Neighborhood Health Plan OF RHODE ISLAND™
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Veopoz® (pozelimab-bbfg)
(Intravenous/Subcutaneous)

Effective Date: 08/01/2024
Dates Reviewed: 05/2024
Scope: Medicaid, Commercial, Medicare-Medicaid Plan (MMP)

I. Length of Authorization

Coverage will be provided for six (6) months and may be renewed.

II. Dosing Limits

A. Quantity Limit (max daily dose) [NDC Unit]:

- Veopoz 400 mg/2 mL SDV – 8 vials, as a loading dose, on day 1 followed by 2 vials starting on day 8 and weekly thereafter

B. Max Units (per dose and over time) [HCPCS Unit]:

- Loading Dose: 3200 mg (3200 units) on day 1
- Maintenance Dose: Beginning on day 8, up to 800 mg (800 units) weekly

III. Summary of Evidence:

Veopoz (pozelimab) is a complement inhibitor indicated for the treatment of adult and pediatric patients 1 year of age and older with CD55-deficient protein-losing enteropathy (PLE), also known as CHAPLE disease. CHAPLE disease is an ultra-rare and life-threatening hereditary immune condition caused by mutations in the CD55 gene leading to overactivation of the complement system, resulting in damage to blood and lymph vessels along the upper digestive tract and loss of circulating proteins. CHAPLE disease affects fewer than 10 patients in the United States, and less than 100 patients worldwide. Efficacy of Veopoz was established in a Phase 2/3, single-arm study where outcomes were compared to pre-treatment data in 10 patients with active CD55-deficient protein-losing enteropathy (PLE) who had hypoalbuminemia. Active CD55-deficient PLE was defined as hypoalbuminemia (serum albumin ≤ 3.2 g/dL) with at least one clinical sign/symptom of hypoalbuminemia within the last six months. The results indicated that the median time for serum albumin to reach at least 3.5 mg/dL was 15.5 days with all 10 patients achieving normalization by Week 12 and maintaining a normal serum albumin concentration range through at least 72 weeks of treatment. In addition, serum IgG concentrations reached normal values in all patients within the first 12 weeks of treatment and maintained through at least 72 weeks of treatment, and improvements were seen in hospitalizations. Veopoz has black box warnings for serious meningococcal infections. The most common adverse effects of Veopoz include upper respiratory tract infections, fractures, urticaria, and alopecia.

IV. Initial Approval Criteria [1]

Coverage is provided in the following conditions:

- Patient is at least 1 year of age; **AND**

- The medication must be prescribed by or in consultation with a hematologist, gastroenterologist, or a specialist familiar with the treatment of rare genetic hematologic diseases; **AND**
- MMP members who have previously received this medication within the past 365 days are not subject to Step Therapy Requirements; **AND**

Universal Criteria [1]

- Patients must be administered a meningococcal vaccine (for serogroups A, C, W and Y, and serogroup B) at least two weeks prior to initiation of therapy and will continue to be revaccinated according to current medical guidelines for vaccine use *(If urgent Veopoz therapy is indicated in an unvaccinated patient, administer meningococcal vaccine(s) as soon as possible and provide patients with two weeks of antibacterial drug prophylaxis.)*; **AND**
- Patient must be administered vaccinations for the prevention of Streptococcus pneumoniae and Haemophilus influenzae type b (Hib) infections according to medical guidelines; **AND**
- Will not be used in combination with other complement therapies (i.e., sutimlimab, ravulizumab, avacopan, eculizumab, pegcetacoplan, etc.); **AND**
- Patient does not have an unresolved Neisseria meningitidis infection; **AND**
- Patient will avoid concomitant therapy with intravenous immunoglobulin, if therapy is unavoidable, the patient will be monitored closely for adverse reactions and/or worsening of disease symptoms; **AND**

Complement Hyperactivation, Angiopathic thrombosis, and Protein-Losing Enteropathy Disease (CHAPLE) † Φ [1]

- Patient has a confirmed clinical diagnosis of CD55-deficient protein-losing enteropathy (PLE) evidenced by biallelic CD55 loss-of-function mutation detected by genotype analysis (genetic testing results must be submitted with request); **AND**
- Patient has active disease as defined as hypoalbuminemia (serum albumin concentration of ≤3.2 g/dL) with one or more of the following signs or symptoms attributed to CD55-deficient PLE within the last six months (chart notes or medical record documentation supporting signs and symptoms of CHAPLE disease must be submitted with request):
  - abdominal pain
  - diarrhea
  - peripheral edema
  - facial edema

