UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2025 P 1420-3
Program	Prior Authorization/Notification
Medication	Opfolda® (miglustat)
P&T Approval Date	11/2023, 11/2024, 11/2025
Effective Date	2/15/2026

1. Background:

Opfolda (miglustat) 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).

2. Coverage Criteria[a]:

A. Initial Authorization

1. **Opfolda** will be approved based on **both** of the following criteria:

   a. Diagnosis of late-onset Pompe disease

   -AND-

   b. Patient has an active UnitedHealthcare medical benefit prior authorization for Pombiliti (cipaglucosidase alfa-atga) for the treatment of late-onset Pompe disease

Authorization will be issued for 12 months.

B. Reauthorization

1. **Opfolda** will be approved based on **both** of the following criteria:

   a. Documentation of positive clinical response to Opfolda plus Pombiliti

   -AND-

   b. Opfolda continues to be prescribed in combination with Pombiliti

Authorization will be issued for 12 months.

[a] State mandates may apply. Any federal regulatory requirements and the member specific benefit plan coverage may also impact coverage criteria. Other policies and utilization management programs may apply.

3. Additional Clinical Rules:

- Notwithstanding Coverage Criteria, UnitedHealthcare may approve initial and re-authorization based solely on previous claim/medication history, diagnosis codes (ICD-10) and/or claim logic. Use of automated approval and re-approval processes varies by program and/or therapeutic class.

4. References:

1. Opfolda [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; September 2024.
2. Pombiliti [package insert]. Philadelphia, PA: Amicus Therapeutics US, LLC; September 2024.

Program	Prior Authorization/Notification - Opfolda (miglustat)
Change Control	
Date	Change
11/2023	New program
11/2024	Clarified criteria without change to clinical intent. Updated references.
11/2025	Annual review with no change to coverage criteria.