HTA - - Health Technology Assessment Report

A Phase 3, Open-Label Study of Lumacaftor/Ivacaftor in Children 1 to Less Than 2 Years of Age with Cystic Fibrosis Homozygous for F508del-CFTR.

Code: PM35771568 **Year:** 2022 **Date:** 2012 **Author:** Rayment JH

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

Included studies: health technology assessments, systematic reviews, meta-analyses, randomized controlled trials, non-randomized controlled trials, diagnostic trials, retrospective controlled analysis, economic evaluations

Participants

Newborns.

Interventions

Any diagnostic test including sweat testing, genetic evaluation and/or extended clinical examination

Outcome measures

Diagnosis accuracy of cystic fibrosis newborn screening (including sensitivity, specificity, positive and negative predictive values). Cost-effectiveness of new born screening for cystic fibrosis

Main results

A total of 280 potential citations were identified by the search in bibliographic databases, with 257 citations being excluded during the title and abstract review based on irrelevance to the questions of interest. The full text documents of the remaining 23 articles were retrieved. One article was detected by the grey literature search. Of the 24 articles, 15 did not meet the eligibility criteria and were excluded; leaving 9 articles for this review. Eight cystic fibrosis newborn screening protocols were identified in the reviewed literature. The cost of cystic fibrosis newborn screening was estimated for two protocols; the screening cost per newborn ranged from $US 4.48 to $US 6.78.

Authors' conclusions

There was strong evidence of high sensitivity of the evaluated screening protocols and suggestive evidence of low positive predictive value (high false screen-positive).

http://dx.doi.org/10.1164/rccm.202204-0734OC

See also

Am J Respir Crit Care Med. 2022 Nov 15;206(10):1239-1247. doi: 10.1164/rccm.202204-0734OC.

Keywords

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