primary studies - published, non RCT

Safety and biological efficacy of a lipid-CFTR complex for gene transfer in the nasal epithelium of adult patients with cystic fibrosis.

Code: PM10933918 **Year:** 2000 **Date:** **Author:** Noone PG

Study design (if review, criteria of inclusion for studies)

Cross sectional study

Participants

CF Center staff completed an anonymous questionnaire regarding pregnancy and infant outcomes for 45 women who used elexacaftor-tezacaftor-ivacaftor (ETI) during pregnancy and/or lactation.

Interventions

ETI during pregnancy and/or lactation.

Outcome measures

Teratogenicity; unknown fetal impact; complications

Main results

Of 45 ETI-exposed pregnancies reported to date, complications in 2 mothers and in 3 infants (2 born to mothers with poorly controlled diabetes) were rated by clinicians as unknown (possible) or suspected relatedness to ETI use. Two women terminated unplanned pregnancies. Miscarriage rates were consistent with that known in the general U.S. Five of the six women who discontinued ETI out of concern for unknown fetal risk restarted because of clinical deterioration. No infant cataracts were reported though only two infants were formally evaluated.

Authors' conclusions

In the context of the known increased rate of complications in women with CF and their infants, data from this retrospective survey is reassuring for women who choose to continue ETI during pregnancy. However, a large, multi-center prospective study is needed to assess impact of use of ETI in pregnancy.

http://www.mrw.interscience.wiley.com/cochrane/clcentral/articles/907/CN-00298907/frame.html

See also

Molecular therapy : the journal of the American Society of Gene Therapy YR: 2000 VL: 1 NO: 1

Keywords

Adult; Aged; CFTR Modulators; Genetic Predisposition to Disease; pharmacological_intervention; placebo; VX-770; VX-661; ivacaftor; Aminophenols; tezacaftor; VX-445; elexacaftor; Trikafta; Pregnancy; kaftrio;