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

# PATIENT REFERRAL FORM INSTRUCTIONS

This guide is intended to help ensure correct completion and submission of the referral form for POMBILITI® (cipaglucosidase alfa-atga) + OPFOLDA® (miglustat) prescription.

## 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).

## 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.

**Please see additional Important Safety Information throughout and on page 6 and full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**

# PATIENT REFERRAL FORM

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

Fax completed form to 1-833-626-4291
For assistance, call 1-833-AMICUS-A (1-833-264-2872)
Monday-Friday, 8AM–8PM ET, or email assist@amicusrx.com

**Product Acquisition** 1 ○ Specialty Pharmacy (SP) ○ Direct Purchase ○ No Access Support Needed

**Step 1. Patient Information** The correct patient information is necessary for timely processing. Click and type to complete all fields and provide accurate information to register the patient with AMICUS ASSIST®.

First name: MI: Last name:
Address: City: State: ZIP:
DOB: Gender: ○ M ○ F Preferred pronouns:
Preferred phone #: ○ Home ○ Work ○ Mobile Can a message be left at the preferred number? ○ Yes ○ No
Email address:
Alternate authorized contact: Relationship to patient: Phone #:

**Step 2. Prescriber Information** Complete all information in this section, as it is essential for the POMBILITI and OPFOLDA prescriptions to be filled.

Prescriber's first name: Last name: NPI:
Office/Clinic/Institution:
Address: City: State: ZIP:
Phone #: Fax #:
Office contact name: Phone # (direct): Email:

**Step 3. Prescription Information** This section serves as a prescription for POMBILITI and OPFOLDA. Include the number of refills. Any changes to this section will require the submission of a new form. Note: This may not serve as a prescription in all states.

ICD-10 Code (Required for prior authorizations): E74.02 (Pompe disease) Patient's current weight: _____ ○ lb ○ kg 2 ○ Late-onset Pompe disease (LOPD)
Has patient been treated previously with enzyme replacement therapy (ERT)? ○ Yes ○ No Current/Prior Pompe medication(s): _____
Known Allergies: ○ Yes ○ No If yes, please list: _____

**Please make sure to fill out all fields in the table below.**

| Medication | 3 Dose/Directions Refer to the full Prescribing Informations for dosing | 4 Check here for Specialty Pharmacy (SP) dispense | 10 Quantity/Refills | 5 Ancillary Supplies (Home Infusion ONLY) |
|---|---|---|---|---|
| Cipaglucosidase alfa-atga 105 mg per vial | ○ 20 mg/kg IV q 2 weeks OR ○ Other: _____ | ○ | Dispense _____ vials (per infusion every 2 weeks) of cipaglucosidase alfa-atga AND ○ Refill for 1 year OR number of refills _____ | Dispense infusion supplies necessary to administer medication per provider protocol. ○ Yes ○ No |
| Miglustat 65 mg capsule | Take by mouth every 2 weeks as instructed before infusion: ○ 4 capsules (260 mg) OR ○ 3 capsules (195 mg) OR ○ 2 capsules (130 mg) Refer to Sections 2.2 and 2.3 in the OPFOLDA full Prescribing Information. | ○ | ○ Dispense miglustat capsules every 2 weeks as prescribed<br>○ Refill for 1 year OR number of refills _____ | Anaphylaxis Order–Specialty pharmacy to provide anaphylaxis kit per provider protocol. ○ Yes ○ No (A prescription is required for Rx products) |

6 ☐ Skilled nursing visit is needed to establish venous access, administer medications, and assess general status and response to therapy.

I certify that the above therapy is medically necessary for the above-named patient and that the information provided is accurate to the best of my knowledge. I understand the medication is indicated for the treatment of adults with late-onset Pompe disease who are not improving on their current treatment and who weigh ≥40 kg. I appoint AMICUS ASSIST, on my behalf, to provide this form or any information contained on this form to the insurer of the above-named patient or to the dispensing pharmacy. I hereby certify that my office has obtained HIPAA-compliant authorization from the above-named patient to disclose the protected health information necessary for Amicus to provide services described in the Patient Authorization on page 4 of the Patient Referral Form. I also allow AMICUS ASSIST to contact the patient/caregiver as needed to process this referral form.

