Clinical Policy: Velmanase Alfa-tycv (Lamzede)

Reference Number: LA.PHAR.601
Effective Date:
Last Review Date: 04.04.24
Line of Business: Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

****Please note: This policy is for medical benefit****

Description

Velmanase alfa-tycv (Lamzede®) is a recombinant human alpha-mannosidase replacement therapy.

FDA Approved Indication(s)
Lamzede is indicated for the treatment of non-central nervous system manifestations of alpha-mannosidosis (AM) in adult and pediatric patients.

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of Louisiana Healthcare Connections® that Lamzede is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Alpha-Mannosidosis (must meet all)**:**

1. Diagnosis of AM confirmed by one of the following (a or b):
   a. Reduced AM activity defined as < 10% of normal activity in leukocytes or fibroblasts cells;
   b. Genetic testing revealing biallelic *MAN2B1* gene mutation;
2. Prescribed by or in consultation with an endocrinologist, neurologist, ophthalmologist, clinical geneticist, or specialist familiar with the treatment of lysosomal storage disorders;
3. Member does not have central nervous system manifestations of AM (*see Appendix D*);
4. Member is able to ambulate independently;
5. Member has not previously received a bone marrow transplant or hematopoietic stem cell transplantation;
6. Documentation of current actual body weight in kg;
7. Dose does not exceed 1 mg/kg (actual body weight) per week.

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255;
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: LA.PMN.53 for Medicaid.

II. Continued Therapy

A. Alpha-Mannosidosis (must meet all)**:**

a. Currently receiving medication via Louisiana Healthcare Connections benefit or member has previously met initial approval criteria;

2. Member is responding positively to therapy as evidenced by stabilization or improvement in, but not limited to, <u>any</u> of the following parameters *(see Appendix D for other examples of individual patient AM disease manifestation profiles)*:
   a. Serum oligosaccharides levels;
   b. 3-minute stair climb test;
   c. 6-minute walk test;
   d. Bruininks-Oseretsky test of motor proficiency;
   e. Forced vital capacity;
3. Documentation of current actual body weight in kg;
4. If request is for a dose increase, new dose does not exceed 1 mg/kg (actual body weight) per week.

Approval duration: 6 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to LA.PMN.255;
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: LA.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – LA.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
AM: alpha-mannosidosis
FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): none reported
- Boxed warning(s): severe hypersensitivity reactions including anaphylaxis

Appendix D: General Information

Individual patient manifestations of AM may include non-central nervous system manifestations such coarse facial features, frequent infections due to immune deficiency, and skeletal abnormalities. Central nervous system manifestations may include mental retardation, speech delay, sensorineural hearing loss, dysostosis multiplex, genu valgum, hypotonia, motor and coordinator disturbances, ataxia, ocular manifestations with strabismus and acute psychotic manifestations, occipital white matter signal aberrations, and delayed myelination as well as hydrocephalus.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
AM	1 mg/kg (actual body weight) IV once every week	1 mg/kg/week

VI. Product Availability

Single-use vial: 10 mg as a lyophilized powder for reconstitution

VII. References

1. Lamzede Prescribing Information. Cary, NC: Chiesi USA, Inc. February 2023. Available at: https://resources.chiesiusa.com/Lamzede/LAMZEDE_PI.pdf. Accessed February 24, 2023.
2. Malm D, Nilseen O. Alpha-mannosidosis . National Library of Medicine. Available at: https://www.ncbi.nlm.nih.gov/books/NBK1396/. Accessed April 5, 2023.
3. European Medicine Agency. Lamzede: EPAR – Product Information; May 2022. Available at: https://www.ema.europa.eu/en/medicines/human/EPAR/Lamzede. Accessed September 21, 2022.
4. Borgwardt L, Guffon N, Amraoui Y, et al. Efficacy and safety of velmanase alfa in the treatment of patients with alpha-mannosidosis: results from the core and extension phase analysis of a phase III multicentre, double-blind, randomized, placebo-controlled trial. H Inherit Metab Dis. 2018; 41(6): 1215-1223. https://doi.org/10.1007/s10545-018-0185-0.
5. Lund AM, Borgwardt L, Cattaneo F, et al. Comprehensive long-term efficacy and safety of recombinant human alpha-mannosidase (velmanase alfa) treatment in patients with alpha-mannosidosis. J Inherit Metab Dis. 2018; 41(6): 1225-1233. https://doi.org/10.1007/s10545-018-0175-2.
6. Harmatz P, Cattaneo F, Ardigo D, et al. Enzyme replacement therapy with velmanase alfa (human recombinant alpha-mannosidase): Novel global treatment response model and outcomes in patients with alpha-mannosidosis. Molecular Genetics and Metabolism: 2018; 124(2): 152-160. https://doi.org/10.1016/j.ymgme.
7. Guffon N, Tylki-Szymanska A, Borgwardt L, et al. Recognition of alpha-mannosidosis in paediatric and adult patients: presentation of a diagnostic algorithm from an international working group. Molecular Genetics and Metabolism. 2019;126:470-4. https://doi.org/10.1016/j.ymgme.

Coding Implications
Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
J0217	Injection, velmanase alfa-tycv, 1 mg

Reviews, Revisions, and Approvals	Date	LDH Approval Date
Converted corporate to local policy.	04.04.24	

Important Reminder
This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. LHCC makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable LHCC administrative policies and procedures.

This clinical policy is effective as of the date determined by LHCC. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. LHCC retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to

recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

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