

Medical Policy Manual **Approved Rev: Do Not Implement until 9/30/26**

Cipaglucosidase Alfa-atga (Pombiliti™)

IMPORTANT REMINDER

We develop Medical Policies to provide guidance to Members and Providers. This Medical Policy relates only to the services or supplies described in it. The existence of a Medical Policy is not an authorization, certification, explanation of benefits or a contract for the service (or supply) that is referenced in the Medical Policy. For a determination of the benefits that a Member is entitled to receive under his or her health plan, the Member's health plan must be reviewed. If there is a conflict between the medical policy and a health plan or government program (e.g., TennCare), the express terms of the health plan or government program will govern.

POLICY

INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Pombiliti is indicated, in combination with Opfolda, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing greater than or equal to 40 kg and who are not improving on their current enzyme replacement therapy (ERT).

All other indications are considered experimental/investigational and not medically necessary.

DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- Initial requests:
 - Acid alpha-glucosidase enzyme assay **reflecting deficiency of enzyme activity (less than 40% of normal controls)** or genetic testing results supporting diagnosis.
 - **Chart notes or medical records documenting clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).**
- Continuation requests:
 - Chart notes **or medical records** documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, muscle strength).

PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

COVERAGE CRITERIA

Late-onset Pompe disease

Authorization of 12 months may be granted for treatment of late-onset Pompe disease when all of the following criteria are met:

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- Member is 18 years of age or older.
- Member weighs greater than or equal to 40 kg.
- Diagnosis was confirmed by enzyme assay demonstrating a deficiency of acid alpha-glucosidase enzyme activity (**less than 40% of normal controls**) or by genetic testing.
- **Member exhibits clinical signs and symptoms of disease (e.g. progressive proximal muscle weakness, respiratory insufficiency, cardiomyopathy).**
- Member is not improving on current enzyme replacement therapy (ERT) (e.g., Lumizyme, Nexvazyme).
- The requested medication will be taken in combination with Opfolda (miglustat).

CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section when both of the following criteria are met:

- Member is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for motor function, walking capacity, respiratory function, or muscle strength).
- The requested medication will be taken in combination with Opfolda (miglustat).

APPLICABLE TENNESSEE STATE MANDATE REQUIREMENTS

BlueCross BlueShield of Tennessee's Medical Policy complies with Tennessee Code Annotated Section 56-7-2352 regarding coverage of off-label indications of Food and Drug Administration (FDA) approved drugs when the off-label use is recognized in one of the statutorily recognized standard reference compendia or in the published peer-reviewed medical literature.

ADDITIONAL INFORMATION

For appropriate chemotherapy regimens, dosage information, contraindications, precautions, warnings, and monitoring information, please refer to one of the standard reference compendia (e.g., the NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) published by the National Comprehensive Cancer Network®, Drugdex Evaluations of Micromedex Solutions at Truven Health, or The American Hospital Formulary Service Drug Information).

REFERENCES

1. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; July 2024.
2. **Sperry E, Leslie N, Berry L, et al. Pompe Disease. 2007 Aug 31 [Updated 2025 Aug 21]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.**

EFFECTIVE DATE 9/30/2026

ID_CHS_2026