The prescriber's signature is required to initiate registration in AMICUS ASSIST and to fill the prescription for POMBILITI and OPFOLDA. (STAMPS NOT ACCEPTED)

7 ○ DISPENSE AS WRITTEN/DO NOT SUBSTITUTE: The prescriber is to comply with his/her state-specific prescription requirements such as e-prescribing, state-specific prescription form, fax language, etc. Noncompliance with state-specific requirements could result in outreach to the prescriber.

8 **Prescriber Signature:** _____ **Date:** _____

For states requiring handwritten expressions of product selection, use this area (eg, medically necessary, substitutions allowed, dispense as written, do not substitute).

9 **Prescriber Signature:** _____ **Date:** _____

**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).

**IMPORTANT SAFETY INFORMATION**

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

**Please see IMPORTANT SAFETY INFORMATION throughout and on page 3 and see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**

Page 1 of 4

## PAGE 1

1. **Select "Specialty Pharmacy (SP)"** for home infusion or to provide the therapy directly to your office. To order product to treat the patient in an office, clinic, or hospital, **select "Direct Purchase," also known as "buy-and-bill."** If you do not want AMICUS ASSIST® support services, **select "No Access Support Needed."**
2. **Check this box to confirm that the patient has been diagnosed with late-onset Pompe disease (LOPD).**
3. POMBILITI® (cipaglucosidase alfa-atga) + OPFOLDA® (miglustat) is a two-component therapy that must be administered together.[1,2] **Please select the appropriate dose for each product (see section 10).**
4. Use this section to indicate whether a specialty pharmacy will be dispensing either cipaglucosidase alfa-atga, miglustat, or both.
5. The specialty pharmacy will contact the prescriber if any ancillary supplies are checked "Yes."
6. **This box must be selected for home infusion.** If selected, the specialty pharmacy will contact the prescriber for specific nurse orders.
7. The prescriber should be aware of their state-specific prescription requirements. Noncompliance with state-specific requirements could result in outreach to the prescriber by the specialty pharmacy and delayed dispensing.
8. **The prescriber's signature and a date are required. Please review the attestation paragraph above before signing.** Please note, no stamped signatures are permitted.
9. If the prescriber resides in a state that requires handwritten expression of product selection, they will need to sign and date again here.

10 Dispense _____ vials (per infusion every 2 weeks) of cipaglucosidase alfa-atga AND
○ Refill for 1 year
OR number of refills _____

Calculate the number of **cipaglucosidase alfa-atga** vials based on the patient's actual body weight in kg.[1]

**For example, a 75-kg patient dosed at 20 mg/kg would be calculated like this[1]:**

**STEP 1:** 75 kg x 20 mg/kg = 1500 mg total dose[1]

**STEP 2:** 1500 mg ÷ 105 mg/vial = 14.29 (round up to 15 vials)[1]

For more information, see section 2.2 of the POMBILITI **Prescribing Information**.

Take by mouth every 2 weeks as instructed before infusion:
○ 4 capsules (260 mg) OR
○ 3 capsules (195 mg) OR
○ 2 capsules (130 mg)
Refer to Sections 2.2 and 2.3 in the OPFOLDA full Prescribing Information.

