PROCYSBI® (CYSTEAMINE BITARTRATE) DELAYED-RELEASE CAPSULES AND DELAYED-RELEASE ORAL GRANULES PATIENT ENROLLMENT FORM INSTRUCTIONS

The Patient Enrollment Form is required to initiate treatment with Amgen Rare Disease prescription medicine, PROCYSBI.

Instructions:

1. Fill out all patient information, including the most recent results of a white blood cell (WBC) cystine level test, recent history with CYSTAGON® (cysteamine bitartrate) capsules, and use of a gastrostomy tube (G-tube).
2. Fill out all required prescriber information, including all contact information for the practice or facility.
3. Complete and/or review all required insurance information for the patient and, if possible, attach copies of the patient's insurance cards for primary as well as supplementary insurance.
4. Complete the prescription and clinical information in its entirety; all fields are required. Reference the included select PROCYSBI dosing instructions or the PROCYSBI Full Prescribing Information for complete dosing information.
5. Review, sign, and date the prescriber certification on page 2 of the Patient Enrollment Form.
6. Check with your patient to ensure he or she has completed the required Patient Authorization and Consent for Amgen By Your Side, a patient support program, in order to initiate patient support.
7. **Fax pages 1 -4 of this form, along with both sides of the patient's medical and prescription drug benefit cards, to the Amgen By Your Side team at 1-877-773-9411, or email them to PROCYSBIABYS@amgen.com. Retain a copy of this form in the patient's records.**
8. If you have any questions or comments, please contact Amgen by Your Side at 1 (855) 888-4004.

Please see IMPORTANT SAFETY INFORMATION on last page and click here for the PROCYSBI Full Prescribing Information.

AMGEN BY your side

PATIENT ENROLLMENT FORM

Once complete, submit pages 1-4 by fax 1 (877) 773-9411, or email it to PROCYSBIABYS@amgen.com

Initiate the patient enrollment process by completing ALL REQUIRED FIELDS indicated by *.
For patient support and/or assistance obtaining patient signature, call Amgen By Your Side at 1 (855) 888-4004.

PROCYSBI® (cysteamine bitartrate) delayed-release capsules delayed-release oral granules

USA-711-80007 02/25

1. PATIENT INFORMATION

First name* _____ **Last name*** _____ Primary language _____

Address* _____ **City*** _____ **State*** _____ **ZIP*** _____

Mobile phone* _____ ○ Primary **Home phone*** _____ ○ Primary **Date of Birth*** ___/___/_____ **Gender*:** ○ Male ○ Female

Email* _____ **Height*:** _____ **Weight*:** _____ lbs or _____ kg

Currently taking CYSTAGON® (cysteamine bitartrate)? ○ Yes ○ No

Last CYSTAGON daily dose (mg/day) _____

Currently on dialysis? ○ Yes ○ No

Does the patient have a G-tube (feeding tube)? ○ Yes ○ No

White blood cell (WBC) test in the last year? ○ Yes ○ No

(A bolus [straight] feeding tube 14 French or larger is recommended.)

ALTERNATIVE CONTACT AND/OR CAREGIVER

First name _____ Last name _____ Mobile phone _____

Home phone _____ Email _____ Relationship to patient _____

2. PRESCRIBER INFORMATION

Preferred Method of Contact ☐ **Email** ☐ **Phone**

First name* _____ **Last name*** _____ **NPI#*** _____ **State License*** _____ **Tax ID*** _____

Address* _____ **City*** _____ **State*** _____ **ZIP*** _____

Phone* _____ **Fax number*** _____ **Prescriber Specialty*** _____

Office contact name* _____ **Office contact email*** _____ **Office contact phone*** _____

3. INSURANCE INFORMATION

Please attach copies of the front and back of patient's medical and prescription insurance cards.

