Cystic Fibrosis Research News

Title:

An open-label extension study of Ivacaftor in children with CF and a CFTR gating mutation initiating treatment at age 2-5 years (KLIMB)

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What was your research question?

We wanted to learn more about the safety of ivacaftor when used for longer periods in children with CF who started taking it aged 2-5 years. We assessed this over an extra 84 weeks for children who had completed 24 weeks use of ivacaftor in an earlier study.

Why is this important?

These data in young children are important because early treatment may have more impact at reducing this progressive disease and because life-long usage of ivacaftor is expected. A better understanding of the safety of longer term use is critically important for families and clinicians in managing this treatment.

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What did you do?

We studied safety outcomes for the 33 children who had completed the earlier 24 week study of ivacaftor in children living with CF, and who had started taking it aged 2-5 years. All of the children received ivacaftor in an open label design, so families were aware the child was taking ivacaftor. At regular clinic visits, we assessed safety for the children based on adverse event reports, laboratory tests and ECGs. In addition, we also studied how well they grew, how aspects of their pancreas function changed, and how their sweat chloride tests changed (to assess impact on the underlying cause of the disease).

What did you find?

Amongst these children, we found that the safety of ivacaftor was consistent with the earlier trial and it was generally well tolerated (and palatable for the children). There was no pattern of adverse events that worsened or increased with prolonged use of ivacaftor and the events were mostly typical for CF children of this age . Specifically, there were no new concerns about eye or liver health over the period of this study. We also found that improved growth was maintained, positive sweat chloride changes were sustained, and that markers of pancreatic function continued to show positive changes across the study.

What does this mean and reasons for caution?

These data indicate that ivacaftor continued to have a favourable benefit to risk profile in this age group and that it may help to reduce the progression of CF disease. Caution is warranted in view of the lack of placebo control in the design and the relatively small patient numbers (due to the rarity of the mutations that were eligible for study).

What's next?

There are on-going plans to continue to assess the safety and effectiveness of ivacaftor in this age group using real world data. It is also planned to study ivacaftor in younger children aged less than 2 years with CF, to support the potential for even earlier intervention.

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