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

YOUR GUIDE TO TREATMENT DAY

Helping you prepare for treatment with POMBILITI + OPFOLDA

What are POMBILITI and OPFOLDA?

POMBILITI and OPFOLDA are prescription medicines used in combination for the treatment of adults with late-onset Pompe disease weighing 88 pounds (40 kg) or more and who are not improving on their current enzyme replacement therapy (ERT).

It is not known if POMBILITI in combination with OPFOLDA is safe and effective in children with late-onset Pompe disease.

IMPORTANT SAFETY INFORMATION

Warning: Hypersensitivity Reactions (including Anaphylaxis), Infusion-Associated Reactions (IARs), and Risk of Acute Cardiorespiratory Failure

POMBILITI in combination with OPFOLDA may cause serious side effects, including:

- **Hypersensitivity reactions (including anaphylaxis):** Severe and potentially life-threatening allergic-type reactions related to the infusion have been reported during and after POMBILITI in combination with OPFOLDA treatment. Your doctor will inform you of the signs and symptoms of hypersensitivity reactions which may include: difficulty breathing or swallowing; rash or hives; low blood pressure; swelling of lips, tongue, throat, or face. Seek medical care immediately should signs and symptoms occur. If a severe reaction occurs, your doctor may decide to immediately discontinue the infusion and provide medical care. Appropriate medical support measures may be administered, and you may require close observation during and after POMBILITI infusion.

Please see Important Safety Information throughout and on page 7 and see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information and Patient Information for OPFOLDA, also available at PombilitiOpfolda.com.

Setting expectations for treatment

Though you're already familiar with enzyme replacement therapy (ERT), it's important to understand what to expect anytime you start a new treatment. POMBILITI™ (cipaglucosidase alfa-atga) + OPFOLDA™ (miglustat) is a two-component therapy for adults with late-onset Pompe disease (LOPD) weighing 88 lbs or more who are not improving on their current ERT.

This brochure includes:

- **Instructions on when and how to take OPFOLDA**
- **Important details about fasting when taking OPFOLDA**
- **Information about the infusion process with POMBILITI**
- **Additional tips and tools to help you prepare for treatment**
- **An overview of the AMICUS ASSIST® patient support program and how it can help**

SELECT IMPORTANT SAFETY INFORMATION

POMBILITI in combination with OPFOLDA may cause serious side effects, including:

- **Infusion-Associated Reactions (IARs):** Severe IARs related to the infusion have been reported during or after POMBILITI in combination with OPFOLDA. Your doctor will inform you of the signs and symptoms of hypersensitivity reactions which may include: hives, itching, shortness of breath, flushing, chills, and low blood pressure. Seek medical care immediately should signs and symptoms occur. If severe IARs occur during infusion, your doctor may decide to immediately discontinue the infusion and provide appropriate medical care. If you have an acute underlying illness at the time of POMBILITI infusion you may be at greater risk for IARs. If you have advanced Pompe disease you may have compromised heart and breathing function, which may put you at a higher risk of severe complications from IARs.

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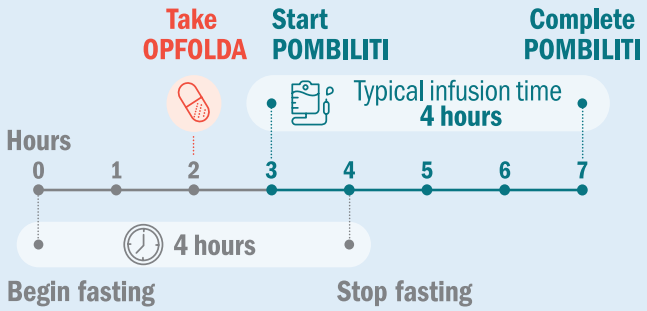
Your treatment day at a glance

POMBILITI™ (cipaglucosidase alfa-atga) is an enzyme replacement therapy (ERT). OPFOLDA™ (miglustat) is an oral enzyme stabilizer designed to stabilize POMBILITI in the bloodstream. Each component was designed to work exclusively with the other. POMBILITI + OPFOLDA must be taken in combination at the right time under the right circumstances. Neither should be taken with another late-onset Pompe disease (LOPD) treatment. That's why it's important to work with your care team to set up your treatment routine.

TREATMENT DAY OCCURS EVERY 2 WEEKS, AND A TYPICAL ONE SHOULD LOOK LIKE THIS:



*Talk to your doctor if you have kidney problems because your OPFOLDA dose may be different.

POMBILITI + OPFOLDA DOSING IS DETERMINED BY YOUR WEIGHT

You will take 3 or 4 OPFOLDA capsules depending on the dosage that your healthcare provider determines is right for you.*

SWITCH WITHOUT DELAY

When switching to POMBILITI + OPFOLDA, you can start POMBILITI + OPFOLDA on your next scheduled treatment day (2 weeks after your last ERT dose).

Your doctor may recommend taking medicine such as an antihistamine, antipyretic (fever reducer), or corticosteroid before your POMBILITI infusion, especially if you took a medication with your previous ERT. Always take your medication exactly as directed by your healthcare provider.

WHY DO I HAVE TO FAST WHILE TAKING OPFOLDA?

OPFOLDA requires fasting in order to bind to POMBILITI in your bloodstream. You can resume drinking and eating 2 hours after taking OPFOLDA.

SELECT IMPORTANT SAFETY INFORMATION

POMBILITI in combination with OPFOLDA may cause serious side effects, including:

- **Risk of Acute Cardiorespiratory Failure:** If you are likely to develop fluid volume overload or have an acute breathing condition or heart and/or breathing problems that require fluid restriction, you may be at risk of worsening of your heart or breathing status during POMBILITI infusion. Your doctor may decide that close observation during and after POMBILITI administration may be necessary.

