Glycerol phenylbutyrate (Ravicti) for urea cycle disorders (hyperammonaemia)  
NIHR HSRIC

Record Status
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Authors' objectives
Glycerol phenylbutyrate (Ravicti) is intended to be used for the treatment of urea cycle disorders (hyperammonaemia) in adults and paediatric patients over two years of age. Glycerol phenylbutyrate is a prodrug of sodium phenylbutyrate, an ammonia scavenger and it provides an alternative pathway to the urea cycle for the disposal of nitrogen waste via renal excretion of phenylacetylglutamine formed from phenylacetic acid and glutamine. Urea cycle disorders (UCDs) include several inherited enzyme transporter deficiencies that result in the accumulation of toxic levels of ammonia in the blood and brain (hyperammonaemia). Urea cycle disorders are inborn errors of ammonia detoxification/arginine synthesis due to defects affecting the catalysts of the Krebs-Henseleit cycle. Inter-current infection is the most common precipitant of acute hyperammonaemia, accounting for 34% of episodes, with respiratory virus a leading cause. The estimated incidence of urea cycle disorder is 1 in 8,000, and it may present either shortly or after birth (50%), or at any age. The main aim of therapy for hyperammonaemia is to correct the biochemical abnormalities and to ensure adequate nutritional intake. Pharmaceutical treatment involves compounds that increase the removal of nitrogen waste and antiemetic agents such as ondansetron, granisetron, palonosetron and dolasetron may also be used. Other management approaches include dietary restriction of protein and/or nitrogen intake and parenteral intake of calories. Surgical interventions include liver transplant or liver cell transplantation. Glycerol phenylbutyrate is currently in phase III clinical trials, comparing its effect on biochemical markers against treatment with sodium phenylbutyrate.

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