**Miglustat** dosing is based on actual body weight and kidney function.[2]

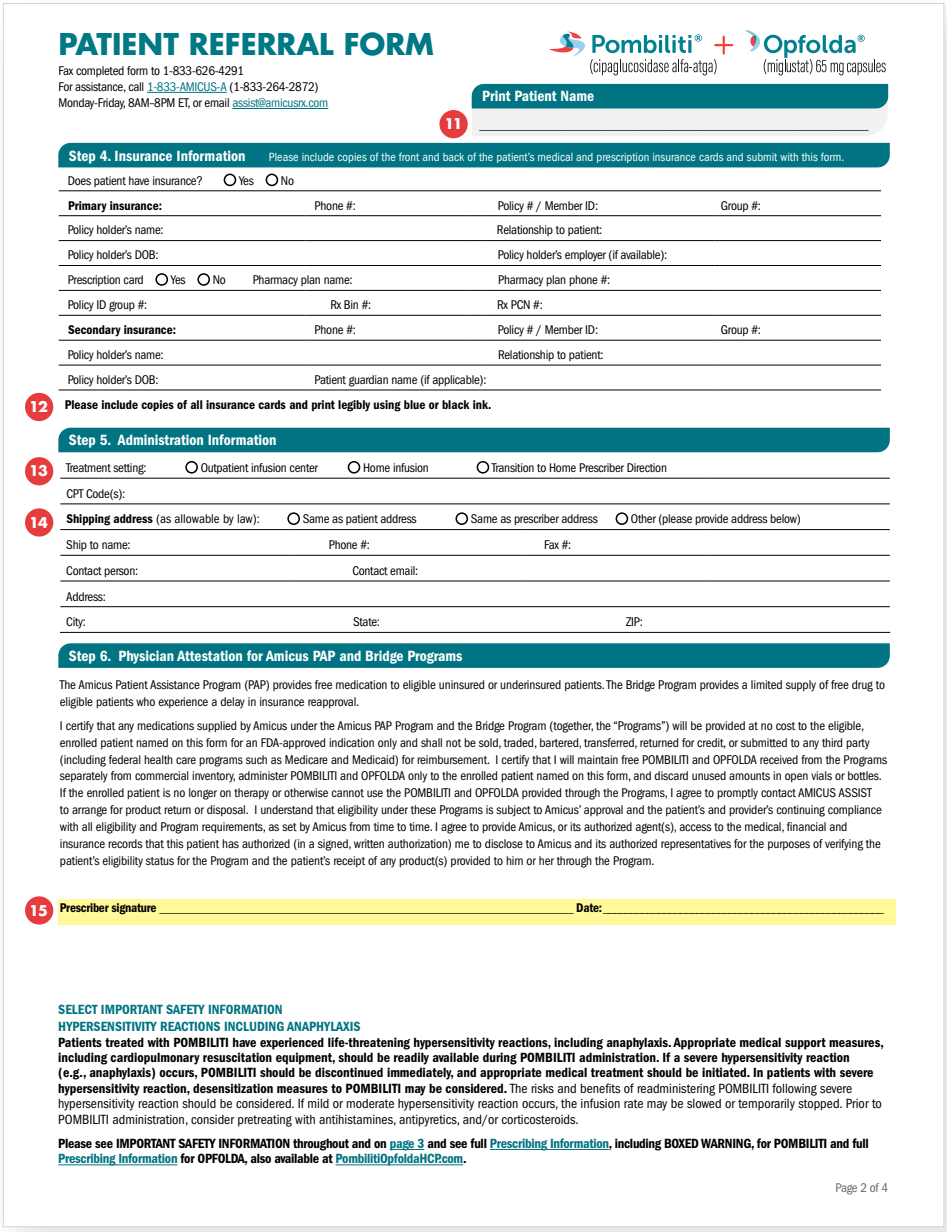
**Recommended dosage for patients weighing:**
- ≥50 kg = 260 mg (4 capsules of 65 mg)
- ≥40 kg to <50 kg = 195 mg (3 capsules of 65 mg)

**Recommended dosage in patients with moderate or severe renal impairment*:**
- ≥50 kg = 195 mg (3 capsules of 65 mg)
- ≥40 kg to <50 kg = 130 mg (2 capsules of 65 mg)

**For patients with mild renal impairment, the recommended dosage is the same as for patients with normal renal function.**

For more information, see sections 2.2 and 2.3 of the OPFOLDA **Prescribing Information**.

*Renal function is classified by creatinine clearance (CLcr) based on the Cockcroft-Gault equation. Mild renal impairment is CLcr 60-89 mL/min, moderate renal impairment is CLcr 30-59 mL/min, and severe renal impairment is CLcr 15-29 mL/min.



