Cochrane Database of Systematic Reviews - - Cochrane Review

Cystic fibrosis newborn screening

Code: HTA-32011000927 **Year:** 2011 **Date:** 2012 - updated: 09 APR 2017 **Author:** Paz-Valinas L

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

Randomised controlled trials of the use of ursodeoxycholic acid for at least three months compared with placebo or no additional treatment in people with cystic fibrosis.

List of included studies (3)

Colombo 1996; Merli 1994; O'Brien 1992

Participants

Children and adults with defined CF, diagnosed clinically and by sweat test or genetic testing, including all ages, all degrees of severity of disease and any degree of liver involvement.

Interventions

UDCA (ursodeoxycholic acid) for at least three months vs placebo or no additional treatment

Outcome measures

Change in weight (kg); Death due to all causes; Death related to liver disease; Development of complications of portal hypertension; Development of portal hypertension; Lack of normalisation of 5â€™ nucleotidase; Lack of normalisation of alanine transferase; Lack of normalisation of all liver enzymes reported in the study; Lack of normalisation of any liver enzyme reported in the study; Lack of normalisation of aspartate transaminase; Lack of normalisation of gammaglutamate transferase; Need for liver transplantation

Main results

Twelve trials have been identified, of which four trials involving 137 participants were included; data were only available from three of the trials (118 participants) since one cross-over trial did not report appropriate data. The dose of ursodeoxycholic acid ranged from 10 to 20 mg/kg/day for up to 12 months. The complex design used in two trials meant that data could only be analysed for subsets of participants. There was no significant difference in weight change, mean difference -0.90 kg (95% confidence interval -1.94 to 0.14) based on 30 participants from two trials. Improvement in biliary excretion was reported in only one trial and no significant change after treatment was shown. There were no data available for analysis for long-term outcomes such as death or need for liver transplantation.

Authors' conclusions

There are few trials assessing the effectiveness of ursodeoxycholic acid. The quality of the evidence identified ranged from low to very low. There is currently insufficient evidence to justify its routine use in cystic fibrosis.

http://www.crd.york.ac.uk/CRDWeb/ShowRecord.asp?ID=32011000927

See also

Health Technology Assessment Database YR: 2011 NO: 1

Keywords

Cholagogues and Choleretics; Gastrointestinal Diseases; Liver Diseases; pharmacological_intervention; UDCA; Gastrointestinal Agents;