiency in one of the enzymes or mitochondrial transporters in the urea cycle. These enzymes are responsible for removing ammonia from the blood. In UCDs, nitrogen, a waste product of protein metabolism, accumulates in the form of ammonia. Elevated blood ammonia, or hyperammonemia, can cause irreversible brain damage, coma and or death. A UCD is estimated to occur in 1 of 30,000 live births in the U.S.

## WHO CAN PARTICIPATE IN THRIVE?

Patients may qualify to participate in THRIVE if they have a confirmed or suspected diagnosis of a UCD. The patient's doctor will explain the study, and will ask the patient to sign an Informed Consent or Assent Form to join the study.

## WHY SHOULD PATIENTS JOIN THRIVE?

As you know, UCDs are a rare group of disorders affecting both children and adults. In the past few years, there has been an increase in research on UCDs, including studies like THRIVE. By allowing their information to be collected in the study, patients may help doctors and researchers better understand how different medicines affect individuals with UCDs, and help doctors take care of patients with UCDs in the future.

## WHAT WILL PATIENTS BE ASKED TO DO?

If a patient chooses to participate in THRIVE, the patient will see the doctor as usual. The doctor will collect information about the patient's treatment and send it to the THRIVE Team for the 10-year duration of the study. The study will not require any additional doctor's visits or tests outside of normal routine visits to the doctor. Patients will be able to continue with current medicine and treatment plans.

Patients will also be asked to complete two surveys annually. Completing these surveys is optional and will not impact their treatment or their ability to participate in THRIVE. If the patient chooses to complete the surveys, they can choose to complete them at home or at the doctor's office during their annual study visit.



## WILL PATIENT PRIVACY BE PROTECTED?

Doctors who participate in THRIVE, their staff, and Hyperion, the company sponsoring the study, respect all patients' privacy. Please see the HIPAA Authorization form for a complete description of how they will use and protect your health information.

## CAN A PATIENT STOP PARTICIPATION?

Patients are asked to participate in THRIVE for 10 years. If a patient signs up and later decides not to continue, the patient may stop at any time. Simply tell the study doctor. The patient's information will be collected and used up to the point at which he/she decides to not participate.

## WHAT ARE THE INFORMED CONSENT AND ASSENT FORMS?

The Informed Consent and Assent Forms describe the study and any potential risks or benefits to the patient. If the patient is under the age of 18, the patient's parent or legal guardian will sign the Informed Consent Form and the minor can sign an Informed Assent Form. Signing these forms says that the patient understands what THRIVE is and wants to participate in the study. It says that the patient has been able to ask questions about anything he/she does not understand. It also includes notification of your rights under the Health Insurance Portability and Accountability Act (HIPAA). The doctor should provide a copy of these documents after signature.

## WHAT IF I HAVE QUESTIONS?

If you have questions about THRIVE at any time, simply ask your doctor.