# PATIENT REFERRAL FORM

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

Fax completed form to 1-833-626-4291
For assistance, call 1-833-AMICUS-A (1-833-264-2872)
Monday-Friday, 8AM–8PM ET, or email assist@amicusrx.com

11 **Print Patient Name** _____

**Step 4. Insurance Information** Please include copies of the front and back of the patient's medical and prescription insurance cards and submit with this form.

Does patient have insurance? ○ Yes ○ No

| | | | |
|---|---|---|---|
| **Primary insurance:** | Phone #: | Policy # / Member ID: | Group #: |
| Policy holder's name: | | Relationship to patient: | |
| Policy holder's DOB: | | Policy holder's employer (if available): | |
| Prescription card ○ Yes ○ No | Pharmacy plan name: | Pharmacy plan phone #: | |
| Policy ID group #: | Rx Bin #: | Rx PCN #: | |
| **Secondary insurance:** | Phone #: | Policy # / Member ID: | Group #: |
| Policy holder's name: | | Relationship to patient: | |
| Policy holder's DOB: | Patient guardian name (if applicable): | | |

12 **Please include copies of all insurance cards and print legibly using blue or black ink.**

**Step 5. Administration Information**

13 Treatment setting: ○ Outpatient infusion center ○ Home infusion ○ Transition to Home Prescriber Direction

CPT Code(s):

14 **Shipping address** (as allowable by law): ○ Same as patient address ○ Same as prescriber address ○ Other (please provide address below)

| | | |
|---|---|---|
| Ship to name: | Phone #: | Fax #: |
| Contact person: | Contact email: | |
| Address: | | |
| City: | State: | ZIP: |

**Step 6. Physician Attestation for Amicus PAP and Bridge Programs**

The Amicus Patient Assistance Program (PAP) provides free medication to eligible uninsured or underinsured patients. The Bridge Program provides a limited supply of free drug to eligible patients who experience a delay in insurance reapproval.

I certify that any medications supplied by Amicus under the Amicus PAP Program and the Bridge Program (together, the "Programs") will be provided at no cost to the eligible, enrolled patient named on this form for an FDA-approved indication only and shall not be sold, traded, bartered, transferred, returned for credit, or submitted to any third party (including federal health care programs such as Medicare and Medicaid) for reimbursement. I certify that I will maintain free POMBILITI and OPFOLDA received from the Programs separately from commercial inventory, administer POMBILITI and OPFOLDA only to the enrolled patient named on this form, and discard unused amounts in open vials or bottles. If the enrolled patient is no longer on therapy or otherwise cannot use the POMBILITI and OPFOLDA provided through the Programs, I agree to promptly contact AMICUS ASSIST to arrange for product return or disposal. I understand that eligibility under these Programs is subject to Amicus' approval and the patient's and provider's continuing compliance with all eligibility and Program requirements, as set by Amicus from time to time. I agree to provide Amicus, or its authorized agent(s), access to the medical, financial and insurance records that this patient has authorized (in a signed, written authorization) me to disclose to Amicus and its authorized representatives for the purposes of verifying the patient's eligibility status for the Program and the patient's receipt of any product(s) provided to him or her through the Program.

15 **Prescriber signature** _____ **Date:** _____

**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.

**Please see IMPORTANT SAFETY INFORMATION throughout and on page 3 and see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCPcom.**

Page 2 of 4

## PAGE 2

11 **Be sure to write the patient's name on each page** in case pages are separated.

12 Photocopies (front and back) of the patient's medical and prescription insurance cards should be included when submitting this form.

13 Select the site of care. Depending on your selection of site of care, AMICUS ASSIST will investigate benefits and eligibility. (Select "Transition to Home Prescriber Direction" if the patient will begin infusions at a treatment center and will transition to home when directed by the prescriber.)

