Twice-daily cysteamine bitartrate therapy for children with cystinosis.

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OBJECTIVE: Cystinosis causes renal and other organ failure. Regular 6-hourly cysteamine bitartrate (Cystagon; Mylan, Morgantown, West Virginia) reduces intracellular cystine and the rate of organ deterioration. A formulation of cysteamine requiring less frequent dosing may improve compliance and possibly patient outcome. METHODS: Enteric-release cysteamine was prepared. For a period of 1 month, patients received their regular cysteamine dose every 6 hours (stage I). The patients then underwent pharmacokinetic and pharmacodynamic studies following washout periods using single-doses of cysteamine and enteric-release cysteamine (stage II). Finally, the patients commenced regular enteric-release cysteamine therapy (stage III). Weekly trough white blood cell (WBC) cystine levels were recorded. RESULTS: Seven children with cystinosis (mean age, 11.8 years; range, 8-17 years) who received cysteamine and enteric-release cysteamine (mean dose, 45 and 28.8 mg/kg body weight/day, respectively) had mean WBC cystine levels of 0.7+/-0.3 and 0.41+/-0.22 nmol half-cystine/mg protein in study stages I and III, respectively. Study stage II showed that the mean time (T(max)) to reach the maximum plasma cysteamine level (C(max)) was longer for enteric-release cysteamine than for cysteamine (176 minutes vs 60 minutes; P=.001), but the mean C(max) at the same dose was similar. Mean serum gastrin levels were similar after ingestion of cysteamine and enteric-release cysteamine. CONCLUSIONS: Twelve-hour enteric-release cysteamine, given at approximately 60% of the previous daily dose of cysteamine, was effective in maintaining trough WBC cystine levels within a satisfactory range.

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