WHAT ARE THE POTENTIAL BENEFITS OF PARTICIPATING?

There is no guarantee that participants will receive any benefit from participating in the clinical trial. However, this trial may yield new information that could benefit people with Rett syndrome in the future by helping to answer questions about the effectiveness and safety of the trial medicine.

WHAT SHOULD I DO NOW?

If you are interested in this clinical trial, please discuss it with those you trust, including your current doctor. If you then wish to take the next step toward possible participation, or simply have more questions, please contact us as directed on the back of this brochure.

FOR MORE INFORMATION, PLEASE CONTACT:

Kennedy Krieger Institute
Clinical Trials Unit
Researchtrials@kennedykrieger.org
443-923-3850

*For Females Ages 2 to 18 at Time of Enrollment*
Rett syndrome is a rare medical condition causing abnormal development in young girls. There is currently no cure for Rett syndrome. Treatment focuses on managing the symptoms through medications and therapy.

Right now, researchers are conducting a clinical trial for a potential new medicine for children with Rett syndrome. The trial medicine is currently referred to by a number, GWP42003-P. We are conducting this clinical trial because we want to find out whether the trial medicine can help reduce the severity of symptoms in children with Rett syndrome. We will also be looking to see how safe it is to use.

The trial medicine has not been approved by regulatory authorities to treat Rett syndrome, and no request for such approval has yet been submitted.

Medical researchers must conduct clinical trials like this one before a new medicine can be approved for regular use in Rett syndrome. We hope that you will carefully consider whether participating in this clinical trial is a good choice for your family, because clinical trials cannot be conducted without the help of volunteer trial participants.

To learn more, please read the following answers to commonly asked questions about this important clinical trial.

**CAN YOU BRIEFLY DESCRIBE THIS CLINICAL TRIAL?**

This clinical trial is for females with Rett syndrome between ages 2 to 18 at time of enrollment.

All trial participants will be allowed to continue any therapy they are currently using for their Rett syndrome.

The trial medicine is a liquid to be taken by mouth, twice per day. Special arrangements are available for trial participants using feeding tubes.

The effects of the trial medicine will be compared to a non-medicine called a placebo. The placebo looks like the trial medicine but has no active ingredients. For every 3 children in the trial, 2 will be given the trial medicine and 1 will be given the placebo. The assignment to receive the trial medicine or placebo is made randomly by a computer. Neither you nor the trial doctor will know which your child receives.

At the end of this trial, the trial doctor may invite your child to participate in an open-label study of the trial medicine. The trial doctor will decide whether it is in your child’s best interests to participate in the open-label study. In the open-label study, there will be no placebo, which means that all participants receive active trial medicine.

This clinical trial will involve up to 252 patients with Rett syndrome.

A parent or guardian of all trial participants will be actively involved in this trial. In addition to bringing their child to all in-person visits, a parent or guardian will be asked to administer the trial medicine, to answer questionnaires about their child’s symptoms, and to complete a brief daily dosing diary and weekly symptom diary. The trial-center staff will provide complete instructions for all these activities.

**DOES IT COST ANYTHING TO PARTICIPATE?**

All medical examinations, trial visits, and the trial medicine, or placebo, are provided to qualified participants at no cost. Compensation will also be available for time and travel. Travel arrangement assistance may also be provided, depending upon the circumstances.

**ARE THERE ANY RISKS TO PARTICIPATING IN THIS CLINICAL TRIAL?**

There may be potential risks to participating in this clinical trial. All drugs and medical procedures carry a risk of side effects; therefore, it is possible that participants may experience some discomfort or other side effects associated with the use of the trial medicine. Before participants and their family are asked to decide whether they wish to participate, the potential risks of participation will be explained in detail. If the child’s parent or guardian believes that it is in their child’s interests to participate, then the trial-center staff will ask them to sign a form agreeing to move forward.

**HOW LONG WILL THE CLINICAL TRIAL LAST AND WHAT WILL BE REQUIRED OF MY CHILD AND ME?**

Complete trial participation will last about 8 months. During this time, trial participants will have 8 in-person visits to the trial center, plus 3 scheduled telephone visits.