14 **Select or provide the appropriate shipping address.**

15 Please review this section and sign to acknowledge the conditions for obtaining and handling product provided through the Amicus Patient Assistance Program (PAP) and Bridge Program for eligible patients.

**Please be sure to obtain signatures in all highlighted areas, as applicable, before submitting to AMICUS ASSIST.**

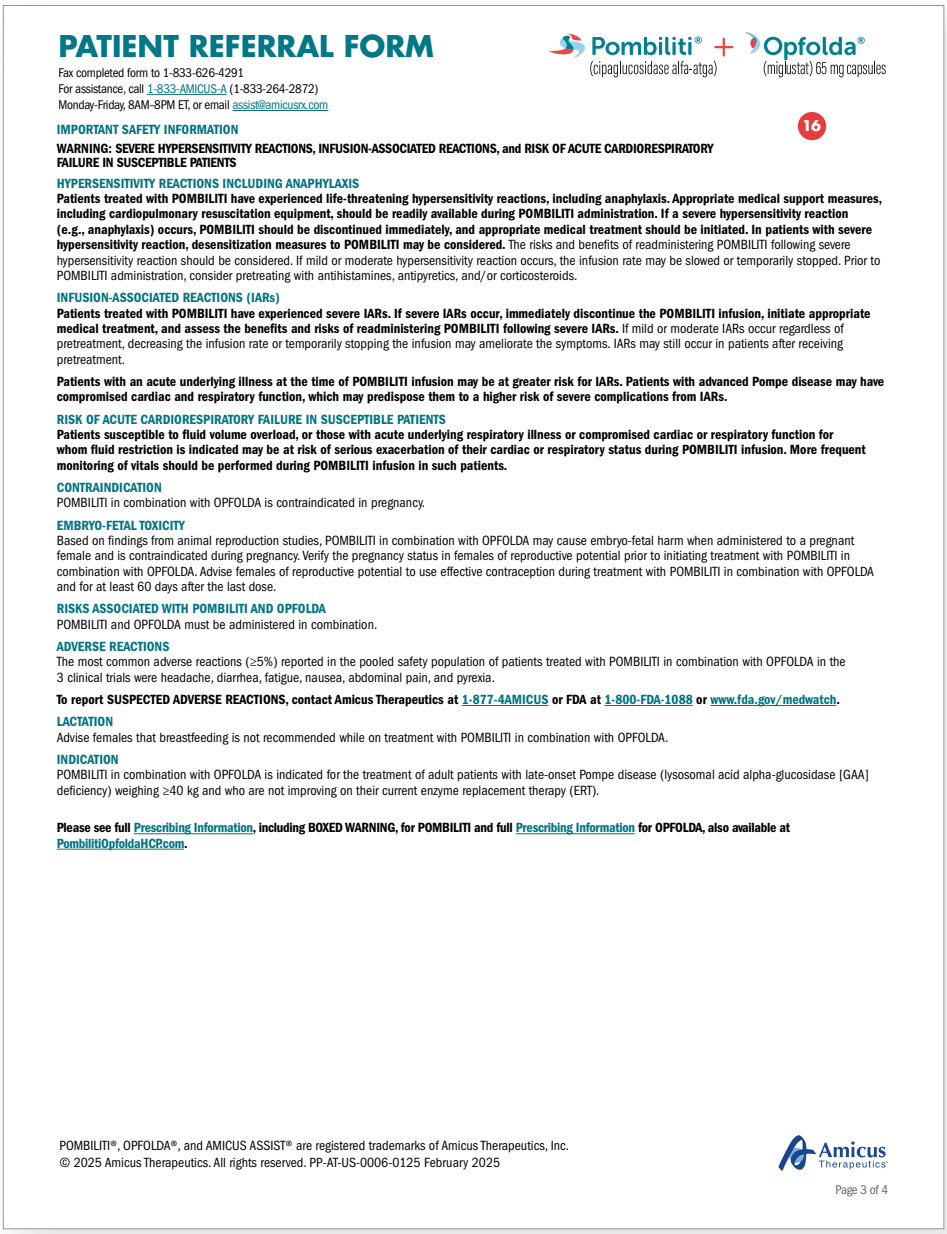
## IMPORTANT SAFETY INFORMATION (continued)

