

INDICATION

ELELYSO™ (taliglucerase alfa) for injection is indicated for long-term enzyme replacement therapy (ERT) for adults and children with a confirmed diagnosis of Type 1 Gaucher disease.



ELELYSO™ (taliglucerase alfa) for injection is FDA approved for adults and pediatric patients

<http://www.eleyso.com/>

DESCRIPTION

Taliglucerase alfa, a hydrolytic lysosomal glucocerebroside-specific enzyme for intravenous infusion, is a recombinant active form of the lysosomal enzyme, β-glucocerebrosidase, which is expressed in genetically modified carrot plant root cells cultured in a disposable bioreactor system (ProCellEx®). β-Glucocerebrosidase (β-D-glucosyl-N-acylsphingosine glucohydrolase, E.C. 3.2.1.45) is a lysosomal glycoprotein enzyme that catalyzes the hydrolysis of the glycolipid glucocerebroside to glucose and ceramide.

ELELYSO is produced by recombinant DNA technology using plant cell culture (carrot). Purified taliglucerase alfa is a monomeric glycoprotein containing 4 N-linked glycosylation sites (Mr = 60,800). Taliglucerase alfa differs from native human glucocerebrosidase by two amino acids at the N terminal and up to 7 amino acids at the C terminal. Taliglucerase alfa is a glycosylated protein with oligosaccharide chains at the glycosylation sites having terminal mannose sugars. These mannose-terminated oligosaccharide chains of taliglucerase alfa are specifically recognized by endocytic carbohydrate receptors on macrophages, the cells that accumulate lipid in Gaucher disease.



ELELYSO is supplied as 200 Units per vial and is available by prescription only.

ELELYSO is now indicated for long-term enzyme replacement therapy (ERT) for adult and pediatric patients with a confirmed diagnosis of Type 1 Gaucher disease. It's also the first prescription medicine to receive kosher certification by the Orthodox Union.

Talk to your doctor and ask if ELELYSO is the right treatment for your child.

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use ELELYSO safely and effectively. See full prescribing information for ELELYSO.

ELELYSO™ (taliglucerase alfa) for injection, for intravenous use
Initial US Approval: 2012

RECENT MAJOR CHANGES

Indications and Usage (1)	8/2014
Dosage and Administration, Recommended Dosage (2.1)	8/2014
Dosage and Administration, Preparation Instructions (2.2)	8/2014
Dosage and Administration, Administration Instructions (2.3)	8/2014
Warnings and Precautions, Hypersensitivity Reactions Including Anaphylaxis (5.1)	8/2014

INDICATIONS AND USAGE

ELELYSO is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for adult and pediatric patients with a confirmed diagnosis of Type 1 Gaucher disease (1).

DOSAGE AND ADMINISTRATION

- Treatment-naïve adult and pediatric patients 4 years of age and older: 60 units/kg administered every other week as a 60 to 120 minute intravenous infusion. (2.1).
- Patients switching from imiglucerase: Begin treatment with ELELYSO at the same unit/kg dose as the patient's previous imiglucerase dose. Physicians can make dosage adjustments based on achievement and maintenance of each patient's therapeutic goals (2.1).

DOSAGE FORMS AND STRENGTHS

For injection: lyophilized powder for reconstitution with diluent, 200 unit single-use vials (3)

CONTRAINDICATIONS

None (4)

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions Including Anaphylaxis: Observe patients during and after the infusion; immediately discontinue infusion if anaphylaxis occurs and initiate appropriate treatment. Reduction in the infusion rate and/or pre-medication may prevent subsequent reactions (5.1, 6.3).

ADVERSE REACTIONS

The most commonly reported adverse reactions (≥5%) in clinical studies were pruritus, flushing, headache, arthralgia, pain in extremity, abdominal pain, vomiting, fatigue, back pain, dizziness, nausea and rash (6.1).

To report SUSPECTED ADVERSE REACTIONS, contact Pfizer Inc. at 1-800-438-1985 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

See Section 17 for PATIENT COUNSELING INFORMATION.

Revised: 8/2014

Clinical Trials of ELELYSO as Initial Therapy

Clinical Trial in Patients 19 Years and Older

The safety of ELELYSO at dosages of either 30 units/kg (n=16) or 60 units/kg (n=16) every other week was assessed in 32 adult treatment-naïve patients (aged 19 to 74 years) with Type 1 Gaucher disease in a 9-month randomized clinical trial.

Table 1: Adverse Reactions in ≥5% of Treatment-Naïve Adult Patients Treated with ELELYSO

Preferred Term	Treatment-Naïve Adults (N=32)	
	n	(%)
Headache	6	(19%)
Arthralgia	4	(13%)
Fatigue	3	(9%)
Nausea	3	(9%)
Dizziness	3	(9%)
Abdominal pain	2	(6%)
Pruritus	2	(6%)
Flushing	2	(6%)
Vomiting	2	(6%)
Urticaria	2	(6%)

Similar adverse reactions were observed in patients who continued ELELYSO treatment during the extension trial for up to 24 months. One patient experienced a mild and intermittent Type III immune-mediated fixed drug eruption and continued in the study.

Clinical Trial in Patients 16 Years and Younger

The safety of ELELYSO at dosages of either 30 units/kg (n=4) or 60 units/kg (n=5) every other week was assessed in 9 pediatric treatment-naïve patients (aged 2 to 13 years) with Type 1 Gaucher disease in a 12-month randomized clinical trial.

The most common adverse reaction (≥10%) was vomiting, which occurred in 4 of 9 patients. Two patients developed hypersensitivity reactions; one patient experienced severe vomiting and gastrointestinal inflammation, and 1 experienced mild throat irritation and chest discomfort. Both patients responded to treatment with antihistamines and continued ELELYSO treatment.