

Cystic Fibrosis Research News

Title:

Outcomes of Prenatal Use of Elexacaftor/Tezacaftor/Ivacaftor in Carrier Mothers to Treat Meconium Ileus in Fetuses with Cystic Fibrosis

Lay Title:

Use of Elexacaftor/Tezacaftor/Ivacaftor in Pregnant Mothers without CF to Treat Meconium Ileus in Fetuses with Cystic Fibrosis

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What was your research question?

If a mother who does not have cystic fibrosis (CF) takes a medicine used to treat cystic fibrosis (elexacaftor/tezacaftor/ivacaftor) because her fetus has CF and ultrasound findings of meconium ileus (intestinal obstruction), will the fetus's bowel obstruction go away?

Why is this important?

Elexacaftor/tezacaftor/ivacaftor (ETI) is a medication used in people with cystic fibrosis (CF), but some complications of CF can happen in utero. Some infants with CF are born with meconium ileus (MI), an obstruction in the small intestine. MI can cause infants to need surgery to remove the obstructed intestine after birth and can cause problems with nutrition and growth. MI develops in utero and can often be seen on prenatal ultrasounds. Some complications of MI could be improved if MI could be fixed before the infant is born.

What did you do?

At our institution, we treated three pregnant mothers with ETI whose fetuses had signs of meconium ileus on ultrasound and a confirmed diagnosis of CF. The pregnant mothers took



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ETI even though they themselves did not have CF. We then observed the infants to see if the meconium ileus resolved.

What did you find?

All the infants and mothers treated tolerated the medication well without significant side effects. In one infant, the MI resolved before birth and the infant did not need any further treatments. In another infant, the MI improved but did not go away. Once the baby was born, the intestinal blockage was cleared with enemas. In the third infant, the MI did not go away, and she needed surgery to remove the blocked intestines. The mother of the infant who required surgery did not receive treatment with ETI until she was 35 weeks pregnant.

What does this mean and reasons for caution?

Like previous cases, we found that treating pregnant mothers with ETI seems to help improve MI in the fetus. However, timing of when ETI was started appears to influence how well the medication works to clear MI. If ETI is started too late in pregnancy, it does not seem to work as well. No significant side effects of the medication were found in the mothers or infants. This is a report of three cases, and more research is needed to find out when ETI should be started and to make sure mothers and infants do not have side effects.

What's next?

Larger studies of mothers without CF taking ETI to improve MI in fetuses with CF are needed to confirm that this treatment is safe and effective for MI.

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