09-J4000-56

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Subject: Pegunigalsidase (Elfabrio®) IV Infusion

THIS MEDICAL COVERAGE GUIDELINE IS NOT AN AUTHORIZATION, CERTIFICATION, EXPLANATION OF BENEFITS, OR A GUARANTEE OF PAYMENT, NOR DOES IT SUBSTITUTE FOR OR CONSTITUTE MEDICAL ADVICE. ALL MEDICAL DECISIONS ARE SOLELY THE RESPONSIBILITY OF THE PATIENT AND PHYSICIAN. BENEFITS ARE DETERMINED BY THE GROUP CONTRACT, MEMBER BENEFIT BOOKLET, AND/OR INDIVIDUAL SUBSCRIBER CERTIFICATE IN EFFECT AT THE TIME SERVICES WERE RENDERED. THIS MEDICAL COVERAGE GUIDELINE APPLIES TO ALL LINES OF BUSINESS UNLESS OTHERWISE NOTED IN THE PROGRAM EXCEPTIONS SECTION.

Dosage/ Administration | Position Statement | Billing/Coding | Reimbursement | Program Exceptions | Definitions

Related Guidelines | Other | References | Updates

DESCRIPTION:

Pegunigalsidase (Elfabrio) is a hydrolytic lysosomal neutral glycosphingolipid-specific enzyme that was approved by the U.S. Food and Drug Administration (FDA) in May 2023 for “the treatment of adults with confirmed Fabry disease”. Elfabrio is the third FDA-approved treatment for Fabry disease (FD); the others being IV agalsidase beta (Fabrazyme) approved in 2003 and oral migalastat (Galafold) approved in 2018. Pegunigalsidase is a PEGylated and crosslinked, chemically modified, recombinant human alpha-galactosidase A enzyme that is produced by genetically modified Bright Yellow 2 (Nicotiana tabacum) plant cells. It was designed to increase plasma half-life and reduce immunogenicity as compared with agalsidase beta (Fabrazyme). However, despite having a longer half-life, both pegunigalsidase and agalsidase beta are administered at the same dosage of 1 mg/kg every 2 weeks.

Fabry disease (a.k.a., angiokeratoma corporis diffusum, ceramide trihexosidosis, and Anderson-Fabry disease) is an X-linked genetic disorder of glycosphingolipid metabolism. It is the second most prevalent lysosomal storage disorder after Gaucher disease. Numerous FD-causing mutations have been found in the GLA gene located on the long arm of the X chromosome (Xq22). Mutations associated with the severe, classic manifestation of the disease are present in approximately 1:22,000 to 1:40,000 males, and mutations associated with atypical presentation are present in approximately 1:1,000 to 1:3,000 males and 1:6,000 to 1:40,000 females. Deficient activity of the lysosomal enzyme alpha-Gal A leads to progressive accumulation of glycosphingolipids, predominantly GL-3, in various body tissues, starting early in life and continuing over decades. In males, diagnosis is made by first testing for low alpha-Gal A activity in leukocytes or plasma, and then confirming with mutation analysis of the GLA gene. Alpha-Gal A activity may be normal in up to one-third of females, so mutational analysis is required to screen for disease in women [unless the woman is an obligate heterozygote (i.e., the father is known to have FD)]. In classically affected males (i.e., alpha-Gal A activity is undetectable or <1% of normal), clinical manifestations usually become apparent by 10 years of age. Initial manifestations usually include neuropathy and characteristic skin lesions (i.e., angiokeratomas). Other signs and symptoms may include corneal opacities, hypo- or anhydrosis, heat and cold intolerance, lymphadenopathy, and gastrointestinal symptoms such as abdominal pain and diarrhea. As patients age, cardiovascular, renal, and neurologic disease become increasingly prominent. Renal disease, particularly proteinuria, occurs in most male patients with a mean age of diagnosis of 35 years. Life-threatening manifestations of FD include renal failure, cardiomyopathy, and cerebrovascular accidents.

There are no studies that definitively guide the timing or duration of enzyme replacement therapy for either symptomatic or asymptomatic patients, and there are no uniform recommendations or guidelines for treatment. The general expert consensus is that classically effected males should receive treatment, regardless of symptoms, as soon as possible after diagnosis. However, the European Renal Best Practice (ERBP) group does not recommend starting treatment in patients with proteinuria (protein-to-creatinine ratio >1 g/g) or eGFR <60 mL/min/1.73 m^2, unless the patient has non-renal indications that warrant treatment. Asymptomatic females and atypically affected males should NOT be routinely treated because not all such patients will develop manifestations of the disease and no rigorous data supports empiric treatment is such patients. The trials supporting FDA approval of pegunigalsidase are detailed below.

