Pombiliti™ (cipaglucosidase alfa-atga) + Opfolda™ (miglustat) 65 mg capsules

GETTING STARTED ON POMBILITI + OPFOLDA: HELP FOR YOUR PATIENTS

INDICATION

POMBILITI in combination with OPFOLDA is indicated 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).

SELECT IMPORTANT SAFETY INFORMATION

WARNING: SEVERE HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, and RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

See full prescribing information for complete boxed warning

Hypersensitivity Reactions Including Anaphylaxis

Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available. If a severe hypersensitivity reaction occurs, POMBILITI should be discontinued immediately and appropriate medical treatment should be initiated.

Infusion-Associated Reactions (IARs)

If severe IARs occur, immediately discontinue POMBILITI and initiate appropriate medical treatment.

Risk of Acute Cardiorespiratory Failure in Susceptible Patients

Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function, may be at risk of serious exacerbation of their cardiac or respiratory status during POMBILITI infusion.

Please see additional Important Safety Information on the last page and full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.

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AMICUS ASSIST® CAN HELP SUPPORT YOUR PATIENTS ON POMBILITI + OPFOLDA

AMICUS ASSIST is a program that provides dedicated and personalized support to individuals who have been prescribed an Amicus medication.

As part of the program, your patient will be assigned a dedicated Patient Education Liaison (PEL) who can offer education and support to patients by:

- Educating about late-onset Pompe disease (LOPD) and POMBILITI + OPFOLDA
- Providing tips to help patients prepare for their treatment days
- Helping patients have more productive conversations with their care team

PELs do not give medical advice or take the place of the patient's healthcare provider.

Your patient will also be assigned a dedicated Case Manager who can help patients navigate treatment access and financial assistance by:

- Helping navigate their insurance coverage
- Helping to coordinate prescription delivery
- Identifying possible sources of financial assistance

Amicus assist®

AMICUS ASSIST is ready to help you and your patients.
Call us toll free at:
1-833-AMICUS-A (1-833-264-2872)
Monday-Friday, 8 AM-8 PM ET, or email to assist@amicusrx.com

Hablamos español. Administradores de casos de habla hispana éstan disponibles.
Llamanos a +1-833-AMICUS-A (+1-833-264-2872)
Lunes-Viernes de 8 AM-8 PM ET

SELECT IMPORTANT SAFETY INFORMATION

HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS

Patients treated with POMBILITI have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during POMBILITI administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, POMBILITI should be discontinued immediately, and appropriate medical treatment should be initiated. In patients with severe hypersensitivity reaction, desensitization measures to POMBILITI may be considered. The risks and benefits of readministering POMBILITI following severe hypersensitivity reaction should be considered. If mild or moderate hypersensitivity reaction occurs, the infusion rate may be slowed or temporarily stopped. Prior to POMBILITI administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids.

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SIMPLE STEPS FROM PRESCRIPTION TO DELIVERY

Once you decide to prescribe POMBILITI™ (cipaglucosidase alfa-atga) + OPFOLDA™ (miglustat), here's what happens next:

Complete the Patient Referral Form (PRF) and send it to AMICUS ASSIST

- Fax number: 1-833-264-2873
- Email: assist@amicusrx.com

To download a PRF, please visit AmicusAssistHCP.com

A Case Manager will reach out to you after you prescribe POMBILITI + OPFOLDA and will also contact your patient

AMICUS ASSIST will:

- Confirm if a Prior Authorization is required
- Provide an explanation of the patient's eligibility and potential financial assistance options
- Provide additional reimbursement support

Depending on the treatment setting, AMICUS ASSIST will do one of the following:

- **Triage the Patient Referral Form (PRF)** to the Specialty Pharmacy (SP). The SP will work directly with the prescriber's office to confirm the prescription and fill requests for ancillary supplies
- Contact the prescriber to review the ordering process for **Direct Purchase** orders

AMICUS ASSIST will help answer questions about the POMBILITI and OPFOLDA delivery based on the treatment setting

SELECT IMPORTANT SAFETY INFORMATION

INFUSION-ASSOCIATED REACTIONS (IARS)

Patients treated with POMBILITI have experienced severe IARs. If severe IARs occur, immediately discontinue the POMBILITI infusion, initiate appropriate medical treatment, and assess the benefits and risks of readministering POMBILITI following severe IARs. If mild or moderate IARs occur regardless of pretreatment, decreasing the infusion rate or temporarily stopping the infusion may ameliorate the symptoms. IARs may still occur in patients after receiving pretreatment.

Patients with an acute underlying illness at the time of POMBILITI infusion may be at greater risk for IARs. Patients with advanced Pompe disease may have compromised cardiac and respiratory function, which may predispose them to a higher risk of severe complications from IARs.

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IMPORTANT SAFETY INFORMATION

WARNING: SEVERE HYPERSENSITIVITY REACTIONS, INFUSION-ASSOCIATED REACTIONS, and RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

HYPERSENSITIVITY REACTIONS INCLUDING ANAPHYLAXIS

Patients treated with POMBILITI have experienced life-threatening hypersensitivity reactions, including anaphylaxis. Appropriate medical support measures, including cardiopulmonary resuscitation equipment, should be readily available during POMBILITI administration. If a severe hypersensitivity reaction (e.g., anaphylaxis) occurs, POMBILITI should be discontinued immediately, and appropriate medical treatment should be initiated. In patients with severe hypersensitivity reaction, desensitization measures to POMBILITI may be considered. The risks and benefits of readministering POMBILITI following severe hypersensitivity reaction should be considered. If mild or moderate hypersensitivity reaction occurs, the infusion rate may be slowed or temporarily stopped. Prior to POMBILITI administration, consider pretreating with antihistamines, antipyretics, and/or corticosteroids.

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Patients with an acute underlying illness at the time of POMBILITI infusion may be at greater risk for IARs. Patients with advanced Pompe disease may have compromised cardiac and respiratory function, which may predispose them to a higher risk of severe complications from IARs.

RISK OF ACUTE CARDIORESPIRATORY FAILURE IN SUSCEPTIBLE PATIENTS

Patients susceptible to fluid volume overload, or those with acute underlying respiratory illness or compromised cardiac or respiratory function for whom fluid restriction is indicated may be at risk of serious exacerbation of their cardiac or respiratory status during POMBILITI infusion. More frequent monitoring of vitals should be performed during POMBILITI infusion in such patients.

CONTRAINDICATION

POMBILITI in combination with OPFOLDA is contraindicated in pregnancy.

EMBRYO-FETAL TOXICITY

Based on findings from animal reproduction studies, POMBILITI in combination with OPFOLDA may cause embryo-fetal harm when administered to a pregnant female and is contraindicated during pregnancy. Verify the pregnancy status in females of reproductive potential prior to initiating treatment with POMBILITI in combination with OPFOLDA. Advise females of reproductive potential to use effective contraception during treatment with POMBILITI in combination with OPFOLDA and for at least 60 days after the last dose.

RISKS ASSOCIATED WITH POMBILITI AND OPFOLDA

POMBILITI and OPFOLDA must be administered in combination.

ADVERSE REACTIONS

The most common adverse reactions (≥5%) reported in the pooled safety population of patients treated with POMBILITI in combination with OPFOLDA in the 3 clinical trials were headache, diarrhea, fatigue, nausea, abdominal pain, and pyrexia.

To report SUSPECTED ADVERSE REACTIONS, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

LACTATION

Advise females that breastfeeding is not recommended while on treatment with POMBILITI in combination with OPFOLDA.

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