TranscendRare
A HORIZON CARES® program

PRESCRIPTION ENROLLMENT FORM

HORIZON PHARMA

Phone (855) 888-4004 | Fax (877) 773-9411
www.transcendrare.com

1. PATIENT INFORMATION

First Name _____ MI _____ Last Name _____

Address _____ City _____ State _____ ZIP _____

Home Phone _____ Mobile Phone _____

Date of Birth _____ Gender ☐ M ☐ F Height _____ Weight _____

Email _____ Preferred Method of Contact ☐ Home ☐ Mobile ☐ Email ☐ Mail

Currently taking Cystagon® ☐ Yes ☐ No Last Cystagon® Daily Dose (mg/day) _____

Currently taking Cystagon® with Food ☐ 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 Best Time to Contact _____

First Name _____ MI _____ Last Name _____

Home Phone _____ Mobile Phone _____

Email _____ Preferred Method of Contact ☐ Home ☐ Mobile ☐ Email ☐ Mail

2. PRESCRIBER INFORMATION

Prescriber First Name _____ MI _____ Last Name _____ Prescriber NPI# _____

Address _____ City _____ State _____ ZIP _____

Phone _____ Fax _____ Email _____

Physician Specialty _____ Office Contact Name _____ Phone _____

3. INSURANCE INFORMATION - Please attach a copy of both sides of the patient's insurance card(s) ☐ No Insurance

PRIMARY INSURANCE

Insurance Carrier _____

Customer Service Phone _____

Subscriber Name _____

Patient's Relationship to Subscriber _____

Subscriber Date of Birth _____

Subscriber ID Number _____

Policy/Employer/Group Number _____

SECONDARY INSURANCE (If any)

Insurance Carrier _____

Customer Service Phone _____

Subscriber Name _____

Patient's Relationship to Subscriber _____

Subscriber Date of Birth _____

Subscriber ID Number _____

Policy/Employer/Group Number _____

4. PRESCRIPTION AND CLINICAL INFORMATION

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

Drug Name: PROCYSBI® (cysteamine bitartrate) delayed-release capsules

Directions: _____ mg Prescribed Total Daily Dose _____ Days Supply _____ Refills

Eg, 600 mg q12h or 500 mg (6x 75 mg + 2x 25 mg) q12h.
Dose Titration E.g., 600 mg (8 x 75 mg capsules) q 12 hours; starting at 150 mg (2 x 75 mg capsules) q 12 hours for one week, increase by one 75 mg capsule per dose per week over six weeks to reach target dose of 600 mg q 12 hours.
Note: The prescriber is to comply with his/her state specific prescription requirements such as e-prescribing, state specific prescription form, fax language, etc.

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

Allergies _____

Physician Certification

By signing below I certify that (a) the above therapy is medically necessary and that I will supervise the patient's treatment accordingly, (b) I have received the necessary authorizations, including those required by state law and the Health Insurance Portability and Accountability Act of 1996 (HIPAA), to release the above information and other health and medical information of the patient to Horizon Orphan LLC, its agents and contracted dispensing pharmacies, to assist the patient in obtaining coverage for PROCYSBI. I appoint Horizon Orphan LLC and its agents to convey this prescription to the dispensing pharmacy.

X Substitution Permitted _____ X Dispense as Written _____ Date _____

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I hereby authorize my health care providers, my health insurance carriers, and my pharmacies to use and disclose my individually identifiable health information, including my medical diagnosis, condition, and treatment (including prescription information and lab test results), my health insurance information, and my name, address, and telephone number, to Horizon Orphan LLC and its agents and representatives, including third parties authorized by Horizon Orphan LLC to administer drug support and to dispense drugs (collectively, "TranscendRare™") for the following purposes: 1) to contact my health care provider to collect, enter, and maintain my health information in a database; 2) to contact my insurers as needed to verify my insurance coverage, review reimbursement issues, and assist with the processing of claims; 3) to determine my eligibility for TranscendRare; 4) to contact me to receive education, study the effectiveness of therapy, and to provide therapy support services designed for people taking drug therapy; 5) to provide me with Patient Access Manager services; and 6) as necessary to facilitate the operation and evaluation of the services provided by Horizon Orphan LLC.