Trial 1 (NCT01678898) was an open-label, dose-ranging trial in adults diagnosed with Fabry disease. Patients received pegunigalsidase at 0.2 mg/kg, 1 mg/kg, or 2 mg/kg given IV every 2 weeks for 52 weeks. This trial enrolled 18 patients who were ERT-naïve or who had not received ERT for more than 26 weeks and had a negative test for anti-pegunigalsidase alfa-iwxj IgG antibodies prior to enrollment. Two patients in the 1 mg/kg treatment group discontinued the trial after their first infusion; one of them discontinued due to severe hypersensitivity reaction. Among the remaining 16 patients who completed Trial 1, 9 (56%) were males and 7 (44%) were females ranging in age from 17 to 54 years with a median age of 30 years. Of the 9 males, 7 (78%) had the classic phenotype. The median baseline eGFR and proteinuria was 115 mL/min/1.73 m^2 and 0.11 g/g, respectively. Among the male patients, the median value of residual alpha-galactosidase A activity was 2.4% (range: 0.0 to 9.3%) in plasma and 1.3% (range: 0.0% to 3.4%) in leukocytes. The average number of globotriaosylceramide (Gb3) inclusions per renal peritubular capillary (PTC) in renal biopsy specimens of patients was assessed by light microscopy using the quantitative Barisoni Lipid Inclusion Scoring System (BLISS). Evaluable renal biopsies were obtained at baseline and at 26 weeks of treatment in 14 of the 16 patients who completed Trial 1. The Table below shows the changes from baseline to 26 weeks in the BLISS score (average number of Gb3 inclusions per renal PTC) for these 14 pegunigalsidase -treated patients.

Table: Trial 1 Results

	All Patients (n=14)	**Males (n=8)**	**Females (n=6)**
Median (range)			
Baseline	3.2 (0.4, 9.0)	6.8 (0.4, 9.0)	1.2 (0.8, 3.3)
Week 26	0.7 (0.3, 2.5)	0.7 (0.3, 2.5)	0.7 (0.3, 1.4)
Change at Week 26	-2.5 (-8.5, 0.5)	-5.3 (-8.5, 0.5)	-0.7 (-2.5, 0.1)
Mean Change at Week 26 (95% CI)	-3.1 (-4.8, -1.4)	-4.7 (-7.1, -2.3)	-1.0 (-2.1, 0.1)

Trial 2 (NCT03566017) was a randomized, double-blind, and active-controlled trial in ERT-experienced adults diagnosed with Fabry disease. Eligible patients were treated with agalsidase beta for at least one year prior to trial entry (the mean duration of agalsidase beta treatment prior to enrollment was 5.7 years). Patients were randomized 2:1 to receive pegunigalsidase (1 mg/kg IV infusion) or agalsidase beta (1 mg/kg IV infusion) every 2 weeks for 104 weeks. A total of 77 patients were randomized and received at least one dose of pegunigalsidase (n=52) or agalsidase beta (n=25). Of these patients, 47 (61%) were males and 30 (39%) were females. Patients were 18 to 60 years of age with a median age of 46 years at baseline. Forty-one (53%) patients had the classic phenotype. The median baseline eGFR and proteinuria was 75 mL/min/1.73 m^2 and 0.11 g/g, respectively. The primary efficacy endpoint was the annualized rate of change in eGFR (eGFR slope) assessed over 104 weeks. The estimated mean eGFR slope was -2.4 and -2.3 mL/min/1.73 m^2/year on pegunigalsidase and agalsidase beta respectively. The estimated treatment difference was -0.1 (95% CI: -2.3, 2.1) mL/min/1.73 m^2/year.

POSITION STATEMENT:

Site of Care: If pegunigalsidase (Elfabrio) is administered in a hospital-affiliated outpatient setting, additional requirements may apply depending on the member's benefit. Refer to 09-J3000-46, Site of Care Policy for Select Non-Oncology Medications.

