

Veopoz™ (pozelimab-bbfg)

When requesting Veopoz™ (pozelimab-bbfg), 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

Veopoz is indicated for the treatment of CD55-deficient protein-losing enteropathy, also known as CHAPLE (complement hyperactivation, angiopathic thrombosis, and protein-losing enteropathy) disease.

Coverage Guidelines

CHAPLE Disease

The individual must meet **all** of the following criteria for approval for initial authorization:

- Is 1 year of age or older;
- Has had a genetic test confirming the diagnosis of CHAPLE disease with a biallelic CD55 loss-of-function mutation;
- Meets both of the following:
 - Patient has a serum albumin level ≤ 3.2 g/dL; AND
 - Has active disease and is experiencing one or more signs or symptoms within the last 6 months;
Note: Examples of signs and symptoms include abdominal pain, diarrhea, vomiting, peripheral edema, or facial edema.
- Meets all of the following:
 - Does not have a history of meningococcal infection; AND
 - Has received or is in compliance with updated meningococcal vaccinations according to the most current Advisory Committee on Immunization Practices (ACIP) recommendations; AND
 - Has received or is in compliance with updated vaccinations for the prevention of *Streptococcus pneumonia* and *Haemophilus influenza* type b infections according to the most current ACIP guidelines;
- Medication is prescribed by a physician with expertise in managing CHAPLE disease.

The individual must meet **all** of the following criteria for reauthorization:

- Is 1 year of age or older;
- Has had a genetic test confirming the diagnosis of CHAPLE disease with a biallelic CD55 loss-of-function mutation;
- Medication is prescribed by a physician with expertise in managing CHAPLE disease;
- Has experienced a response to therapy.

Note: Examples of a response to therapy include increased serum albumin levels, maintenance of serum albumin levels within a normal range, a reduction in albumin transfusions, increases in or maintenance of protein and/or immunoglobulin levels, improvement in clinical outcomes after receipt of therapy (e.g., decreases in the frequency of problematic abdominal pain, bowel movement frequency, facial edema severity, and peripheral edema severity), reduced frequency in hospitalizations, increase in growth percentiles (e.g., body weight-for age and/or stature-for-age percentiles), and/or reduced use of corticosteroids.

Approval duration (initial): 3 months

Approval duration (renewal): 12 months

Dosing Recommendations

The recommended dosage of Veopoz is as follows:

- Day 1: Administer a single 30 mg/kg dose by intravenous infusion after dilution.
- Day 8 and Thereafter: Inject 10 mg/kg as a subcutaneous injection once weekly starting on Day 8.

The maintenance dosage may be increased to 12 mg/kg once weekly if there is inadequate clinical response after at least 3 weekly doses (i.e., starting from Week 4).

The maximum maintenance dosage is 800 mg once weekly.

References

1. Veopoz™ intravenous infusion and subcutaneous injections [prescribing information]. Tarrytown, NY: Regeneron; August 2023.
2. Ozen A, Chongsrisawat V, Sefer AP, et al, for the Pozelimab CHAPLE working group. A phase 2/3 study evaluating the efficacy and safety of pozelimab in patients with CD55 deficiency with hyperactivation of complement, angiopathic thrombosis, and protein-losing enteropathy (CHAPLE Disease). *Lancet* [preprint]. Posted 27 Jul 2023. Available at SSRN: <https://ssrn.com/abstract=4485593> or <http://dx.doi.org/10.2139/ssrn.4485593>.
3. FDA news. FDA approves first treatment for CD55-deficiency protein-losing enteropathy (CHAPLE disease). Available at: <https://www.fda.gov/drugs/news-events-human-drugs/fda-approves-first-treatment-cd55-deficient-protein-losing-enteropathy-chaple-disease>. Accessed on August 23, 2023.
4. Ozen A, Comrie WA, Ardy RC. CD55 deficiency, early-onset protein-losing enteropathy, and thrombosis. *N Engl J Med*. 2017;377(1):52-61.
5. Dho SH, Lim JC, Kim LK, et al. Beyond the role of CD55 as a complement component. *Immune Netw*. 2018;18(1):e11.
6. Latuszek A, Liu Y, Olsen O, et al. Inhibition of complement pathway activation with pozelimab, a fully human antibody to complement component C5. *PLoS One*. 2020;15(5):e0231892.