FDA approves Procysbi for nephropathic cystinosis

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Affecting approximately 500 patients in the United States and an estimated 3,000 worldwide, cystinosis can lead to slow body growth and small stature, weak bones, and developing and worsening kidney failure. Procysbi has been approved to treat the most severe form of cystinosis—nephropathic cystinosis.

A major study involving 43 patients found Procysbi to be as effective at

controlling cystine levels as Cystagon, an immediate-release tablet approved by the FDA in 1994. Recorded common side effects included gastrointestinal discomfit, bad breath, constipation, headache, drowsiness, and dizziness. More severe but less common side effects included ulcers or bleeding of the stomach or intestine, altered mental state, seizures, severe skin rashes, and allergic reactions.

"Procysbi is the only delayed-release product approved by FDA to treat nephropathic cystinosis, offering patients with this rare disease an important new treatment option," Andrew E. Mulberg, M.D., deputy director of the Division of Gastroenterology and Inborn Errors Products in the FDA's Center for Drug Evaluation and Research, said in a statement.

Procysbi is manufactured by Raptor Pharmaceuticals, based in Novato, Calif.

More information: More Information