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Why your routine matters

Now that you know why POMBILITI and OPFOLDA are taken together, it's important to understand when and how OPFOLDA is taken. The following instructions should be followed every 2 weeks on each treatment day:

- **Be sure to take OPFOLDA exactly as your healthcare provider directs**
- **Do not eat anything for at least 2 hours prior to taking OPFOLDA**
- **Take your prescribed OPFOLDA approximately 1 hour prior to your infusion**
- **Once you've taken OPFOLDA, be sure not to eat anything for at least 2 hours**
  - It's okay to drink certain fluids throughout your treatment day. You can have unsweetened beverages like water, tea, or coffee with no cream, sugar, or other sweeteners. Do not drink other beverages or eat food during the fasting window.
- **2 hours after taking OPFOLDA, you are free to eat and drink whatever you'd like**

WHAT SHOULD I DO AFTER MY INFUSION?

Once your infusion is complete, remember to schedule your next infusion appointment. POMBILITI + OPFOLDA should be administered once every 2 weeks.

If you have any questions, concerns, or problems with your treatment, be sure to reach out to your healthcare team.

SELECT IMPORTANT SAFETY INFORMATION

Do not take POMBILITI in combination with OPFOLDA if you are pregnant.

Before taking POMBILITI in combination with OPFOLDA, tell your healthcare provider about all of your medical conditions, including if you:

- have kidney problems
- are pregnant or plan to become pregnant. POMBILITI in combination with OPFOLDA may cause harm to your unborn baby.

  Females who are able to become pregnant:
  - Your healthcare provider will check if you are pregnant before you start treatment with POMBILITI in combination with OPFOLDA.
  - You should use effective birth control (contraception) during treatment with POMBILITI in combination with OPFOLDA and for at least 60 days after the last dose.
  - Tell your healthcare provider right away if you become pregnant or think you might be pregnant during treatment with POMBILITI in combination with OPFOLDA.
- are breastfeeding or plan to breastfeed. It is not known if OPFOLDA alone or in combination with POMBILITI passes into your breast milk. **Do not** breastfeed during treatment with POMBILITI in combination with OPFOLDA. Talk to your healthcare provider about the best way to feed your baby during this time.

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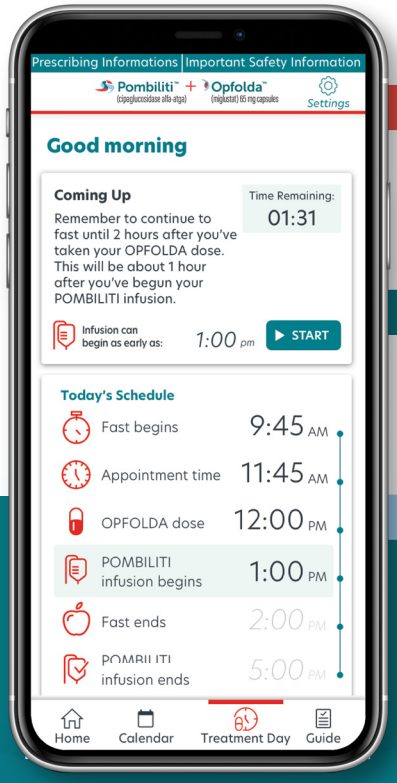
Tools to plan ahead

The POMBILITI™ (cipaglucosidase alfa-atga) + OPFOLDA™ (miglustat) MyDay Pompe™ app allows you to stay on top of your treatment by providing the information you need right on your mobile device.

WHAT FEATURES DOES THE APP INCLUDE?

- **Notifications that help you stay on top of each treatment day step**
- **Calendars that can be used to manage upcoming appointments**
- **Resources and information on what to expect on treatment day**



Download the POMBILITI + OPFOLDA MyDay Pompe app today by scanning the QR code or visiting the App Store or Google Play.

SELECT IMPORTANT SAFETY INFORMATION

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

POMBILITI and OPFOLDA must be taken in combination. POMBILITI in combination with OPFOLDA will be given to you 1 time every other week.

The most common side effects of POMBILITI in combination with OPFOLDA include: headache, diarrhea, tiredness, nausea, stomach area pain, and fever.

POMBILITI in combination with OPFOLDA may cause fertility problems in females and males, which may affect the ability to have children. Talk to your healthcare provider if you have concerns about fertility.

These are not all the possible side effects of POMBILITI and OPFOLDA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

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AMICUS ASSIST®: A friendly face at every step

It's okay to have questions. And you can count on AMICUS ASSIST to help you find answers. AMICUS ASSIST is a patient support program that is staffed by Patient Education Liaisons (PELs) and Case Managers to assist you throughout your journey.

Amicus assist®

WHAT IS A PATIENT EDUCATION LIAISON (PEL)?

Your dedicated PEL is available to offer education and support throughout treatment by:

- Answering questions about LOPD and your treatment
- Providing tips to help you prepare for your treatment days
- Helping you have more productive conversations with your care team

Please remember, while your PEL is available to provide support throughout your treatment, PELs do not provide medical advice. Your healthcare provider is your go-to resource for any questions related to your care.

WHAT IS A CASE MANAGER?

Your Case Manager will help you navigate treatment access and financial resources by:

- Verifying and explaining your insurance coverage
- Helping coordinate prescription deliveries
- Identifying possible sources of financial assistance

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Questions or concerns on treatment day?

If you need any assistance while receiving treatment, be sure to speak up and notify your healthcare team. They will know how to best assist you.

You can also reach out to your AMICUS ASSIST® Patient Education Liaison or Case Manager with other questions about your treatment day.

For more information about the infusion process, feel free to contact AMICUS ASSIST, Monday through Friday from 8AM–8PM ET, at **1-833-AMICUS-A (1-833-264-2872)**.

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