### 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.**

**Please see additional Important Safety Information throughout and on page 6 and full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**



## PATIENT REFERRAL FORM

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

Fax completed form to 1-833-626-4291
For assistance, call 1-833-AMICUS-A (1-833-264-2872)
Monday-Friday, 8AM–8PM ET, or email assist@amicusrx.com

16

**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.

**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.**

**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.

**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).

**Please see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**



Amicus Therapeutics



## PAGE 3

16 **This page includes the full Indication and Important Safety Information for POMBILITI® (cipaglucosidase alfa-atga) + OPFOLDA® (miglustat).** Please see full **Prescribing Information**, including BOXED WARNING, for POMBILITI and full **Prescribing Information** for OPFOLDA, also available at **PombilitiOpfoldaHCP.com**.

## IMPORTANT SAFETY INFORMATION (continued)

### 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.**

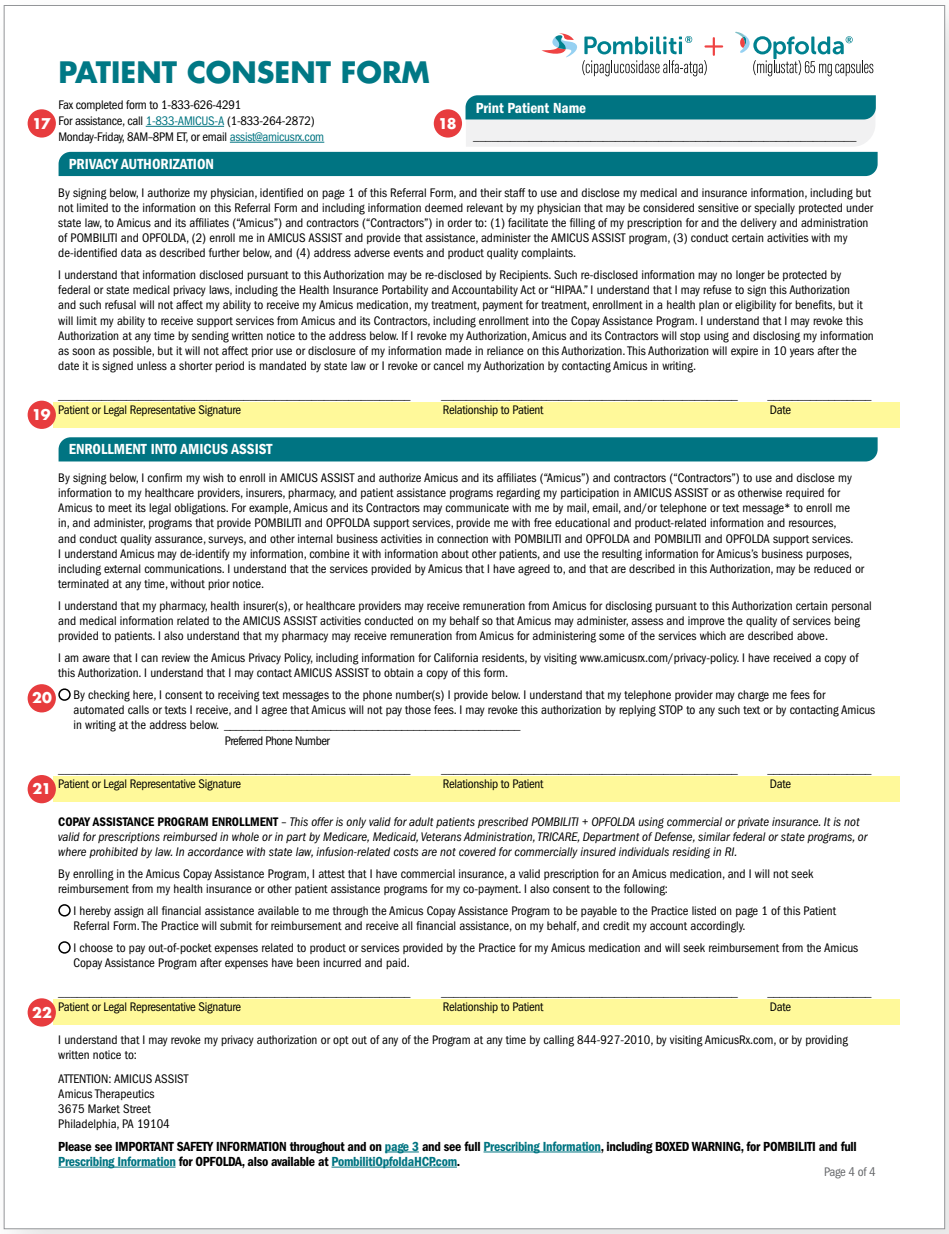
### CONTRAINDICATION

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.

**Please see additional Important Safety Information throughout and on page 6 and full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**



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

## PATIENT CONSENT FORM

17 Fax completed form to 1-833-626-4291
For assistance, call 1-833-AMICUS-A (1-833-264-2872)
Monday-Friday, 8AM–8PM ET, or email assist@amicusrx.com

18 Print Patient Name _____

**PRIVACY AUTHORIZATION**

By signing below, I authorize my physician, identified on page 1 of this Referral Form, and their staff to use and disclose my medical and insurance information, including but not limited to the information on this Referral Form and including information deemed relevant by my physician that may be considered sensitive or specially protected under state law, to Amicus and its affiliates ("Amicus") and contractors ("Contractors") in order to: (1) facilitate the filling of my prescription for and the delivery and administration of POMBILITI and OPFOLDA, (2) enroll me in AMICUS ASSIST and provide that assistance, administer the AMICUS ASSIST program, (3) conduct certain activities with my de-identified data as described further below, and (4) address adverse events and product quality complaints.

