HAP CareSource™

PHARMACY POLICY STATEMENT
HAP CareSource™ Marketplace

DRUG NAME	**Pombiliti (cipaglucosidase alfa-atga) and Opfolda (miglustat)**
BENEFIT TYPE	Medical
STATUS	Prior Authorization Required

Pombiliti and Opfolda, approved by the FDA in 2023, are indicated to be used in combination 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). Pombiliti provides an exogenous source of the deficient GAA enzyme, and Opfolda is an enzyme stabilizer.
Pompe disease is a rare, genetic lysosomal storage disorder that results in the buildup of glycogen in cell lysosomes causing serious and life-threatening muscle damage and weakness. It can be broadly classified as infantile onset within the first few months of life (IOPD) or late onset beyond infancy (LOPD). Classic IOPD is rapidly progressive with severe cardiomyopathy. Non-classic IOPD progresses slower with less severe cardiomyopathy. LOPD does not typically present with cardiomyopathy and has more variable symptoms, especially skeletal muscle weakness. Pombiliti and Opfolda are only indicated to treat LOPD.
Of note, miglustat is also the active ingredient in Zavesca, which is approved for Gaucher disease.

Pombiliti (cipaglucosidase alfa-atga) and Opfolda (miglustat) will be considered for coverage when the following criteria are met:

Pompe Disease

For **initial** authorization:
1. Member is at least 18 years of age; AND
2. Medication must be prescribed by or in consultation with a geneticist, neurologist, pulmonologist, or metabolic specialist; AND
3. Pombiliti and Opfolda are being prescribed in combination; AND
4. Member has a diagnosis of late onset Pompe disease confirmed by an enzyme activity assay showing GAA deficiency (2% to 40% of normal); AND
5. Molecular genetic testing shows pathogenic mutation of the GAA gene; AND
6. Member is not improving on their current enzyme replacement therapy (i.e., Lumizyme or Nexviazyme); AND
7. Member must show signs or symptoms (i.e., motor weakness, reduced respiratory parameters); AND
8. Documentation of baseline percent-predicted forced vital capacity (FVC) and 6-minute walk test (6MWT); AND
9. If female, attestation that the member is not pregnant.
10. **Dosage allowed/Quantity limit:** Starting 2 weeks after the last ERT dose:
    Pombiliti: 20 mg/kg every other week IV infusion
    Opfolda: Orally every other week; 260 mg for patients weighing ≥50 kg or 195 mg for patients weighing ≥40 kg to <50 kg

If all the above requirements are met, the medication will be approved for 12 months.

For **reauthorization**:

1. Chart notes must document positive clinical response such as improved or stabilized respiratory muscle strength (i.e., forced vital capacity (FVC)) or functional endurance (e.g., 6-minute walk test).

If all the above requirements are met, the medication will be approved for an additional 12 months.

HAP CareSource considers Pombiliti (cipaglucosidase alfa-atga) and Opfolda (miglustat) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
10/25/2023	New policy for Pombiliti and Opfolda created.

References:

1. Pombiliti [prescribing information]. Amicus Therapeutics US, LLC; 2023.
2. Opfolda [prescribing information]. Amicus Therapeutics US, LLC; 2023.
3. Schoser B, Roberts M, Byrne BJ, et al. Safety and efficacy of cipaglucosidase alfa plus miglustat versus alglucosidase alfa plus placebo in late-onset Pompe disease (PROPEL): an international, randomised, double-blind, parallel-group, phase 3 trial [published correction appears in Lancet Neurol. 2023 Oct;22(10):e11]. *Lancet Neurol.* 2021;20(12):1027-1037. doi:10.1016/S1474-4422(21)00331-8
4. Cupler EJ, Berger KI, Leshner RT, et al. Consensus treatment recommendations for late-onset Pompe disease. *Muscle Nerve*. 2012;45(3):319-333. doi:10.1002/mus.22329
5. Van der Ploeg AT, Kruijshaar ME, Toscano A, et al. European consensus for starting and stopping enzyme replacement therapy in adult patients with Pompe disease: a 10-year experience. *Eur J Neurol*. 2017;24(6):768-e31. doi:10.1111/ene.13285
6. Wang RY, Bodamer OA, Watson MS, Wilcox WR; ACMG Work Group on Diagnostic Confirmation of Lysosomal Storage Diseases. Lysosomal storage diseases: diagnostic confirmation and management of presymptomatic individuals. *Genet Med*. 2011;13(5):457-484. doi:10.1097/GIM.0b013e318211a7e1
7. Tarnopolsky M, Katzberg H, Petrof BJ, et al. Pompe Disease: Diagnosis and Management. Evidence-Based Guidelines from a Canadian Expert Panel. *Can J Neurol Sci*. 2016;43(4):472-485. doi:10.1017/cjn.2016.37

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