☐ **No Insurance**

Primary insurance* _____

Insurance company phone* _____

Policy Type:* ○ Commercial ○ Medicaid ○ Medicare ○ Other

Policy #* _____ **Group #*** _____

Policyholder's name* _____

Relationship* _____ **Policyholder's Date of birth*** ___/___/_____ (MM/DD/YYYY)

Prescription Card*: ○ Yes ○ No

Secondary insurance _____

Insurance company phone _____

Policy Type: ○ Commercial ○ Medicaid ○ Medicare ○ Other

Policy # _____ Group # _____

Policyholder's name _____

Relationship _____ Policyholder's Date of birth ___/___/_____ (MM/DD/YYYY)

If yes, carrier _____ Phone _____

Identification # _____ Policy/Group # _____ Policyholder's Date of birth ___/___/_____ (MM/DD/YYYY)

Policyholder Name _____ Relationship _____

Complete signatures and prescription information on next page

USA-711-80007 02/25

4. PATIENT CONSENT AND AUTHORIZATION (Required—please see language on pages 3-4.)

You must read the Consent to Health Data Processing on page 4 and then select one of the below responses.
Select "I consent" to proceed with enrollment. If you select "I do not consent," you will not be able to enroll in Amgen By Your Side

- ○ **I consent to the collection, processing, and disclosure of my Health Data for the purposes set forth on page 4.**
- ○ **I do not consent to the collection, processing, or disclosure of my Health Data for the purposes set forth on page 4.**

By signing below, I am indicating that I have read and understood the Authorization for Use and Disclosure of Protected Health Information (pages 3-4), that I am legally authorized to consent, and that I am providing my consent as the patient or the patient's legal representative for Amgen and its contractors and business partners to use and share the personal information I provide for the purposes described within the Authorization for Use and Disclosure of Protected Health Information.

Patient name*

Name of Legal Representative (if needed)

Signature of Patient (or legal representative)*

Date* (MM/DD/YYYY)

5. PRESCRIPTION AND CLINICAL INFORMATION

Patient first name*

Patient last name*

Date of Birth* (MM/DD/YYYY)

Diagnosis (ICD-10-CM Code) ☐ E72.04 ☐ Other _____

Drug Name: ☐ PROCYSBI Capsules ☐ 25 mg _____ Quantity and/or ☐ 75 mg _____ Quantity

Directions: _____ mg Prescribed Total Daily Dose

_____ Days' Supply _____ Refills

Drug Name: ☐ PROCYSBI Granule Packets: ☐ 75 mg _____ Quantity and/or ☐ 300 mg _____ Quantity

Directions: _____ mg Prescribed Total Daily Dose

_____ Days' Supply _____ Refills

eg, Capsules: 600 mg q12h or 500 mg (6 x 75 mg capsules + 2 x 25 mg capsules) q12h.
Packets: 600 mg q12h or 525 mg (1 x 300 mg packets + 3 x 75 mg packets) q12h.

Dose Titration, see PROCYSBI Dosing Information for Healthcare Prescribers for more information.

Note: 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.**

Is the patient allergic to penicillamine, cysteamine, or any other medication? If yes, please list: _____

○ No known drug allergies (NKDA)

State requirements: 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.

Signature below indicates prescription authorization and prescriber certification.

Prescriber Signature (Dispense as Written)*

Written or e-signature only; stamps not acceptable.

Prescriber Signature (Substitutions Allowed)

Date* (MM/DD/YYYY)

