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IN BRIEF

Uridine Triacetate (*Xuriden*) for Hereditary Orotic Aciduria

The FDA has approved the pyrimidine analog uridine triacetate (*Xuriden* [zur' uh den] – Wellstat Therapeutics) for treatment of hereditary orotic aciduria, a rare autosomal recessive disorder (estimated birth prevalence: <1:1,000,000) in infants and children caused by a deficiency in uridine 5'-monophosphate (UMP) synthase. This deficiency prevents synthesis of uridine nucleotides and causes developmental delays, failure to gain weight, hematologic abnormalities, and excessive urinary excretion of orotic acid, which can lead to urinary obstruction.¹ *Xuriden* is the first drug to be approved in the US for this indication. Uridine triacetate is also available as *Vistogard* for treatment of fluorouracil (5-FU) or capecitabine (*Xeloda*, and generics) overdose or severe or life-threatening toxicity.

In an unpublished, single-arm, 6-week trial with a 6-month extension (summarized in the package insert), 4 patients 3-19 years old with hereditary orotic aciduria were treated with uridine triacetate 60 mg/kg once daily for 6 weeks, followed by 60-120 mg/kg once daily for 6 months. After 6 months of treatment, hematologic parameters (neutrophil count, WBC count, or mean corpuscular volume) either improved or remained stable and orotic acid levels remained stable in all 4 patients. Weight growth either improved or remained stable and height growth remained stable in 3 patients measured after 6 months of treatment. No adverse effects were reported.

Xuriden is available as oral granules in 2-gram single-use packets. The recommended starting dosage is 60 mg/kg once daily. The dose can be increased to 120 mg/kg (max 8 grams) once daily if urine orotic acid levels increase or remain above normal, or for worsening disease. The granules should be mixed in applesauce, pudding, yogurt, milk, or infant formula and swallowed immediately, without chewing. Once the packet is opened, any unused granules should be discarded. The cost for 30 2-gram packets of *Xuriden* is \$22,500.² ■

1. S Balasubramaniam et al. Inborn errors of pyrimidine metabolism: clinical update and therapy. *J Inherit Metab Dis* 2014; 37:687.
2. Approximate WAC = wholesaler acquisition cost, or manufacturer's published price to wholesalers; WAC represents published catalogue or list prices and may not represent an actual transactional price. Source: AnalySource[®] Monthly. March 5, 2016. Reprinted with permission by First Databank, Inc. All rights reserved. ©2016. www.fdbhealth.com/policies/drug-pricing-policy.

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