Initiation of pegunigalsidase (Elfabrio) **meets the definition of medical necessity** when **ALL** of the following criteria are met ("1" to "5"):

1. The member has a confirmed diagnosis of Fabry disease (FD) as identified by mutational analysis - laboratory documentation of the gene sequencing results showing a pathogenic or likely pathogenic mutation in the galactosidase alpha gene (GLA) must be submitted*

   **One exception is for female members whose biological father has confirmed FD, in which case either the member's or the father's gene sequencing results can be submitted for validation*

2. The member meets **EITHER** of the following criteria ("a" or "b"):

   a. Alpha-galactosidase A (alpha-Gal A) enzyme activity is undetectable or less than 1% of mean normal enzyme activity (i.e., the "classic" form of FD) – laboratory documentation of serum, blood spot, or leukocyte alpha-Gal A enzyme activity less than 1% of mean normal enzyme activity must be submitted

   b. **BOTH** of the following ("i" and "ii"):

      i. Alpha-Gal A enzyme activity is unknown or 1% or greater than mean normal enzyme activity (i.e., "atypical" or "variant" forms of FD)

      ii. Member has clinically-relevant manifestations of FD that include **ANY** of the following – a medical record note documenting the FD-related condition(s) must be submitted

         - Cardiac disease (e.g., ventricular hypertrophy, fibrosis, heart failure, coronary artery disease, valve disorders, conduction defects)
         - Cerebrovascular disease (e.g., history of stroke or TIA, brain lesions found on imaging studies)
         - Persistent and severe gastrointestinal symptoms not explained by other conditions
         - Persistent hearing problems (e.g., hearing loss, tinnitus, vertigo)
         - Severe neuropathy requiring prescription drug treatment
         - Renal disease (e.g., proteinuria, renal cysts, GL-3 accumulation on renal biopsy)

3. Treatment with pegunigalsidase is prescribed by, or in consultation with, a specialist with experience in treating patients with FD (e.g., nephrologist, neurologist, endocrinologist, clinical geneticist, cardiologist)
4. Pegunigalsidase will **NOT** be used in combination with agalsidase beta (Fabrazyme) or migalastat (Galafold)
5. The dosage of pegunigalsidase does not exceed 1 mg/kg (rounded down to the closest 5-mg increment, e.g., 73 kg = 75 mg, 41 kg = 40 mg) every 2 weeks

Approval duration: 6 months

Continuation of pegunigalsidase (Elfabrio) **meets the definition of medical necessity** when **ALL** of the following criteria are met ("1" to "4"):

1. An authorization or reauthorization for pegunigalsidase **OR** agalsidase beta (Fabrazyme), has been previously approved by Florida Blue or another health plan in the past 2 years for the treatment of Fabry disease (if another health plan, documentation of a health plan-paid claim for pegunigalsidase **OR** agalsidase beta during the 90 days immediately before the request must be submitted), **OR** the member meets **ALL** indication-specific initiation criteria
2. Treatment with pegunigalsidase is prescribed by, or in consultation with a specialist with experience in treating patients with FD (e.g., nephrologist, neurologist, endocrinologist, clinical geneticist, cardiologist); **AND** the member is clinically assessed by this specialist at least annually – a chart note confirming the specialist visit within the past year must be submitted

3. Pegunigalsidase will **NOT** be used in combination with agalsidase beta (Fabrazyme) or migalastat (Galafold)
4. The dosage of pegunigalsidase does not exceed 1 mg/kg (rounded down to the closest 5-mg increment, e.g., 73 kg = 75 mg, 41 kg = 40 mg) every 2 weeks

Approval duration: 12 months

DOSAGE/ADMINISTRATION:

THIS INFORMATION IS PROVIDED FOR INFORMATIONAL PURPOSES ONLY AND SHOULD NOT BE USED AS A SOURCE FOR MAKING PRESCRIBING OR OTHER MEDICAL DETERMINATIONS. PROVIDERS SHOULD REFER TO THE MANUFACTURER'S FULL PRESCRIBING INFORMATION FOR DOSAGE GUIDELINES AND OTHER INFORMATION RELATED TO THIS MEDICATION BEFORE MAKING ANY CLINICAL DECISIONS REGARDING ITS USAGE.