Prescriber Certification: I certify that the above therapy is medically necessary, that the information provided is accurate to the best of my knowledge and that my patient is being administered PROCYSBI, in accordance with the labeled use of the product. I represent that my patient has requested and authorized the disclosure of their personal information to Amgen, Inc. and its affiliates and their respective employees or agents (collectively, "Amgen") for Amgen to administer the Amgen By Your Side program (the "Program"), which provides patient-focused support, including providing logistical and non-medical treatment support for PROCYSBI, as prescribed, and educating about the insurance process. I further represent that I have explained to the patient, and the patient indicated they understand and have consented to, the following: 1) Amgen will use the patient's name, date of birth, contact information, prescriptions, and other necessary health information to administer the Program; 2) Amgen will then disclose the patient's personal information to the patient's insurer(s) for the same purposes; 3) the patient can withdraw their consent by contacting Amgen at 1-844-469-4297 or visiting www.amgen. com/DataSubjectRights, but if +the patient does not agree to, or withdraws consent for, these uses and disclosures, the patient cannot receive these patient support services for this medication which necessarily requires Amgen to process the patient's personal information; and 4) the patient can view more details about Amgen's privacy practice at www.amgen. com/privacy. I authorize Amgen to transmit this prescription on my behalf to the appropriate pharmacy designated by the patient utilizing their benefit plan by any means allowed under appliable law. I further understand and agree that (a) any medication or service provided through the Program as a result of this form is for the named patient only and is not being made in exchange for any express or implied agreement or understanding that I would recommend, prescribe, or use PROCYSBI or any other Amgen product or service, for any other person; (b) my decision to prescribe PROCYSBI was based solely on my professional determination of medical necessity; and (c) I will not seek reimbursement for any medication or service provided by or through the Program from any government program or third-party insurer. I understand that Amgen may modify or terminate the Program at any time without notice. The completion and submission of coverage- or reimbursement-related documentation are the responsibility of the patient and healthcare provider. Amgen makes no representation or guarantee concerning coverage or reimbursement for any item or service.

State requirements: I certify that the prescription I am submitting as part of this Patient Enrollment Form complies with my state's prescription requirements (e.g., e-prescribing, state-specific prescription form, fax language). I understand that noncompliance with my state's specific prescription requirements will result in outreach to me to obtain a compliant prescription.

By filling out and signing this form, the enrollment process in Amgen By Your Side has initiated; however, your patient must sign a Patient Authorization to complete enrollment in Amgen By Your Side. Please note that your patient will not benefit from the services and support offered by the Program unless your patient signs a Patient Authorization, consenting to receiving such services. If your patient does not sign the Patient Authorization contained within this form, Amgen will contact the patient to determine whether the patient is interested in signing a separate Patient Authorization.

AUTHORIZATION FOR USE AND DISCLOSURE OF PROTECTED HEALTH INFORMATION

Please read and provide signature in Patient Consent and Authorization section on page 2. USA-711-80007 02/25

Uses and Disclosure of Protected Health Information

I authorize Amgen and its data processors (collectively, "Amgen") to collect, use, and disclose my protected health information for the following purposes:

- To operate, administer, enroll me in, and/or continue my participation in the Amgen By Your Side program or any other Amgen-affiliated patient support services and activities related to my condition or treatment (for example, co-pay card programs, reimbursement assistance programs, drug coverage verification, patient access liaison services, adherence program and disease management support);
- To contact, with my permission, my doctor and the rest of my health care team and share with them my health information that may be useful for my care;
- To improve, develop, and evaluate Amgen's products, services, materials and programs related to my condition or treatment.

In order for Amgen to provide me with the services and/or programs described above, Amgen needs to collect and use my personal information, including my protected health information. I understand that my protected health information may include any information, in electronic or physical form, in the possession of or derived from a health care provider, health care plan, pharmacy, pharmaceutical company, laboratory and/or their contractor (each, a "Health Care Provider"). This may include select information from or about my medical history and general health, my health care plan benefits, payment limits or restrictions covered by my health care plan policy, and/or my adherence to my treatment.

I authorize my Health Care Providers to disclose my protected health information to Amgen, and between themselves, as necessary, but only for the purposes stated above in this Authorization. I understand that certain of my Health Care Providers (such as pharmacies and specialty pharmacies) may receive remuneration from Amgen in exchange for disclosing my protected health information and/or for using my information to contact me with communications about Amgen products which have been prescribed to me (for example, medication reminder programs and other patient support services).

Expiration, Right to Obtain a Copy, and Right to Cancel

I understand that by signing this form, I authorize my Health Care Providers or others who might hold my health information to disclose it to Amgen. I also understand I am authorizing my personal information, including my protected health information, to be used for the purposes described above. I understand and agree that by signing below, I am authorizing those who rely on this Authorization to disclose my protected health information for the earlier of five (5) years or until my participation in the Amgen By Your Side program ends through my cancellation, unless a shorter time period is required by state law.