I understand that information disclosed pursuant to this Authorization may be re-disclosed by Recipients. Such re-disclosed information may no longer be protected by federal or state medical privacy laws, including the Health Insurance Portability and Accountability Act or "HIPAA." I understand that I may refuse to sign this Authorization and such refusal will not affect my ability to receive my Amicus medication, my treatment, payment for treatment, enrollment in a health plan or eligibility for benefits, but it will limit my ability to receive support services from Amicus and its Contractors, including enrollment into the Copay Assistance Program. I understand that I may revoke this Authorization at any time by sending written notice to the address below. If I revoke my Authorization, Amicus and its Contractors will stop using and disclosing my information as soon as possible, but it will not affect prior use or disclosure of my information made in reliance on this Authorization. This Authorization will expire in 10 years after the date it is signed unless a shorter period is mandated by state law or I revoke or cancel my Authorization by contacting Amicus in writing.

19 Patient or Legal Representative Signature _____ Relationship to Patient _____ Date _____

**ENROLLMENT INTO AMICUS ASSIST**

By signing below, I confirm my wish to enroll in AMICUS ASSIST and authorize Amicus and its affiliates ("Amicus") and contractors ("Contractors") to use and disclose my information to my healthcare providers, insurers, pharmacy, and patient assistance programs regarding my participation in AMICUS ASSIST or as otherwise required for Amicus to meet its legal obligations. For example, Amicus and its Contractors may communicate with me by mail, email, and/or telephone or text message* to enroll me in, and administer, programs that provide POMBILITI and OPFOLDA support services, provide me with free educational and product-related information and resources, and conduct quality assurance, surveys, and other internal business activities in connection with POMBILITI and OPFOLDA and POMBILITI and OPFOLDA support services. I understand Amicus may de-identify my information, combine it with information about other patients, and use the resulting information for Amicus's business purposes, including external communications. I understand that the services provided by Amicus that I have agreed to, and that are described in this Authorization, may be reduced or terminated at any time, without prior notice.

