SmartHealth Ascension

Enzyme Replacement Therapy (ERT) (Gaucher Disease)

Imiglucerase (Cerezyme®) J1786 (10 units)
Taliglucerase (Elelyso®) J3060 (10 units)
Velaglucerase (Vpriv®) J3385 (100 units)

Covered with prior authorization

Imiglucerase (Cerezyme®) may be authorized when the following criteria are met:

- Individual is pediatric or adult individual (**age ≥ 2 years**); **AND**
- Individual has a confirmed diagnosis of Type 1 Gaucher disease; **AND**
- Individual has at least of the following conditions as a result of Gaucher disease:
    - moderate to severe anemia; **OR**
    - thrombocytopenia with bleeding tendency; **OR**
    - bone disease; **OR**
    - significant hepatomegaly; **OR**
    - significant splenomegaly; **AND**
- Individual and/or guardian has attested that they will adhere to the treatment plan; **AND**
- Prescribed by or in consultation with a specialist, preferably one specializing in hematology, oncology, hepatic diseases, genetics, or orthopedics; **AND**
- A current weight with current dose and projected dose for duration of PA is provided; **AND**
- Current and projected doses do not exceed the recommended dose range from 2.5 units/kg three times a week to 60 units/kg once every two weeks.

Taliglucerase (Elelyso) may be authorized when the following criteria are met:

- Individual is pediatric or adult individual (**age ≥ 4 years**); **AND**
- Individual has a confirmed diagnosis of Type 1 Gaucher disease; **AND**
- Individual has at least of the following conditions as a result of Gaucher disease:
    - moderate to severe anemia; **OR**
    - thrombocytopenia with bleeding tendency; **OR**
    - bone disease; **OR**
    - significant hepatomegaly; **OR**
    - significant splenomegaly; **AND**
- Individual and/or guardian has attested that they will adhere to the treatment plan; **AND**
- Prescribed by or in consultation with a specialist, preferably one specializing in hematology, oncology, hepatic diseases, genetics, or orthopedics; **AND**
- A current weight with current dose and projected dose for duration of PA is provided; **AND**
- Current and projected doses do not exceed the recommended dosages:

- Treatment-naïve individuals: 60 units/kg of body weight administered every other week; **OR**
- individuals switching from imiglucerase should begin treatment at same units/kg dosage as the imiglucerase (two weeks following last imiglucerase dose); **OR**
- Administer treatment every other week where dosage adjustments can be made based on achievement and maintenance of individual's therapeutic goals (typical maintenance dose is 60 mg/kg every other week).

Velaglucerase (Vpriv®) may be authorized when the following criteria are met:

- Individual is pediatric or adult individual (**age ≥ 4 years**); **AND**
- Individual has a confirmed diagnosis of Type 1 Gaucher disease; **AND**
- Individual has at least of the following conditions as a result of Gaucher disease:
  - moderate to severe anemia; **OR**
  - thrombocytopenia with bleeding tendency; **OR**
  - bone disease; **OR**
  - significant hepatomegaly; **OR**
  - significant splenomegaly; **AND**
- Individual and/or guardian has attested that they will adhere to the treatment plan; **AND**
- Prescribed by or in consultation with a specialist, preferably one specializing in hematology, oncology, hepatic diseases, genetics, or orthopedics; **AND**
- A current weight with current dose and projected dose for duration of PA is provided; **AND**
- Current and projected doses do not exceed the recommended dosages:
  - Treatment-naïve individual: 60 Units/kg; **OR**
  - individuals switching from imiglucerase should begin treatment at same units/kg dosage as the imiglucerase (two weeks following last imiglucerase dose); **OR**
  - Administer treatment every other week where dosage adjustments can be made based on achievement and maintenance of individual's therapeutic goals (typical maintenance dose is 60 mg/kg every other week).

Non-FDA approved Indication

Imiglucerase (Cerezyme®), Taliglucerase (Elelyso), OR Velaglucerase (Vpriv®) may be authorized when the only exception to the criteria above is:

- Individual has a confirmed diagnosis of Type 3 Gaucher disease (rather than Type 1)
- Note: Due to substantial evidence for using ERT in Type 3 Gaucher individuals, products are commonly used off-label, although all FDA-approved treatments for Gaucher disease are specifically indicated for Type 1.

