Generic Name:
Migalastat
Trade Name:
Galafold
Company:
Amicus Therapeutics U.S.
Notes:

FDA approved migalastat, the first oral medication for the treatment of adults with Fabry disease, a rare and serious genetic disorder caused by mutations in the alpha-galactosidase A (GLA) gene located on the X-chromosome. The disease results from buildup of globotriaosylceramide (GL-3) in blood vessels, the kidneys, the heart, the nerves, and other organs.

It is estimated that classic Fabry disease (the most severe type) affects approximately one in 40,000 males. The later-onset type is more frequent and in some populations may occur in one in 1,500 to 4,000 males. Patients with Fabry disease develop slowly progressive kidney disease, cardiac hypertrophy, arrhythmias, stroke, and early death.

Efficacy was demonstrated in a 6-month, placebo-controlled clinical trial in 45 adults with Fabry disease. Patients treated with migalastat had a greater reduction in GL-3 in blood vessels of the kidneys (as measured in kidney biopsy samples) compared with patients on placebo. Migalastat's safety was studied in four clinical trials that included 139 patients with Fabry disease.

The most common adverse drug reactions were headache, nasal and throat irritation, urinary tract infection, nausea, and fever.

Medication Monitor Categories:
New Drug Approvals