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Section:	Prescription Drugs	**Effective Date:**	April 1, 2025
Subsection:	Endocrine and Metabolic Agents	**Original Policy Date:**	January 26, 2024
Subject:	Opfolda	**Page:**	1 of 4

Last Review Date: March 7, 2025

Opfolda

Description

Opfolda (miglustat)

Background
Opfolda (miglustat) is indicated for use in combination with Pombiliti for the treatment of late-onset Pompe disease, a rare genetic disorder. In Pompe disease, a gene mutation prevents the body from making an enzyme or making enough of the enzyme called acid alpha-glucosidase (GAA), necessary for proper muscle function. GAA is used by the heart and muscle cells to convert stored glycogen into energy. Without sufficient enzyme action, glycogen builds up in the cells, ultimately weakening the heart and other muscles. Infusion of Pombiliti replaces the deficient GAA, reducing the accumulated glycogen in the body. Opfolda is used in combination with Pombiliti to stabilize and prolong its action (1).

Regulatory Status
FDA-approved indication: Opfolda is an enzyme stabilizer indicated, in combination with Pombiliti, a hydrolytic lysosomal glycogen-specific enzyme, for the treatment of adult patients with late-onset Pompe disease (lysosomal acid alpha-glucosidase [GAA] deficiency) weighing ≥40 kg and who are not improving on their current enzyme replacement therapy (ERT) (1).

Opfolda in combination with Pombiliti may cause embryo-fetal harm. Females of reproductive potential should use effective contraception during treatment and for at least 60 days after the last dose (1).

Opfolda must be used in combination with Pombiliti and prescribers should refer to the Pombiliti prescribing information for a description of risks, warnings and precautions for Pombiliti. Opfolda is not approved for use in Gaucher's diesease (1).

The safety and effectiveness of Opfolda in pediatric patients less than 18 year of age have not been established (1).

Related policies
Lumizyme, Nexviazyme, Pombiliti

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Opfolda may be considered **medically necessary** if the conditions indicated below are met.

Opfolda may be considered **investigational** for all other indications.

Prior-Approval Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

1. Late-onset Pompe disease (acid alpha-glucosidase (GAA) deficiency)

AND ALL of the following:

a. Patient has not improved on enzyme replacement therapy (ERT)
b. Patient weight ≥40 kg
c. Used in combination with Pombiliti
d. Females of reproductive potential **only**: patient will be advised to use effective contraception during treatment and for 60 days after the last dose

Prior–Approval *Renewal* Requirements

Age 18 years of age and older

Diagnosis

Patient must have the following:

1. Late-onset Pompe disease (acid alpha-glucosidase (GAA) deficiency)

AND ALL of the following:

a. Patient weight ≥40 kg
b. Used in combination with Pombiliti
c. Females of reproductive potential **only**: patient will be advised to use effective contraception during treatment and for 60 days after the last dose

Policy Guidelines

Pre–PA Allowance

None

Prior–Approval Limits

Duration 2 years

Prior–Approval *Renewal* Limits

Same as above

Rationale

Summary

Opfolda is an enzyme stabilizer indicated, in combination with Pombiliti, a hydrolytic lysosomal glycogen-specific enzyme, indicated for adults with late-onset Pompe disease (acid α-glucosidase [GAA] deficiency). Opfolda is not indicated for the treatment of Gaucher's disease.

The safety and effectiveness of Opfolda in pediatric patients less than 18 years of age have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Opfolda while maintaining optimal therapeutic outcomes.

References

1. Opfolda [package insert]. Philadelphia, PA: Amicus Therapeutics, Inc.; July 2024.

Policy History

Date	Action
January 2024	Addition to PA
March 2024	Annual review
March 2025	Annual review and reference update

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 7, 2025 and is effective on April 1, 2025.