TranscendRare agrees to protect my health information by using and disclosing my information only for the reasons listed above, pursuant to the requirements imposed under the Health Insurance Portability and Accountability Act of 1996 (HIPAA). I understand that federal privacy laws may no longer protect my health information after its disclosure to TranscendRare and that it may be subject to re-disclosure.

I understand that I am entitled to a copy of this signed Authorization and may revoke (withdraw) this Authorization at any time by faxing a signed, written request to TranscendRare at (877) 773-9411. TranscendRare will no longer seek disclosure of my health information from my health care providers and my health insurance carriers once it has received and processed my revocation. However, revoking this Authorization will not affect any use and disclosure of the health information that has already occurred in reliance on my authorization. If I revoke this Authorization I will no longer be able to receive TranscendRare Support services. My authorization is valid for five (5) years from the date I sign unless I revoke it earlier.

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 Horizon Orphan LLC, as well as my 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 TranscendRare to collect this 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.

Patient's Signature _____ Date _____

Print Patient's Name _____

Legally Authorized Representative's Signature (if needed) _____

Print Legally Authorized Representative's Name _____

Relationship to Patient ☐ Spouse ☐ Legal Guardian ☐ Representative per Power of Attorney

Representative's Address _____

Phone _____ Mobile Phone _____

Fax this form, along with both sides of the patient's Medical and Prescription Drug Benefit cards, to TranscendRare at 1-877-773-9411. Retain a copy of this form in the patient's records.

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PROCYSBI® (cysteamine bitartrate) delayed-release capsules

PROCYSBI® (cysteamine bitartrate) DOSING WORKSHEET

FOR HEALTH CARE PRESCRIBERS

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

- Starting total daily dose of PROCYSBI is equal to the previous total daily dose of IR cysteamine

Available as:	**60 25-mg capsules/bottle** **250 75-mg capsules/bottle**

Patients naïve to cysteamine:

- Treatment with cysteamine should be started immediately after diagnosis
- Patients should be on a "low and slow" titration schedule
- A titration period of 4 to 6 weeks starting at 1/6 to 1/4 of the maintenance dose helps reduce the risk of side effects.[1] The recommended starting dosage of PROCYSBI for cysteamine-naive patients is 0.2 to 0.3 g/m^2 per day divided into 2 doses given every 12 hours
- The weight–based dose corresponding to the recommended maintenance dose of 1.3 g/m^2/day can be estimated using the table below[1]

PROCYSBI Weight-Based Dosage*

(per recommended 1.3 grams/m^2/day maintenance dosage)[1] divided into 2 divided doses

Weight in kilograms	PROCYSBI Target Maintenance Dose (mg/12 hours)	Number of Capsules Every 12 Hours					
		Starting Dosage as a Fraction of the Maintenance Dosage				Target Maintenance Dose	
		1/6 of Target†		1/4 of Target†			
		75 mg	25 mg	75 mg	25 mg	75 mg	25 mg
0-5	200	0	1	0	2	2	2
6-10	300	0	2	1	0	4	0
11-15	400	1	0	1	1	5	1
16-20	500	1	1	1	2	6	2
21-25	600	1	1	2	0	8	0
26-30	700	1	2	2	1	9	1
31-40	800	1	2	2	2	10	2
41-50	900	2	0	3	0	12	0
51 and greater	1000	2	1	3	1	13	1

- PROCYSBI capsules are available in 25-mg and 75-mg strengths
- If a patient's precise calculated dosage cannot be obtained, round to the nearest 25 mg
- After maintenance dose has been achieved, measure the white blood cell (WBC) cystine concentration and titrate the PROCYSBI dosage as needed to achieve target WBC cystine concentrations[1]

* Used as an approximation for body surface area.