I understand that I can obtain a copy of this Authorization or cancel this Authorization at any time by calling Amgen at 1-844-469-4297 or by writing to Amgen By Your Side, 1 Horizon Way, Deerfield, IL 60015. If I cancel this Authorization, I will no longer qualify for the services described. I also understand that if a Health Care Provider is disclosing my protected health information to Amgen in reliance on this Authorization on an on-going basis, my cancellation with Amgen will be effective with respect to any such Health Care Providers as soon as they receive notice of my cancellation.

No Effect on Treatment

I understand I do not have to sign this Authorization and that my enrollment in any of the services and/or programs described above is entirely voluntary. I understand that Amgen, as well as Health Care Providers, cannot require me, as a condition of having access to medications, prescription drugs, treatment or other care, to sign this Authorization. Federal Law (including HIPAA) requires a signed authorization in order for Amgen to collect my protected health information from my Health Care Providers. I understand I cannot participate in the listed services and/or programs without signing this Authorization or an equivalent authorization with my Health Care Providers.

Information Received from Health Care Providers

I understand that once my protected health information has been disclosed to Amgen, federal privacy laws may no longer apply and may no longer protect it from further disclosure, and that Amgen may disclose my protected health information to its data processors, contractors, and business partners for its business purposes. Amgen agrees, however, to protect my protected health information by only using and disclosing it as stated in the Authorization or as otherwise allowed or required by law.

U.S. STATE LAW CONSENT TO PROCESS HEALTH DATA FOR AMGEN BY YOUR SIDE

Please read and provide response in Patient Consent and Authorization section on page 2.

Consent to Health Data Processing for Amgen By Your Side

I consent to Amgen processing my Health Data for the following purposes:

- To enroll me and manage my participation in the Amgen By Your Side program, which includes activities related to my condition or treatment (for example, co-pay card programs, payer medication coverage verification, patient access liaison support, disease management support), and to manage Amgen's products, services, and programs related to my condition or treatment.

Amgen uses the following when it administers the Amgen By Your Side program:

- Health Data – my name (and the name of my caregiver if applicable), gender, date of birth, contact information and information relating to my health condition or treatment.

I understand that my consent to processing is required for me to participate in the Amgen By Your Side program. I also understand that Amgen will not sell my Health Data to third parties, but Amgen may disclose my Health Data to Amgen's data processors, contractors, and business partners for Amgen's business purposes related to the Amgen By Your Side program. I understand that Amgen may use my Health Data to contact me by mail, email, telephone, or text for the above purposes. Mobile Terms & Conditions can be found at AmgenByYourSide.com/mobile-terms-and-conditions. I also understand that if I do not consent to the use of my Health Data for the above purposes, I will not be able to participate in the Amgen By Your Side program. Finally, I understand that I may withdraw my consent to processing my Health Data for the above purposes at any time using one of the methods listed in the Additional Disclosures section below and that if I withdraw my consent, I will no longer be able to participate in the Amgen By Your Side program.

Additional Disclosures

I understand that participation in the Amgen By Your Side program is an optional service at no cost to me. The consent(s) above in no way affects my right to obtain any medications and I do not have to provide consent to be able to receive any medications. To obtain a copy of the consent(s) above or to withdraw my consent to collection, processing, and/or disclosure of my Health Data for any of the above purposes to which I have consented, I can contact Amgen by visiting www.amgen.com/DataSubjectRights or calling 1-844-469-4297. For more information about Amgen's privacy practices, Amgen's Privacy Statement can be found at http://www.amgen.com/privacy

AMGEN Rare Disease

PROCYSBI DOSING INFORMATION

FOR HEALTHCARE PRESCRIBERS

PROCYSBI®
(cysteamine bitartrate)
delayed-release capsules
delayed-release oral granules

