

Elfabrio™ (pegunigalsidase alfa intravenous infusion)

When requesting Elfabrio™ (pegunigalsidase), the individual requiring treatment must be diagnosed with the following FDA-approved indication and meet the specific coverage guidelines and applicable safety criteria for the covered indication.

FDA-approved indication

Elfabrio is indicated for the treatment of adults with confirmed Fabry disease.

Coverage Guidelines

Fabry Disease

Individual must meet all of the following for authorization:

- Is 18 years of age or older;
- Meets one of the following:
 - o Laboratory test demonstrating deficient α -galactosidase A activity in leukocytes or fibroblasts; OR
 - o Molecular genetic test demonstrating pathogenic mutations in the galactosidase alpha gene;
- Elfabrio is prescribed by or in consultation with a geneticist, endocrinologist, a metabolic disorder sub-specialist, or a physician who specializes in the treatment of lysosomal storage disorders.

Approval duration: 12 months

Dosing Recommendation

The recommended dosage of Elfabrio, based on actual body weight, is 1 mg/kg administered by intravenous infusion every 2 weeks.

References

1. Elfabrio® intravenous infusion [prescribing information]. Parma, Italy: Chiesi; May 2023.
2. Schiffmann R. Fabry Disease. *Handb Clin Neurol*. 2015;132:231-248.
3. Arends M, Wanner C, Hughes D, et al. Characterization of Classical and Nonclassical Fabry Disease: A Multinational Study. *J Am Soc Nephrol*. 2017;28:1631-1641.
4. Laney DA, Bennett RL, Clarke V, et al. Fabry Disease Practice Guidelines: Recommendations of the National Society of Genetic Counselors. *J Genet Counsel*. 2013;22:555-564.
5. Spada M, Pagliardini S, Yasuda M, Tükel T, Thiagarajan G, Sakuraba H, Ponzzone A, Desnick RJ. High incidence of later-onset fabry disease revealed by newborn screening. *Am J Hum Genet*. 2006 Jul;79(1):31-40.