# PRIOR AUTHORIZATION (PA) CHECKLIST FOR PROCYSBI® (cysteamine bitartrate) DELAYED-RELEASE CAPSULES AND DELAYED-RELEASE ORAL GRANULES

This checklist is for informational purposes only. For health plan–specific criteria, please contact a representative from **Amgen By Your Side**, a patient support program. Initiate your patient's enrollment in Amgen By Your Side by submitting the Patient Enrollment Form. Your patient must complete enrollment to access these patient-focused services and resources.

Although requirements vary by plan, below are the common criteria that may be requested for PROCYSBI. Case Managers can provide education about navigating insurance processes and accessing treatment during your patient's access journey.

## 1 BENEFITS INVESTIGATION

- ☐ PA requirements vary between plans. Contact the health plan to understand the process, step therapy requirements, duration of approval, and other relevant information

## 2 PA REQUIREMENTS

**Patient/Provider Information**

- ☐ Name
- ☐ Date of birth
- ☐ Health plan
- ☐ Provider name
- ☐ Provider identification number

Some plans may require documentation of specific information, while some may require physician attestation.

**Diagnosis Information**

- ☐ Diagnosis/ICD-10-CM code
  - — Cystinosis E72.04
- ☐ Diagnosis confirmed by one or more of the following methods:
  - — WBC cystine concentration count above the upper limit of normal reference range*
  - — Genetic test confirming mutation of the *CTNS* gene
  - — Demonstration of cystine corneal crystals by slit-lamp examination

Be sure to provide relevant clinical support, such as clinical notes, laboratory results, etc.

*CTNS*, cystinosin, lysosomal cystine transporter; ICD-10-CM, International Classification of Diseases, Tenth Revision, Clinical Modification; WBC, white blood cell.

*Ideal WBC cystine concentration is defined as <1.9 nmol ½ cystine/mg protein. When using the mixed leukocyte assay, the recommended target WBC cystine concentration is <1 nmol ½ cystine/mg protein.

### 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.

### SELECT IMPORTANT SAFETY INFORMATION

### CONTRAINDICATIONS

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

**Please see additional Important Safety Information on page 3 and see Full Prescribing Information.**

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

# PA CHECKLIST FOR PROCYSBI (CONT'D)

## 2 PA REQUIREMENTS (CONT'D)

**Treatment Information**

- ☐ Note any and all previous medications, including name, dosage, and dates or duration of treatment
- ☐ Note patient experience on other cystine-depleting agents
  - — If medication was discontinued, list all the reasons for discontinuation, including side effects, nonadherence, or comorbidities, if applicable
- ☐ Note attestation of the inability to swallow capsules or if gastrostomy tube placement is required, if requesting granules
- ☐ Include a letter of medical necessity
- ☐ Note reauthorization criteria (ie, documentation of positive clinical response to PROCYSBI)
- ☐ Note any consultations with a specialist (eg, geneticist, urologist, nephrologist, or endocrinologist)

Step therapy requirements may vary between plans.

## 3 PA SUBMISSION

- ☐ Submit the PA directly to the health plan or by using an electronic PA submission service, such as CoverMyMeds®
- ☐ Verify that the PA (including the number of pages) was received
- ☐ Check with the patient's plan to see how long it typically takes for a PA to be reviewed
- ☐ Communicate with the team at Amgen By Your Side to follow up on status and see if any additional information is required

PA, prior authorization.

Amgen By Your Side is a support program for patients prescribed PROCYSBI. After your patient has enrolled, they will be paired with a dedicated support partner, called a Patient Access Liaison (PAL). Their PAL can be a partner, providing nonmedical education to help them navigate their unique treatment experience – including information on insurance, financial support options, important appointment-related information, and other patient support services.

AMGEN BY your side

Available Monday through Friday, 9 AM to 8 PM ET

**1-844-469-4297**

**SELECT IMPORTANT SAFETY INFORMATION**

**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.

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# INDICATION and IMPORTANT SAFETY INFORMATION

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### WARNINGS AND PRECAUTIONS

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- **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 Full Prescribing Information.**

AMGEN Rare Disease



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