

GALACTOSEMIA GAZETTE: CLINICAL TRIAL UPDATE



The Clinical Trial Continues: And there is still time to enroll

The pediatric drug trial for Galactosemia conducted by Applied Therapeutics has restarted and there is still time to get involved.

There are three new clinical trial sites including a new location in Atlanta, one in Ann Arbor, Michigan and another located in Denver, Colorado. The clinical trial sites specialize in pediatric clinical trials. Children ages 2 – 17 are eligible to participate.

Part A of the trial, which will determine the proper dose for the three age groups (2-6, 7-12, 13-17) will soon conclude and Part B of the trial will start right away. Those who are participating in Part A will automatically roll over to Part B and new participants can also enroll in Part B.



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Some participants will receive the active drug and others will receive a placebo. Participants who receive the placebo will be able to start on the active drug at the end of the study. Throughout the trial, participants will receive a daily dose of the active medicine, or placebo. During the clinic site visit at the beginning and end of the trial, and during home visits throughout the trial, participants will be monitored with blood draws and other tests.

Before your first visit to the clinical trial site, a nurse will come to your home for initial testing and information. After the first clinical site visit concludes, a home health nurse will visit monthly, and participants will be required to return to the clinic site for follow up after 90 days and then every 6 months until the conclusion of the trial. All travel expenses and hotel stays are covered as well as a food allowance and daily compensation while you are onsite for the trial.

Participation in rare disease clinical trials is essential to ensure there are enough participants to generate sufficient data and show statistical significance. Every patient counts and it is an opportunity to convey the importance of research and clinical trials which are needed to find a treatment for Galactosemia, as soon as possible.

Initial results in the adult clinical trial show that the drug AT-007 reduces galactitol levels – which is believed to be the cause of galactosemia complications.

If you're interested to learn more or want to enroll your child, email galactosemia@appliedtherapeutics.com.

If you're still unsure, keep reading to hear from families who are currently participating:

Leigh-Anne Bennett decided to enroll her daughter Lexi because after 16 years of being told there is no cure and to just manage a strict diet, this was the first promising treatment.

But they have experienced unexpected benefits too, "Lexi has been able to meet and connect with several other teens and has formed friendships we hope will last a lifetime," she said. "We were even able to take Lexi and her friend Ava out for a sweet treat during our time in Atlanta for the trial."

Leigh-Anne encourages others to get involved, "I know it's not a cure, but if this drug is successful it could help our kids have less complications secondary to galactosemia," she said. While the frequent blood draws have been hard on Lexi, she says the experience has been good, overall. "So far we have really liked the nurses who have come for the home health visits," she said. Applied Therapeutics has been easy to deal with and it's always nice to be with other families affected by galactosemia."



Michelle Hendrickson said her family decided to participate in the trial after reading the adult study data. “We wanted to be part of the possible change that this can bring to Galactosemics,” she said. We presented the information to our daughter and she decided that she wanted to do the trial for all the other kids and future galactosemics.”



Michelle said the experience has been good. “It’s been busy and lots of lab days, but it’s also been handled well by the rare disease research center and the nurses.”

They have also experienced other benefits, “Ava has made a pretty good friend through this experience and would like to get to know other kids a little better soon,” Michelle said. “Through this we have seen growth and maturity in our daughter.”

She encourages others to participate because it can change the kids’ futures and outcomes.



What's Next?

After the clinical trial is complete, Applied Therapeutics will submit the information to the FDA for approval. This can be a lengthy process, and this is where we have an opportunity as a community. The FDA has an office called Patient Affairs and they invite patient advocacy groups as well as patients, caregivers, and families to share their personal experiences about their life living with rare diseases like Galactosemia. We want the FDA to know that future treatments for Galactosemia are desperately needed and we need a treatment urgently to help our children. By stepping up and speaking out about the challenges created by galactosemia, about the uncertain futures and the hope we have in new treatments – we can help show the FDA that their fast action is important to our small but mighty community. If you are willing to share your family’s story about galactosemia and how a new treatment could improve your situation, please contact Brittany Cudzilo at brittany.cudzilo@galactosemia.org. This may involve a written submission or a video submission talking about why fast action from the FDA could make a difference to your family.

We know that we are stronger together, and we can show that by participating in the clinical trial and/or sharing our story to help support the need for access to new treatments as soon as possible.