Exclusion criteria:

Requests may not be approved for the following:

- Individuals with Type 2 Gaucher disease;
- Individuals less than
  - 2 years of age for Imiglucerase (Cerezyme®)
  - 4 years of age for Taliglucerase (Elelyso®) or Velaglucerase (Vpriv®)
- Concurrent use of another enzyme replacement therapy agent or substrate reduction therapy agent (ie, Cerdelga (eliglustat), Zavesca (miglustat) for the treatment of Gaucher disease;

- Product use for non-FDA approved indications or indications not supported by industry-accepted guidelines;
- Doses, durations, or dosing intervals that exceed FDA maximum limits for any FDA-approved indication or are not supported by industry-accepted practice guidelines or peer-reviewed literature for the relevant off-label use;
- Individuals with significant known risk factors unless the record provides an assessment of clinical benefit that outweighs the risk;

Step/Alternative Therapies:

Two therapy types are available for Gaucher disease:

- Three enzyme-replacement therapy (ERT) agents (as addressed in this Criteria for use)
- Two substrate-reduction therapy (SRT) agents:
  - Zavesca (miglustat)
  - Cerdelga (eliglustat)
- Most individuals are first treated with an ERT (intravenous infusion every 2 weeks) with the oral SRTs therapy reserved as an alternative treatment if symptoms persist.
- NOTE: SRTs are indicated ONLY for adult individuals

Initial authorization is up to 12 months.

Annual reauthorizations will require medical chart documentation that the patient has been seen within the past 12 months and that markers of disease are improved by therapy

Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement.

U.S. Food and Drug Administration:

This section is to be used for informational purposes. FDA approval alone is not a basis for coverage.

Imiglucerase (Cerezyme®) is a hydrolytic lysosomal glucocerebrosidase-specific enzyme indicated for treatment of adults and pediatric individuals 2 years of age and older with Type 1 Gaucher disease that results in one or more of the following conditions: anemia, thrombocytopenia, bone disease, hepatomegaly or splenomegaly

Taliglucerase (Elelyso®) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for the treatment of individuals 4 years and older with a confirmed diagnosis of Type 1 Gaucher disease

Velaglucerase (Vpriv®) is a hydrolytic lysosomal glucocerebroside-specific enzyme indicated for long-term enzyme replacement therapy (ERT) for individuals with type 1 Gaucher disease

References:

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Elelyso® (taliglucerase). (2021, July). highlights of prescribing information. Retrieved June 16, 2022, from https://labeling.pfizer.com/showlabeling.aspx?id=798
ELELYSO® (taliglucerase alfa). (2012, May). Accessdata.fda.gov. Retrieved June 16, 2022, from https://www.accessdata.fda.gov/drugsatfda_docs/label/2012/022458lbl.pdf

ELELYSO® (taliglucerase alfa). (2020, November). Accessdata.fda.gov. Retrieved June 16, 2022, from https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/022458s024lbl.pdf
Enzyme Replacement Therapy for Gaucher Disease. (undocumented date). National Gaucher Foundation. Retrieved June 16, 2022, from https://www.gaucherdisease.org/gaucher-diagnosis-treatment/treatment/enzyme-replacement-therapy/
Rich, S. R. (2017, January 10). *Kids with Gaucher Type 3 Show Rapid and Lasting Benefits of...* Gaucher Disease News. Retrieved June 16, 2022, from https://gaucherdiseasenews.com/2017/01/10/long-term-benefits-seen-in-gaucher-type-3-children-treated-with-cerezyme-imiglucerase-ert/
VPRIV® (velaglucerase alfa). (2020, December). Accessdata.fda.gov. Retrieved June 16, 2022, from https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/022575s026lbl.pdf

Criteria History/ Revision Information:

Date	Summary of Changes
June 2022	Criteria for use summary developed by Ascension Medical Specialty Prior Authorization Team
July 2022	Criteria for use summary approved by Ascension Therapeutic Affinity Group

If you have questions, call 833-980-2352 to speak to a member of the Ascension Rx prior authorization team or email your questions to smarthealthspecialty@ascension.org.