Cystic Fibrosis Research News

Title

CFTR modulators and pregnancy outcomes: early findings from a nationwide cohort study

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What was your research question?

This study investigated whether CFTR modulators, a group of medications used to treat cystic fibrosis (CF), are safe to use during pregnancy.

Why is this important?

CFTR modulators have significantly improved the health and life expectancy of people with CF. As a result, more women with CF are choosing to have children. However, there is limited data on whether these medications are safe to take during pregnancy. Because pregnant women are typically excluded from clinical trials, healthcare providers have had little information to guide treatment decisions. Understanding the effects of CFTR modulators on pregnancy is crucial for ensuring the best outcomes for both mothers and babies.

What did you do?

We conducted a nationwide study in France using health insurance data from 2018 to 2023. We identified 590 pregnancies in women with CF, of which 148 were exposed to CFTR modulators. The study compared pregnancy outcomes between women who used CFTR modulators and those who did not. Data on birth outcomes, birth defects, preterm births,

and infant health were analyzed to assess whether taking these medications affected pregnancy safety.

What did you find?

Elexacaftor/tezacaftor/ivacaftor (ELX/TEZ/IVA) was the mostly used CFTR modulator and was maintained throughout whole pregnancy for almost all the patients. No increased risk of birth defects was observed in babies exposed to CFTR modulators. The rate of preterm birth was similar between exposed and unexposed pregnancies. Interestingly, babies born to mothers using CFTR modulators were less likely to be small for their gestational age, suggesting potential benefits for fetal growth in relation to a better maternal health.

What does this mean and reasons for caution?

These findings are reassuring and suggest that CFTR modulators do not increase the risk of major birth defects or other serious pregnancy complications. However, there are still important limitations. The study relied on drug reimbursement, but it could not confirm whether women actually took their medication as prescribed. Furthermore, although this is the largest study to date, the number of pregnancies is relatively limited and might prevent for identifying unknown rare adverse events. Finally, babies were followed for a short time after birth, so long-term effects are unknown.

What's next?

Further research is needed to confirm these findings and explore the long-term effects of CFTR modulators on children exposed in the womb. In the meantime, these early results provide hopeful news for women with CF who wish to have children while continuing their treatment.

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