PHARMACY POLICY STATEMENT
Ohio Medicaid

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) heir 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 792 r78(a)56(n)-14(t)- y83 Tm0 G[(atg 2o5 1 0 0 1 475.83 494575.95 594Dbe

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.
01/25/2024	