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Clinical Expert Summary
Ursodeoxycholic acid (Ursofalk®) 250 mg hard capsules, 250 mg/5 ml suspension, 500 mg film-coated tablets

Ursodeoxycholic acid (Ursofalk®) for the treatment of hepatobiliary disorders associated with cystic fibrosis (CF) in children aged 1 month to 18 years.

1. Existing guidelines

There are local guidelines in accordance with international recommendations and CF Trust standards of care on the detection and monitoring of cystic fibrosis-related liver disease (CFLD):

- Cystic Fibrosis our focus. Standards for the clinical care of children and adults with cystic fibrosis in the UK. Second Edition 2011[1].
- Best practice guidance for the diagnosis and management of cystic fibrosis-associated liver disease 2011[2].

In addition experts highlighted:

- Paediatric Respiratory Medicine. Children's Hospital for Wales, Cardiff. CF related liver disease 2014[3].

2. Disease prevalence/incidence

CFLD is a rare complication of CF; thus 10-20% of children with CF aged less than 16 years have liver disease and 1-2% have established cirrhosis with or without portal hypertension. There are 180 children and adolescents under the care of the tertiary CF unit and currently about 25-35 children receive ursodeoxycholic acid (UDCA) for CFLD. There are none with established cirrhosis with or without portal hypertension.

3. Current treatment options

UDCA was confirmed as the first-line treatment for CFLD. Experts reported that the treatment can improve bile flow and may affect the natural history of CFLD. The medicine is started in children who either have two liver abnormalities (e.g. abnormal ultrasound, raised liver transaminases, abnormal prothrombin time or palpable liver) or in those who have one abnormality in two consecutive years. Asymptomatic gallstones in children with CF should be treated with UDCA. Oral vitamin K should also be given to all children with established liver disease.

4. Unmet needs

There is a need for a good preparation of multivitamin containing vitamin K, mendiol is currently used.

5. Knowledge of product in given indication

The medicine has been extensively used off-label for the management of CFLD in children and adolescents for many years. Equivocal cases would probably be treated with UDCA as the treatment is benign and the disease is the second largest cause of death in CF patients. It is prescribed in the form of elixir for younger children who are unable to swallow tablets or capsules.

REFERENCES

1. Cystic Fibrosis Trust. Standards for the clinical care of children and adults with cystic fibrosis in the UK. Second Edition. 2011. Available at: https://www.cysticfibrosis.org.uk/the-work-we-do/clinical-care/consensus-documents. Accessed March 2016.

2. Debray D KD, Houwen R, Strandvik B. Best practice guidance for the diagnosis and management of cystic fibrosis-associated liver disease. *J Cystic Fibrosis.* 2011;10(Suppl 2):8. Available at: http://cysticfibrosis.elsevierresource.com/articles/best-practice-guidance-diagnosis-and-management-cystic-fibrosis-associated-liver-disease/fulltext. Accessed March 2016.
3. Wales TCHf. Paediatric Respiratory Medicine, CF-related liver disease. 2014. Available at: http://www.uhwchildren.com/respiratory/?page_id=9#liverdisease. Accessed Apr 2016.