Notes

Notes

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Treating your Fabry disease

There are currently three Health Canada approved therapies available to treat Fabry disease in Canada. You and your doctor will decide which option is best for you.

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Enzyme Replacement Therapy (ERT)

ERT is a medical treatment replacing an enzyme in patients in whom that particular enzyme is deficient or absent. In the case of Fabry disease, the enzyme that is deficient is called α-galactosidase A (α-Gal A) enzyme.

ERT does not affect the underlying genetic defect, but increases the level of enzyme in which the patient is deficient. ERT (Fabrazyme® and Replagal®), as well as oral chaperone therapy (Galafold™), are all life-long treatments with regular and frequent intravenous infusions of enzyme biweekly throughout the patient's life.

There are two ERT drugs approved by Health Canada: Fabrazyme® and Replagal®.

Fabrazyme® (agalsidase beta)	
What is this medication used for?	Fabrazyme® is used to treat people with a confirmed diagnosis of Fabry disease
How does it work?	Fabrazyme® is a form of the human enzyme α-galactosidase, which may be absent or defective in people with Fabry disease. Fabrazyme® replaces the deficient enzyme to help treat some of the symptoms of Fabry disease. Fabrazyme® also reduces levels of a fat material called globotriaosylceramide (GL-3) and slows the rate of progression of Fabry disease in the kidney, heart and brain.
Who can use it?	People 8 years of age or older with a confirmed diagnosis of Fabry disease
How long has it been available in Canada?	15 years
What is the usual dose?	1.0 mg/kg body weight administered every 2 weeks as an intravenous (IV) infusion • For patients <30 kg, the infusion time would be less than 2 hours • For patients ≥30 kg, the infusion time would be greater than 1.5 hours
Can I take this medication at home?	Yes. Ask your doctor about home infusions.
Can I travel with it?	Please contact your Fabry specialist for your travel options

Adapted from Fabrazyme® Product Monograph.

Replagal® (agalsidase alfa)	
What is this medication used for?	Replagal® is used to treat people with a confirmed diagnosis of Fabry disease
How does it work?	Replagal® is a human α-galactosidase A, that is produced in a human cell line. It replaces the missing or deficient α-Gal A enzyme. Treatment with Replagal® has been shown to reduce accumulation of GL-3 in many cell types and to stabilize the heart and slow the rate of decline in kidney function.
Who can use it?	People 7 years of age or older with a confirmed diagnosis of Fabry disease
How long has it been available in Canada?	15 years
What is the usual dose?	0.2 mg/kg every 2 weeks by a 40-minute IV infusion
Can I take this medication at home?	Yes. Ask your doctor about home infusions.
Can I travel with it?	Please contact your Fabry specialist for your travel options

Adapted from Replagal® Product Monograph.

Oral Chaperone Therapy

Many individuals with Fabry disease make some α-Gal A enzyme that is capable of degrading fatty material called GL-3. However, because of a genetic mutation, the enzyme does not get to the lysosomes, where it is needed.

Galafold™ is an oral, small molecule drug designed to bind to and stabilize the α-Gal A that is made in the patient's own cells. It helps bring the α-Gal A to the area of the cell where it works to degrade the accumulated GL-3. This approach is only meant for patients with certain genetic mutations known as "amenable mutations" because they are capable of responding to oral Galafold™. You can learn if your mutation is amenable by asking your Fabry specialist.

Galafold™ (migalastat)	
What is this medication used for?	Galafold™ is used for the long-term treatment of Fabry disease in adults who have certain genetic mutations (changes) in an enzyme called alpha-galactosidase A (α-Gal A). It should not be used in people with Fabry disease who have other genetic mutations. Your doctor will perform a blood test to determine whether or not you have a mutation that is amenable to chaperone therapy.
How does it work?	Galafold™ works by fixing a defect in the α-Gal A enzyme so that it can work better to reduce the amount of a fatty material known as GL-3 that has built up in your cells and tissues. This helps the organs affected by Fabry disease work better.
Who can use it?	Adults 18 years of age or older with Fabry disease who have certain genetic changes in α-Gal A
How long has it been available in Canada?	2 years
What is the usual dose?	One 123 mg capsule every other day at the same time of the day Do not eat for at least 2 hours before and 2 hours after you take Galafold™
Can I take this medication at home?	Yes
Can I travel with it?	Yes. Ensure you store Galafold™ at room temperature (15–30°C) in the original packaging to protect from moisture.

Adapted from Galafold™ Product Monograph.