† Proposed starting dose in cysteamine-naïve patients as a fraction of the maintenance dosage to be gradually titrated over 4 to 6 weeks until maintenance dosage is achieved.

If tolerability issues occur with PROCYSBI[1]:

- **Patients experiencing tolerability issues should restart PROCYSBI at a lower dose and gradually increase to a dose that achieves target WBC cystine levels[1]**

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 [package insert]. Novato, CA: Horizon Orphan LLC; 2016. **2.** Gahl WA, Thoene JG, Schneider JA. Cystinosis. *N Engl J Med*. 2002;347(2):111-121. **3.** Brodin-Sartorius A, Tète 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.

Please see IMPORTANT SAFETY INFORMATION on next page and accompanying Full Prescribing Information.

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IMPORTANT SAFETY INFORMATION

PROCYSBI®
(cysteamine bitartrate)
delayed-release capsules

INDICATIONS AND USAGE: PROCYSBI® (cysteamine bitartrate) delayed-release capsules is a cystine-depleting agent indicated for the treatment of nephropathic cystinosis in adult and pediatric patients 2 years of age and older.

CONTRAINDICATIONS:
Hypersensitivity 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.

- These include molluscoid pseudotumors (purplish hemorrhagic lesions), skin striae, bone lesions (including osteopenia, compression fractures, scoliosis and genu valgum), leg pain, and joint hyperextension.
- One patient on immediate-release cysteamine bitartrate with serious skin lesions subsequently died of acute cerebral ischemia with marked vasculopathy.
- Monitor patients for development of skin or bone lesions and interrupt PROCYSBI dosing if patients develop these lesions. PROCYSBI may be restarted at a lower dose under close supervision, then slowly increase to the appropriate therapeutic dose.

Skin Rash: Severe skin rashes such as erythema multiforme bullosa or toxic epidermal necrolysis have been reported in patients receiving immediate-release cysteamine bitartrate. If severe skin rashes develop, permanently discontinue use of PROCYSBI.

Gastrointestinal Ulcers and Bleeding: Gastrointestinal (GI) ulceration and bleeding have been reported in patients receiving immediate-release cysteamine bitartrate.

- GI tract symptoms including nausea, vomiting, anorexia, and abdominal pain, sometimes severe, have been associated with cysteamine. If severe GI tract symptoms develop, consider decreasing the dose of PROCYSBI.

Central Nervous System Symptoms: Central Nervous System (CNS) symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy have been associated with immediate-release cysteamine.

- Neurological complications have also been described in some patients with cystinosis who have not been treated with cysteamine.
- Carefully evaluate and monitor patients who develop CNS symptoms. Interrupt medication or adjust the dose as necessary for patients with severe symptoms or with symptoms that persist or progress.
- Inform patients that PROCYSBI may impair their ability to perform tasks such as driving or operating machinery.

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. If tests values remain elevated, consider decreasing the dose or discontinuing the drug 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 patients for signs and symptoms of PTC, including headache, tinnitus, dizziness, nausea, diplopia, blurry vision, loss of vision, pain behind the eye or pain with eye movement. If signs/symptoms persist, interrupt dosing or decrease the dose and refer the patient to an ophthalmologist. If the diagnosis is confirmed, permanently discontinue use of PROCYSBI.

ADVERSE REACTIONS:
The most common adverse reactions (≥5%) in patients treated in clinical trials are vomiting, nausea, abdominal pain, breath odor, diarrhea, skin odor, fatigue, rash, and headache.

To report SUSPECTED ADVERSE REACTIONS, contact Horizon Orphan LLC at 1-855-888-4004 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

DRUG INTERACTIONS:

- Drugs that Increase Gastric pH: Administer PROCYSBI at least 1 hour before or 1 hour after medications containing bicarbonate or carbonate.
- 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:

- Breastfeeding is not recommended while taking PROCYSBI.

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