raptor Cares®
www.RaptorCares.com
PRESCRIPTION ENROLLMENT FORM

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Phone: 1-855-888-4004 | Fax: 1-877-773-9411

1 Patient Information

Patient First Name _____ MI ____ Last Name _____ Date of Birth _____

Address _____ City _____ State _____ Zip _____

Gender ☐ M ☐ F Home Phone _____ Mobile Phone _____ Email _____

Height _____ Weight _____ Preferred Contact ☐ Home Phone ☐ Mobile Phone ☐ Email ☐ Postal Mail

☐ Currently taking Cystagon® Last Cystagon® Daily Dose (mg/day) _____ ☐ Currently taking Cystagon® with food

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)

Alternate Contact and/or Caregiver Best Time to Contact _____

First Name _____ MI ____ Last Name _____

Home Phone _____ Mobile Phone _____ Email _____

2 Prescriber Information

Prescriber's First Name _____ MI ____ Last Name _____

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)

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 _____

☐ No Insurance

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

_____ mg Prescribed Total Daily Dose _____ Days Supply _____ Refills

Directions:

Eg, 600 mg q12h or 500 mg (6 x 75 mg + 2 x 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: TN prescribers—quantity must be written in both numerals and words, eg: three (3) doses.

Is the patient allergic to any medications, 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 Raptor Pharmaceuticals Inc., its agents and contracted dispensing pharmacies, to assist the patient in obtaining coverage for PROCYSBI. I appoint Raptor and its agents to convey this prescription to the dispensing pharmacy.

X Physician Signature _____ Dispense as Written

Prescriber's full, usual, and actual signature is required — no stamps. This form cannot be processed without the prescriber's signature.

Date _____ Prescriber NPI# _____

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I hereby authorize my healthcare providers and my health insurance carriers 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 Raptor Pharmaceuticals Inc. and its agents and representatives, including third parties authorized by Raptor to administer drug support and to dispense drugs (collectively, "Raptor").

Raptor takes patient privacy seriously. Raptor may receive, use, and disclose my health information to determine my eligibility for RaptorCares; provide me with services (including reimbursement support and educational and therapy support services); administer and improve RaptorCares; and study the effect of cystine-depleting medication.

I understand that once my health information is shared with Raptor, federal privacy laws may no longer protect the information, which may be subject to redisclosure. Any findings published as a result of research using my information will include only aggregate data and will not identify me.

I further understand that:

- I do not have to sign this Authorization. My treatment, payment for treatment, insurance enrollment, or eligibility for insurance benefits, will not be directly affected. However, if I do not sign, I will not be eligible to participate in RaptorCares.
- I am entitled to a copy of this signed Authorization.
- I may revoke (cancel) this Authorization at any time by faxing a signed, written request to RaptorCares at 1-877-773-9411. RaptorCares will notify my healthcare providers and insurers of my revocation, at which point they will no longer disclose my health information to Raptor. However, revoking this Authorization will not affect Raptor's ability to use and disclose my health information that has already been received to the extent permitted under applicable law. If I revoke this Authorization I will no longer be able to receive RaptorCares services. Authorization is valid for 2 years.

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 RaptorCares at 1-877-773-9411. Retain a copy of this form in the patient's records.

12-HOUR
PROCYSBI®
(cysteamine bitartrate)
delayed-release capsules

PROCYSBI DOSING WORKSHEET FOR HEALTHCARE PRESCRIBERS

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

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

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

Patients naïve to cysteamine:

- 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 weight–based dose corresponding to the recommended maintenance dose of 1.3 grams/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]

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	
		⅛ of Target†		¼ 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: Raptor Pharmaceuticals Inc.; 2015. **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.

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

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IMPORTANT SAFETY INFORMATION

INDICATIONS AND USAGE: PROCYSBI® (cysteamine bitartrate) delayed-release capsule 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 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 Raptor Pharmaceuticals Inc. at 1-855-888-4004 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

DRUG INTERACTIONS:

- PROCYSBI should be administered 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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