FDA-approved

- Indicated for the treatment of adults with confirmed Fabry disease.
- In enzyme replacement therapy (ERT)-experienced patients, if pretreatment with antihistamines, antipyretics, and/or corticosteroids was used prior to ERT administration, consider similar pretreatment with these medications before the first several pegunigalsidase infusions. After 4 to 6 infusions, a stepwise decrease in the pretreatment medication dose(s) and/or discontinuation of the pretreatment medication(s) may be considered if treatment was tolerated. In ERT-naïve patients, pre-treating with antihistamines, antipyretics, and/or corticosteroids may be considered. Appropriate medical support measures including cardiopulmonary resuscitation equipment should be readily available during administration.
- The recommended dosage, based on actual body weight, is 1 mg/kg administered by IV infusion every 2 weeks. Must be diluted in 0.9% Sodium Chloride Injection prior to infusion. The initial recommended infusion rates for ERT-experienced or ERT-naïve patients are based on actual body weight (refer to the package insert for the recommended rates). In the event of a severe hypersensitivity reaction (e.g., anaphylaxis) or severe infusion-associated reaction (IAR), immediately discontinue administration and initiate appropriate medical treatment. In the event of a mild to moderate hypersensitivity reaction or a mild to moderate IAR, consider temporarily holding the infusion for 15 to 30 minutes or slowing the infusion rate by 25% to 50%, and initiating appropriate medical treatment. Refer to the package insert for additional recommendations.

Dose Adjustments

- Adverse Reactions – Dosage adjustment are not recommended for adverse reactions; however, slowing the infusion ate may be required for hypersensitivity reaction or infusion-associated reactions.
- Hepatic Impairment - Specific guidelines for dosage adjustments in hepatic impairment are not available; it appears that no dosage adjustments are needed
- Renal Impairment - Specific guidelines for dosage adjustments in renal impairment are not available, however, a case of membranoproliferative glomerulonephritis leading to declining renal function was reported in clinical trials.

Drug Availability

- Single-dose vial containing 5 mg/2.5 mL (2 mg/mL) of pegunigalsidase. Available in cartons containing one vial.
- Single-dose vial containing 20 mg/10 mL (2 mg/mL) of pegunigalsidase. Available in cartons containing one, five, or ten vials.
- Store refrigerated at 2°C to 8°C (36°F to 46°F). Do not freeze. Do not shake.

PRECAUTIONS:

Boxed Warning

- WARNING: HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS

  Patients treated with Elfabrio have experienced hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during Elfabrio administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue Elfabrio immediately and initiate appropriate medical treatment. In patients with severe hypersensitivity reaction, a desensitization procedure to Elfabrio may be considered.

Contraindications

- None

Precautions/Warnings

- **Hypersensitivity Reactions Including Anaphylaxis** – Hypersensitivity reactions including anaphylaxis have been reported. In clinical trials, 20 (14%) of pegunigalsidase -treated patients experienced hypersensitivity reactions. In these trials, 4 patients (3%; 1 naïve to enzyme replacement therapy (ERT) and 3 ERT-experienced patients) experienced anaphylaxis during the initial infusion and were positive for anti-pegunigalsidase alfa-iwxj IgE antibodies (referred to as IgE ADA). The risk of pegunigalsidase alfa-iwxj-related hypersensitivity may be increased in certain patients with pre-existing ADA from prior ERT. Anaphylaxis (reported as Type I hypersensitivity reaction, hypersensitivity reaction, or bronchospasm) occurred within 5 to 40 minutes of the start of the initial infusion. Signs and symptoms included headache, nausea, vomiting, throat tightness, facial and oral edema, truncal rash, tachycardia, hypotension, rigors, urticaria, intense pruritus, moderate upper airway obstructions, macroglossia, and mild lip edema. Patients received treatment that included epinephrine, antihistamines and/or systemic corticosteroids. Prior to administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, discontinue pegunigalsidase immediately and initiate appropriate medical treatment. Consider the risks and benefits of re-administering following severe hypersensitivity reactions (including anaphylaxis). Patients may be rechallenged using slower infusion rates. In patients with severe hypersensitivity reaction, desensitization measures to pegunigalsidase may be considered. If the decision is made to readminister, ensure the patient tolerates the infusion. If the patient tolerates the infusion, the rate may be increased to reach the recommended rate. If a mild or moderate hypersensitivity reaction occurs, consider temporarily holding the infusion or slowing the infusion rate. Consider monitoring patients who demonstrate hypersensitivity reactions during pegunigalsidase treatment for the presence of IgG and IgE ADA.
- **Infusion-Associated Reactions** - Infusion-associated reactions (IARs) have been reported. In clinical trials, 41 (29%) of pegunigalsidase -treated patients experienced one or more IARs, defined as any adverse reaction with onset after start of the infusion and up to 24 hours after the end of infusion. The risk of pegunigalsidase alfa-iwxj-related IARs may be increased in certain patients with pre-existing ADA from prior ERT. IARs included anaphylaxis reactions during the initial pegunigalsidase administration. In addition to the hypersensitivity reactions described above, other IARs included nausea, chills, pruritus, rash, chest pain, dizziness, vomiting, asthenia, pain, sneezing, dyspnea, nasal congestion, throat irritation, abdominal pain, erythema, diarrhea, burning sensation, neuralgia, headache, paresthesia, tremor, agitation, increased body temperature, flushing, bradycardia, myalgia, hypertension, and hypotension. Up to 40% of patients were pretreated with diphenhydramine, prednisone and/or acetaminophen at least once during the clinical trials. Severe reactions in the trials were generally managed with administration of antipyretics, antihistamines, corticosteroids, IV fluids, and/or oxygen. IARs were more frequently observed in pegunigalsidase -treated patients who developed IgG anti-drug antibodies (ADA) including patients who had pre-

