PROJECT INFORMATION

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Developmental Outcomes in Children with Duarte Galactosemia

Principal investigator

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Organization

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What was the research about?

Galactosemia is a group of rare, inherited health problems. Babies with galactosemia have problems breaking down galactose, a sugar in milk. Babies with the most severe type of galactosemia get sick and may die if they drink breast milk or milk-based formula. Even if they don't drink milk, they may have developmental complications when they are older. Developmental complications include problems moving, speaking, learning, hearing, or getting along with people.

Duarte galactosemia, or DG, is a less serious type of galactosemia. In DG, babies can break down some but not all of the galactose in milk. Few studies have looked at the risks of DG. Doctors don't know whether babies with DG can drink breast milk and milk-based formula safely or whether drinking milk will cause problems when these babies are older.

In this study, the research team looked at whether

- Children with DG have more developmental complications than their siblings without DG
- Children with DG who drank breast milk or milk-based formula when they were babies have more developmental complications than children with DG who drank low-galactose formula

What were the results?

Children with DG didn't have more developmental complications than their siblings without DG.

Children with DG who drank breast milk or milk-based formula didn't have more developmental complications than children with DG who drank low-galactose formula.

Who was in the study?

The study included 206 children ages 6 to 12 with DG from 17 states and Washington, DC. Of the children with DG, 40 percent drank breast milk or milk-based formula as babies, and 60 percent drank low-galactose formula. The study also included 144 siblings of these children. The siblings were in the same age range but didn't have DG. Of all the children, 90 percent were white, 2 percent were black, and 5 percent were another race; 3 percent were Hispanic. Because of the genetics of DG, most children with DG are white. The average age was nine, and 53 percent were boys.

What did the research team do?

The research team asked parents or guardians of children with DG to fill out a survey. The survey asked about

- Their children's health and diet, including whether they drank milk before age one
- Special educational help children received before age three
- Other children in the family without DG who could be in the study

Next, the research team checked children for developmental complications. The team tested the children's abilities to move, speak, learn, hear, and get

along with people. Team members also did genetic tests to make sure which children did and didn't have DG.

Parents of children with DG, doctors, and people from advocacy groups worked with the research team on the study.

What were the limits of the study?

Parents or guardians may not have been able to accurately remember their children's health and diet as babies. Also, results may have differed if the research team had tested children older than 12.

Future research could look at whether children with DG have other kinds of health problems. Researchers could also look at children older than the children in this study.

How can people use the results?

Doctors and parents can use the results when thinking about whether to avoid breast milk and milk-based formula for babies with DG.

To learn more about this project, visit www.pcori.org/FridovichKeil281.