

UnitedHealthcare Pharmacy
Clinical Pharmacy Programs

Program Number	2024 P 1420-2
Program	Prior Authorization/Notification
Medication	Opfolda™ (miglustat)
P&T Approval Date	11/2023, 11/2024
Effective Date	2/1/2025

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:

<p>A. <u>Initial Authorization</u></p> <p>1. Opfolda will be approved based on both of the following criteria:</p> <p style="margin-left: 40px;">a. Diagnosis of late-onset Pompe disease</p> <p style="text-align: center;">-AND-</p> <p style="margin-left: 40px;">b. Patient has an active UnitedHealthcare medical benefit prior authorization for Pombiliti (cipaglucosidase alfa-atga) for the treatment of late-onset Pompe disease</p> <p>Authorization will be issued for 12 months.</p> <p>B. <u>Reauthorization</u></p> <p>1. Opfolda will be approved based on both of the following criteria:</p> <p style="margin-left: 40px;">a. Documentation of positive clinical response to Opfolda plus Pombiliti</p> <p style="text-align: center;">-AND-</p> <p style="margin-left: 40px;">b. Opfolda continues to be prescribed in combination with Pombiliti</p> <p>Authorization will be issued for 12 months.</p> <p>^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.</p>
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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.