NHSEED - - Economic Study or Review

A phase 3, double-blind, parallel-group study to evaluate the efficacy and safety of tezacaftor in combination with ivacaftor in participants 6 through 11 years of age with cystic fibrosis homozygous for F508del or heterozygous for the F508del-CFTR mutation and a residual function mutation.

Code: PM32967799 **Year:** 2021 **Date:** 2014 **Author:** Davies JC

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

cost-effectiveness study. A previously developed decision analysis model for NBSCF was fed with model parameters mainly based on a study evaluating two novel screening strategies among 145,499 newborns in The Netherlands.

Participants

newborns

Interventions

Four screening strategies for NBSCF, i.e. immunoreactive trypsinogen-testing followed by pancreatitis-associated protein-testing (IRT-PAP), IRT-DNA, IRT-DNA-sequencing, and IRT-PAP-DNA-sequencing, each compared to no-screening.

Outcome measures

Cost-effectiveness

Main results

The four screening strategies had cost-effectiveness ratios varying from â‚¬23,600 to â‚¬29,200 per life-year gained. IRT-PAP had the most favourable cost-effectiveness ratio. Additional life-years can be gained by IRT-DNA but against higher costs. When treatment costs reduce with 5% due to early diagnosis, screening will lead to financial savings.

Authors' conclusions

NBSCF is as an economically justifiable public health initiative. Of the four strategies tested IRT-PAP is the most economic and this finding should be included in any decision making model, when considering implementation of newborn screening for CF.

http://dx.doi.org/10.1016/j.jcf.2020.07.023

See also

J Cyst Fibros. 2021 Jan;20(1):68-77. doi: 10.1016/j.jcf.2020.07.023. Epub 2020 Sep 21.

Keywords

Neonatal Screening; Newborn; non pharmacological intervention - diagn; screening; diagnostic procedures;