existing IgG ADA. Consider monitoring patients who demonstrate IARs during treatment for the presence of IgG and IgE ADA. Patients with advanced Fabry disease may have compromised cardiac function which may predispose them to a higher risk of severe complications from IARs. Closely monitor patients with compromised cardiac function if administered to these patients. Prior to administration, consider pre-treating with antihistamines, antipyretics, and/or corticosteroids to reduce the risk of IARs. However, IARs may still occur in patients after receiving pre-treatment. If a severe IAR occurs, discontinue immediately and initiate appropriate medical treatment. Consider the risks and benefits of re-administering pegunigalsidase following a severe IAR. Patients may be rechallenged using slower infusion rates. Once a patient tolerates the infusion, the infusion rate may be increased to reach the recommended infusion rate. If a mild or moderate IAR occurs, consider temporarily holding the infusion or slowing the infusion rate.

- **Membranoproliferative Glomerulonephritis** - A case of membranoproliferative glomerulonephritis with immune depositions in the kidney was reported during clinical trials. This event led to a decline in renal function that slowly improved upon discontinuation of pegunigalsidase but did not return to baseline by the end of the trial. Monitor serum creatinine and urinary protein to creatinine ratio. If glomerulonephritis is suspected, discontinue pegunigalsidase until a diagnostic evaluation can be conducted.

HCPCS Coding

J2508	Injection, pegunigalsidase alfa-iwxj, 1 mg

ICD-10 Diagnosis Codes That Support Medical Necessity

E75.21	Fabry (-Anderson) disease

REIMBURSEMENT INFORMATION:

Refer to section entitled **POSITION STATEMENT**.

PROGRAM EXCEPTIONS:

Federal Employee Program (FEP): Follow FEP guidelines.

State Account Organization (SAO): Follow SAO guidelines.

Medicare Part D: Florida Blue has delegated to Prime Therapeutics authority to make coverage determinations for the Medicare Part D services referenced in this guideline.

Medicare Advantage: No National Coverage Determination (NCD) and/or Local Coverage Determination (LCD) were found at the time of the last guideline review date. The Site of Care Policy for Select Specialty Medications does not apply to Medicare Advantage members.

If this Medical Coverage Guideline contains a step therapy requirement, in compliance with Florida law 627.42393, members or providers may request a step therapy protocol exemption to this requirement if based on medical necessity. The process for requesting a protocol exemption can be found at Coverage Protocol Exemption Request.

DEFINITIONS:

None

RELATED GUIDELINES:

Agalsidase Beta (Fabrazyme), 09-20000-59
Genetic Testing, 05-82000-28

Migalastat (Galafold) Capsule, 09-J3000-12

OTHER:

None

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COMMITTEE APPROVAL:

This Medical Coverage Guideline (MCG) was approved by the Florida Blue Pharmacy Policy Committee on 04/08/26.

GUIDELINE UPDATE INFORMATION:

09/15/23	New Medical Coverage Guideline.
01/01/24	Revision: Added HCPCS code J2508 and deleted code J3590.
05/15/24	Review and revision of guidelines consisting of updates to the references.
05/15/25	Review and revision of guidelines consisting of updates to the references.
05/15/26	Review and revision of guidelines consisting of updates to the position statement, drug availability, precautions, and references.
07/01/26	Revision: Update to Site of Care Statement in Position Statement.