PROCYSBI is available as[1]:

25 mg: 60 delayed-release capsules/bottle
75 mg: 250 delayed-release capsules/bottle
75 mg: 60 delayed-release packets/box
300 mg: 120 delayed-release packets/box

Patients starting PROCYSBI who are cysteamine naïve[1]

- Initiate cysteamine treatment immediately after diagnosis of nephropathic cystinosis
- Patients should be started on PROCYSBI at a fraction (1/6 to 1/4) of the maintenance dosage and gradually titrated up to the maintenance dosage over 4 to 6 weeks
  - o Patients 1 year to less than 6 years: Increase the dosage in 10% increments to the maintenance dosage, while monitoring white blood cell (WBC) cystine concentrations. Allow a minimum of 2 weeks between dosage adjustments. If a patient achieves the therapeutic target WBC cystine concentration at a dosage below the recommended weight-based maintenance dosage, then stop dosage escalation and use the dosage as the patient's maintenance dosage
  - o Patients 6 years of age and older: Gradually increase the dosage over 4 to 6 weeks until the maintenance dosage is achieved
- The maintenance dosage after initial dose escalation is 1.3 g/m^2 of body surface area per day divided into 2 doses given every 12 hours. The table below shows the recommended starting and maintenance dosages of PROCYSBI, converted from body surface area to body weight

Patients converting to PROCYSBI from immediate-release (IR) cysteamine (CYSTAGON)[1]

- When switching patients from IR cysteamine bitartrate to PROCYSBI, the starting total daily dose of PROCYSBI is equal to the previous total daily dose of IR cysteamine bitartrate. Divide the total daily dose by 2 and administer every 12 hours

Starting and Maintenance Dosage of PROCYSBI by Body Weight in Cysteamine-Naïve Patients 1 Year of Age and Older (Dosage Rounded Using Available Capsule and Packet Strengths)

Weight in kg	Starting PROCYSBI Dosage in mg Every 12 Hours, as a Fraction of the Maintenance Dosage		Maintenance PROCYSBI Dosage in mg Every 12 Hours*
	1/6 of dosage	1/4 of dosage	
5 or less	25	50	200
6 to 10	50	75	300
11 to 15	75	100	400
16 to 20	100	125	500
21 to 25	100	150	600
26 to 30	125	175	700
31 to 40	125	200	800
41 to 50	150	225	900
51 and greater	175	250	1000

*Higher dosages may be required to achieve target therapeutic WBC cystine concentration.

Monitoring dosage[1]

- If a patient's precise calculated dosage cannot be obtained, round to the nearest 25 mg for capsules or 75 mg for packets. Only use whole capsules and packets
- After maintenance dosage of PROCYSBI has been achieved, measure the WBC cystine concentration and titrate the PROCYSBI dosage as needed to achieve target WBC cystine concentrations
- If a dosage adjustment is necessary, increase the dosage by 10%. For patients 1 year to less than 6 years of age, allow a minimum of 2 weeks between dose increments. The maximum dosage of PROCYSBI is 1.95 g/m2 per day

If tolerability issues occur with PROCYSBI[1]

- **If adverse reactions occur, decrease the PROCYSBI dosage and then gradually increase to the maintenance dosage**
- **For cysteamine-naïve patients who have initial intolerance, temporarily discontinue PROCYSBI and then restart at a lower dosage and gradually increase to the maintenance dosage**

Please click here for the Full Prescribing Information for complete dosing and administration instructions.

Adherence to cystine-depleting therapy is critical for optimal cystine control[2,3]

- Patients/caregivers should be urged to take PROCYSBI consistently according to the dosing schedule recommended in the prescribing information[1]

References: 1. PROCYSBI (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules [prescribing information] Amgen. **2.** Gahl WA, Thoene JG, Schneider JA. Cystinosis. *N Engl J Med.* 2002;347(2):111-121. **3.** Brodin-Sartorius A, Tête M-J, Niaudet P, et al. Cysteamine therapy delays the progression of nephropathic cystinosis in late adolescents and adults. *Kidney Int.* 2012;81(2):179-189.

