

PATIENT INFORMATION
OPFOLDA™ (op fol dah)
(miglustat)
capsules, for oral use

What is OPFOLDA?

OPFOLDA is a prescription medicine used in combination with Pombiliti 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 OPFOLDA in combination with Pombiliti is safe and effective in children with late-onset Pompe disease.

Do not take OPFOLDA in combination with Pombiliti if you are pregnant. See “**Before taking OPFOLDA, tell your healthcare provider about all of your medical conditions, including if you.**”

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

- have kidney problems
- are pregnant or plan to become pregnant. OPFOLDA in combination with Pombiliti 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 OPFOLDA in combination with Pombiliti.
- You should use effective birth control (contraception) during treatment with OPFOLDA in combination with Pombiliti 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 OPFOLDA in combination with Pombiliti.
- 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 OPFOLDA in combination with Pombiliti. Talk to your healthcare provider about the best way to feed your baby during this time.

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

How should I take OPFOLDA?

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

- Take OPFOLDA exactly as your healthcare provider tells you to.
- Take your prescribed dose of OPFOLDA every other week about 1 hour before the start of your Pombiliti infusion.
- Swallow OPFOLDA capsules whole only with unsweetened beverages, including water, tea or coffee with no cream, sugar, or sweeteners. **Do not** drink other beverages or eat food for at least 2 hours before and 2 hours after taking OPFOLDA.
- Your healthcare provider will stop your current ERT before starting your treatment with OPFOLDA in combination with Pombiliti.
- If you miss a dose of OPFOLDA, you should not receive your Pombiliti infusion. Call your healthcare provider right away to reschedule your Pombiliti infusion.

What are the possible side effects of OPFOLDA in combination with Pombiliti?

The most common side effects of OPFOLDA in combination with Pombiliti include:

- | | |
|-------------|---------------------|
| • headache | • nausea |
| • diarrhea | • stomach area pain |
| • tiredness | • fever |

OPFOLDA in combination with Pombiliti 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 of the possible side effects of OPFOLDA in combination with Pombiliti.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store OPFOLDA?

- Store OPFOLDA at room temperature between 68°F to 77°F (20°C to 25°C).
- Keep OPFOLDA capsules in the original container to protect from light.

Keep OPFOLDA and all medicines out of the reach of children.

General information about the safe and effective use of OPFOLDA.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use OPFOLDA for a condition for which it was not prescribed. Do not give OPFOLDA to other people, even if they have the same symptoms you have. It may harm them. You can ask your pharmacist or healthcare provider for information about OPFOLDA that is written for health professionals.

What are the ingredients in OPFOLDA?

Active ingredient: miglustat

Inactive ingredients: colloidal silicon dioxide, magnesium stearate, microcrystalline cellulose, pregelatinized maize starch, and sucralose. The capsule shell contains: black iron oxide, gelatin, and titanium dioxide. The edible printing ink contains: black iron oxide and shellac.

Manufactured for:

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For more information, call AMICUS at 1-877-426-4287 or go to pombilitiopfolda.com.

This Patient Information has been approved by the U.S. Food and Drug Administration.

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