PHARMACY POLICY STATEMENT
Indiana Medicaid

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

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.

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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