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Xuriden (uridine triacetate) now approved for patients with hereditary orotic aciduria

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Today, the U.S. Food and Drug Administration approved Xuriden (uridine triacetate), the first FDA-approved treatment for patients with hereditary orotic aciduria. Hereditary orotic aciduria is a rare metabolic disorder, which has been reported in approximately 20 patients worldwide.

Hereditary orotic aciduria is inherited from a recessive gene. The disease is due to a defective or deficient enzyme, which results in the body being unable to normally synthesize uridine, a necessary component of ribonucleic acid (RNA). Signs and symptoms of the disease include blood abnormalities (anemia, decreased white blood cell count, decreased neutrophil count), urinary tract obstruction due to the formation of orotic acid crystals in the urinary tract, failure to thrive, and developmental delays.

"Today's approval and rare pediatric disease priority review voucher underscore the FDA's commitment to making treatments available to patients with rare diseases," said Amy G. Egan, M.D., M.P.H., deputy director of the Office of Drug Evaluation III in the FDA's Center for Drug Evaluation and Research (CDER). "Prior to Xuriden's approval, patients with this rare disorder had no approved treatment options."

The FDA granted Xuriden orphan drug designation because it treats a rare disease. Orphan drug designation provides financial incentives, like clinical trial tax credits, user fee waivers, and eligibility for market exclusivity to promote rare disease drug development. Xuriden was also granted priority review. An FDA priority review provides for an expedited review of drugs for serious diseases or conditions that may offer major advances in treatment. The manufacturer of Xuriden was granted a rare pediatric disease priority review voucher – a provision that encourages development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

Xuriden is an orally administered product intended to replace uridine. Xuriden is approved as oral granules that can be mixed with food or in milk or infant formula, and is administered once daily.

The safety and effectiveness of Xuriden were evaluated in a single arm, six-week, open-label trial in four patients with hereditary orotic aciduria, ranging in age from three to 19 years of age, and in a six-month extension phase of the trial. The study assessed changes in the patients' pre-specified hematologic parameters during the trial period. At both the six-week and six-month assessments, Xuriden treatment resulted in stability of the hematologic parameters in all four clinical trial patients. The safety and effectiveness of uridine replacement therapy were further supported by case reports from the published literature.

No side effects were observed in patients treated with Xuriden for up to nine months.

Xuriden is marketed by Wellstat Therapeutics Corporation, based in Gaithersburg, Maryland.

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