I understand that my pharmacy, health insurer(s), or healthcare providers may receive remuneration from Amicus for disclosing pursuant to this Authorization certain personal and medical information related to the AMICUS ASSIST activities conducted on my behalf so that Amicus may administer, assess and improve the quality of services being provided to patients. I also understand that my pharmacy may receive remuneration from Amicus for administering some of the services which are described above.

I am aware that I can review the Amicus Privacy Policy, including information for California residents, by visiting www.amicusrx.com/privacy-policy. I have received a copy of this Authorization. I understand that I may contact AMICUS ASSIST to obtain a copy of this form.

20 ○ By checking here, I consent to receiving text messages to the phone number(s) I provide below. I understand that my telephone provider may charge me fees for automated calls or texts I receive, and I agree that Amicus will not pay those fees. I may revoke this authorization by replying STOP to any such text or by contacting Amicus in writing at the address below. _____
Preferred Phone Number

21 Patient or Legal Representative Signature _____ Relationship to Patient _____ Date _____

**COPAY ASSISTANCE PROGRAM ENROLLMENT** – *This offer is only valid for adult patients prescribed POMBILITI + OPFOLDA using commercial or private insurance. It is not valid for prescriptions reimbursed in whole or in part by Medicare, Medicaid, Veterans Administration, TRICARE, Department of Defense, similar federal or state programs, or where prohibited by law. In accordance with state law, infusion-related costs are not covered for commercially insured individuals residing in RI.*

By enrolling in the Amicus Copay Assistance Program, I attest that I have commercial insurance, a valid prescription for an Amicus medication, and I will not seek reimbursement from my health insurance or other patient assistance programs for my co-payment. I also consent to the following:

○ I hereby assign all financial assistance available to me through the Amicus Copay Assistance Program to be payable to the Practice listed on page 1 of this Patient Referral Form. The Practice will submit for reimbursement and receive all financial assistance, on my behalf, and credit my account accordingly.

○ I choose to pay out-of-pocket expenses related to product or services provided by the Practice for my Amicus medication and will seek reimbursement from the Amicus Copay Assistance Program after expenses have been incurred and paid.

22 Patient or Legal Representative Signature _____ Relationship to Patient _____ Date _____

I understand that I may revoke my privacy authorization or opt out of any of the Program at any time by calling 844-927-2010, by visiting AmicusRx.com, or by providing written notice to:

ATTENTION: AMICUS ASSIST
Amicus Therapeutics
3675 Market Street
Philadelphia, PA 19104

**Please see IMPORTANT SAFETY INFORMATION throughout and on page 3 and see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**

Page 4 of 4

## PAGE 4

17 To submit the form, fax it to **1-833-626-4291**.

18 **Be sure to write the patient's name on each page** in case pages are separated.

19 **The patient should sign here** to authorize the sharing of their personal information with Amicus for the stated purposes.

20 **The patient should check here** if they would like to consent to receiving text messages from AMICUS ASSIST®. If they check here, they must provide a cell phone number.

21 **The patient should sign here** to enroll in AMICUS ASSIST support services.

22 **The patient should sign here** to enroll in the Amicus Co-Pay Assistance Program, if eligible. The patient should select whether they would like to assign the co-pay assistance benefits to the Practice or pay out-of-pocket costs and seek reimbursement.

If you have any questions or need further assistance, please contact the AMICUS ASSIST team at

**1-833-AMICUS-A (1-833-264-2872)**

They're available Monday–Friday, 8AM–8PM ET

**REFERENCES: 1.** POMBILITI. Prescribing information. Amicus Therapeutics US, LLC; 2024.
**2.** OPFOLDA. Prescribing information. Amicus Therapeutics US, LLC; 2024.

## IMPORTANT SAFETY INFORMATION (continued)

### 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.

**Please see additional Important Safety Information throughout and on page 6 and full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**

## 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.

### 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.**

### 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.

### 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).

**Please see full Prescribing Information, including BOXED WARNING, for POMBILITI and full Prescribing Information for OPFOLDA, also available at PombilitiOpfoldaHCP.com.**



Amicus Therapeutics®