† FDA Approved Indication(s); **‡** Compendia Recommended Indication(s); **Φ** Orphan Drug

V. Renewal Criteria [1]

Coverage may be renewed based upon the following criteria:

- Patient continues to meet the universal and other indication-specific relevant criteria identified in section III; **AND**
- Absence of unacceptable toxicity from the drug. Examples of unacceptable toxicity include: serious meningococcal infections (septicemia and/or meningitis), other serious bacterial infections, serious hypersensitivity reactions, etc.; **AND**

- Patient exhibits disease response compared to pretreatment baseline in ALL of the following with documentation provided**:**
    - Normalization/improvement in serum proteins (e.g., albumin, or immunoglobulin G, etc.); **AND**
    - Stabilization/improvement in signs and symptoms of disease (e.g., reduction in abdominal pain or diarrhea, reduction of peripheral or facial edema, etc.); **AND**
    - Reduction in albumin transfusion requirements, exogenous immunoglobulin, and/or hospitalization (as applicable)

VI. Dosage/Administration [1]

Indication	Dose
CHAPLE Disease	**Day 1 (Loading Dose):** Administer a single 30 mg/kg dose by intravenous infusion. **Day 8 and Thereafter (Maintenance Dosage):** **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 Week 4).* • *The maximum maintenance dosage is 800 mg once weekly, doses greater than 400 mg require 2 injections.* ** Veopoz for subcutaneous use must be prepared and administered by a healthcare provider.*

VII. Billing Code/Availability Information

HCPCS Code:

- J9376 - Injection, pozelimab-bbfg, 1 mg

NDC(s):

- Veopoz 400 mg/2 mL single-dose vials for injection: 61755-0014-xx

VIII. Investigational Use

All therapies are considered investigational when used at a dose or for a condition other than those that are recognized as medically accepted indications as defined in any one of the following standard reference compendia: American Hospital Formulary Service Drug information (AHFS-DI), Thomson Micromedex DrugDex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs, or Peer-reviewed published medical literature indicating that sufficient evidence exists to support use. Neighborhood does not provide coverage for drugs when used for investigational purposes.

IX. References

1. Veopoz [package insert]. Tarrytown, NY; Regeneron Pharmaceuticals, Inc; August 2023. Accessed August 2023.
2. Ozen A, Chongsrisawat V, Sefer AP, et al. 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). The Lancet PrePrint article, available at SSRN: https://ssrn.com/abstract=4485593 or http://dx.doi.org/10.2139/ssrn.4485593

3. Ozen A. CHAPLE syndrome uncovers the primary role of complement in a familial form of Waldmann's disease. Immunol Rev. 2019 Jan;287(1):20-32. doi: 10.1111/imr.12715. PMID: 30565236.
4. Ozen A, Comrie WA, Ardy RC, et al. CD55 Deficiency, Early-Onset Protein-Losing Enteropathy, and Thrombosis. N Engl J Med. 2017 Jul 6;377(1):52-61. doi: 10.1056/NEJMoa1615887. Epub 2017 Jun 28. PMID: 28657829; PMCID: PMC6690356.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
D84.1	Defects in the complement system

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determination (NCD), Local Coverage Articles (LCAs) and Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. They can be found at: https://wyww.cms.gov/medicare-coverage-database/search.aspx. Additional indications may be covered at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdictio	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA, LLC
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA, LLC
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC

Policy Rationale:

Veopoz was reviewed by the Neighborhood Health Plan of Rhode Island Pharmacy & Therapeutics (P&T) Committee. Neighborhood adopted the following clinical coverage criteria to ensure that its members use Veopoz according to Food and Drug Administration (FDA) approved labeling and/or relevant clinical literature. Neighborhood worked with network prescribers and pharmacists to draft these criteria. These criteria will help ensure

its members are using this drug for a medically accepted indication, while minimizing the risk for adverse effects and ensuring more cost-effective options are used first, if applicable and appropriate. For INTEGRITY (Medicare-Medicaid Plan) members, these coverage criteria will only apply in the absence of National Coverage Determination (NCD) or Local Coverage Determination (LCD) criteria. Neighborhood will give individual consideration to each request it reviews based on the information submitted by the prescriber and other information available to the plan.