USA-711-80007 02/25

INDICATION AND IMPORTANT SAFETY INFORMATION

INDICATION

PROCYSBI (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules is a cystine-depleting agent indicated for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

- Patients with serious hypersensitivity reaction, including anaphylaxis to penicillamine or cysteamine.

WARNINGS AND PRECAUTIONS

- **Ehlers-Danlos-like Syndrome:** Skin and bone lesions that resemble clinical findings for Ehlers-Danlos-like syndrome have been reported in patients treated with high doses of immediate-release cysteamine bitartrate or other cysteamine salts. Monitor patients for development of skin or bone lesions and reduce PROCYSBI dosing if patients develop these lesions.
- **Skin Rash:** Severe skin rashes such as erythema multiforme bullosa or toxic epidermal necrolysis have been reported in patients receiving immediate-release cysteamine bitartrate. Discontinue use if severe skin rash occurs.
- **Gastrointestinal (GI) Ulcers and Bleeding:** GI ulceration and bleeding have been reported in patients receiving immediate-release cysteamine bitartrate. Monitor for GI symptoms and consider decreasing the dose if severe symptoms occur.
- **Fibrosing Colonopathy:** Fibrosing colonopathy has been reported with postmarketing use of PROCYSBI. Evaluate patients with severe, persistent, and/or worsening abdominal symptoms for fibrosing colonopathy. If the diagnosis is confirmed, permanently discontinue PROCYSBI and switch to immediate-release cysteamine bitartrate capsules.
- **Central Nervous System (CNS) Symptoms**: CNS symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy have been associated with immediate-release cysteamine. Monitor for CNS symptoms; interrupt or reduce the dose for severe symptoms or those that persist or progress.
- **Leukopenia and/or Elevated Alkaline Phosphatase Levels:** Cysteamine has been associated with reversible leukopenia and elevated alkaline phosphatase levels. Monitor white blood cell counts and alkaline phosphatase levels; decrease or discontinue the dose until values revert to normal.
- **Benign Intracranial Hypertension:** Benign intracranial hypertension (pseudotumor cerebri; PTC) and/or papilledema has been reported in patients receiving immediate-release cysteamine bitartrate treatment. Monitor for signs and symptoms of PTC; interrupt or reduce the dose for signs/symptoms that persist, or discontinue if diagnosis is confirmed.

ADVERSE REACTIONS

The most common adverse reactions reported in PROCYSBI clinical trials (≥ 5%): were:

- Patients 2 years of age and older previously treated with cysteamine: vomiting, nausea, abdominal pain, headache, conjunctivitis, influenza, gastroenteritis, nasopharyngitis, dehydration, ear infection, upper respiratory tract infection, fatigue, arthralgia, cough, and pain in extremity.
- Patients 1 year of age and older naïve to cysteamine treatment: vomiting, gastroenteritis/viral gastroenteritis, diarrhea, breath odor, nausea, electrolyte imbalance, headache.

DRUG INTERACTIONS

- Drugs that increase gastric pH may alter the pharmacokinetics of cysteamine due to the premature release of cysteamine from PROCYSBI and increase WBC cystine concentration. Monitor WBC cystine concentration with concomitant use.
- Consumption of alcohol with PROCYSBI may increase the rate of cysteamine release and/or adversely alter the pharmacokinetic properties, as well as the effectiveness and safety of PROCYSBI.
- PROCYSBI can be administered with electrolyte (except bicarbonate) and mineral replacements necessary for management of Fanconi Syndrome as well as vitamin D and thyroid hormone.

USE IN SPECIFIC POPULATIONS

- Lactation: Because of the potential risk for serious adverse reactions in breastfed children from cysteamine, breastfeeding is not recommended during treatment with PROCYSBI.

Please see accompanying full Prescribing Information.

AMGEN Rare Disease

PROCYSBI® (cysteamine bitartrate) delayed-release capsules delayed-release oral granules