Patient Letter of Medical Necessity for Elfabrio® (pegunigalsidase alfa-iwxj): Considerations and Sample Template

Dear Provider,

You can consider asking patients to prepare a letter of medical necessity to the insurer to support an appeal for ELFABRIO treatment. A patient narrative can accompany your appeal. You can give the patient the template provided on page 3 to complete or use the list of topic suggestions below to help the patient get started.

Suggestions for Patients to Write Their Own ELFABRIO Appeal Letter

Here are some examples of questions you can think about to help describe the impact of Fabry disease on your life. You can include other issues that are important to you:

- At what age were you diagnosed with Fabry disease?
- Do other family members also have Fabry disease?
- What symptoms of Fabry disease do you experience on a regular basis?
- How has Fabry disease impacted your quality of life?
- What treatments have you tried for managing the symptoms of Fabry disease? (List as many as you have tried, with their approximate dates, a description of how well they worked, and any reactions that you had from them.)
- If you are writing to support a request for lower copay or coinsurance, describe why the current cost is a financial burden

Include the following information of your appeal letter:

- The name of your insurance plan
- Your group and member number for your insurance plan
- Your address and phone number(s)

Indication and Important Safety Information

Indication
Elfabrio® (pegunigalsidase alfa-iwxj) is indicated for the treatment of adults with confirmed Fabry disease.

Important Safety Information

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 (eg, anaphylaxis) occurs, discontinue Elfabrio immediately and initiate appropriate medical treatment. In patients with severe hypersensitivity reaction, a desensitization procedure to Elfabrio may be considered.

reactions and infusion-associated reactions (IARs), and instruct them to seek medical care immediately if such symptoms occur.

- If a severe hypersensitivity reaction (including anaphylaxis) or severe IAR occurs, immediately discontinue Elfabrio administration and initiate appropriate medical treatment.
- If a mild to moderate hypersensitivity reaction or IAR occurs, consider slowing the infusion rate or temporarily withholding the dose.

In clinical trials, 20 (14%) Elfabrio-treated patients experienced hypersensitivity reactions. Four Elfabrio-treated patients (3%) experienced anaphylaxis reactions that occurred within 5 to 40 minutes of the start of the initial infusion. The signs and symptoms of hypersensitivity reactions and anaphylaxis 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.

In clinical trials, 41 (29%) Elfabrio-treated patients experienced one or more infusion-associated reactions, including hypersensitivity, 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.

A case of membranoproliferative glomerulonephritis with immune depositions in the kidney was reported during clinical trials. Monitor serum creatinine and urinary protein-to-creatinine ratio. If glomerulonephritis is suspected, discontinue treatment until a diagnostic evaluation can be conducted.

When switching to Elfabrio from a prior enzyme replacement therapy, the risk of hypersensitivity reactions and infusion-associated reactions may be increased in certain patients with pre-existing anti-drug antibodies (ADAs). Consider monitoring IgG and IgE ADAs and clinical or pharmacodynamic response (eg, plasma lyso-Gb3 levels).

The most common adverse reactions (≥15%) were infusion-associated reactions, nasopharyngitis, headache, diarrhea, fatigue, nausea, back pain, pain in extremity, and sinusitis.

Please see Full Prescribing Information for Elfabrio.

©Chiesi USA. 2025. All rights reserved. Elfabrio® is a registered trademark of CHIESI FARMACEUTICI S.p.A. PP-RA-00396 V1.0.

Patient Letter of Medical Necessity for Elfabrio® (pegunigalsidase alfa-iwxj) Template

[Date]
[Patient name]
[Patient Address]
[Patient Phone Number(s)]
[Patient Date of Birth]

[Insurance Plan Name]
[Insurance Group Number]
[Insurance Member Number]
[Prescribing Provider Name]

To whom it may concern:

My doctor, [enter name of doctor], recommended Elfabrio® (pegunigalsidase alfa-iwxj) for the treatment of Fabry disease. I have had Fabry disease since I was [enter age at diagnosis] years old. I have been under the care of [enter name of doctor] since [enter date you started seeing current doctor].

I have tried other treatments to control my Fabry Disease, which include [name treatments]. On [named treatment], I experienced [factual statement of your reactions and experience].

[Repeat for additional treatments tried.] Due to my experience on other treatments, my doctor and I agree that switching to Elfabrio makes sense.

[If you are writing to request a lower copay, describe why the current copay amount is a financial burden.]

I have lived with Fabry disease for [enter number of years]. I tried the treatments listed above and, as I described Fabry disease continues to affect my life in many ways.

I would be very grateful if you would cover Elfabrio as requested by my doctor. My doctor can be reached at [insert doctor's phone number].

Sincerely,

[Print your name]

[Sign your name]

[Insert your address]

[Insert your phone number]