FOR HEALTHCARE PROVIDER OFFICE STAFF USE ONLY. DO NOT DISTRIBUTE.

Dear Healthcare Provider,

There are times when a prior authorization request may be denied by your patient's health plan. If that happens, an appeal can be submitted to the plan requesting that the decision be reconsidered. Appeal requirements may vary according to the health plan.

To use the sample letter provided as a separate Word document, modify the content as needed based on your medical judgment and discretion when providing a diagnosis and characterization of your patient's medical condition. For additional guidance, a checklist and tips have been included below and on page 2.

Use of the information in this document does not guarantee that the health plan will provide coverage for PROCYSBI® (cysteamine bitartrate) delayed-release capsules and/or delayed-release oral granules, and it is not intended to be a substitute for, or an influence on, your independent medical judgment.

Before sending the appeal letter to the health plan, please ensure all variable text (as indicated by brackets in pink and open text fields) is filled in or deleted as required.

APPEAL CHECKLIST

Documents for Filing a Response to Treatment Denial

Each appeal may require different information based on the plan's requirements. Below is a list of materials that you may need to include in an appeal package. Review each denial and the health plan's requirements to determine what to include in a patient's appeal package.

Commonly Required Documents Include

- ☐ Letter of appeal
- ☐ Letter of medical necessity
- ☐ Patient authorization and notice of release of information
- ☐ Copy of the patient's health plan and/or prescription card (front and back)
- ☐ Denial information, including the patient's denial letter and/or explanation of benefits
- ☐ Supporting documentation:
  - ☐ PROCYSBI Prescribing Information
  - ☐ PROCYSBI clinical studies
  - ☐ Clinical documentation such as:
    - ☐ White blood cell cystine concentration level above the upper limit of normal reference range
    - ☐ Genetic test confirming mutation of *CTNS* gene
    - ☐ Cystine corneal crystals by slit lamp examination
    - ☐ Treatment history, including therapeutics, dosage, and duration
    - ☐ Any relevant clinical/chart notes

CTNS, cystinosin, lysosomal cystine transporter.

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.

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

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

APPEAL TIPS

Tips for Filing an Appeal of Treatment Denial

This document provides information that may be useful when creating an appeal letter. Some plans have specific coverage authorization forms that must be used. It is important to determine the plan's requirements and follow them when requesting an appeal for PROCYSBI to avoid further treatment delays. Please contact health plans directly for specific information about their current coverage policies. Please note that Amgen By Your Side is a patient support program that provides education to healthcare providers and appropriate office staff to answer nonmedical logistical questions as well as information about insurance processes and accessing treatment.

Identify the Reason for Denial

Find out in writing why the authorization request has been denied. The denial letter from the patient's health plan or the explanation of benefits letter should outline the reason(s) for denial. These can be obtained from the health plan if you did not receive them. The denial is also summarized in the health plan's online portal or should be available where you submitted the prior authorization.

Determine the Appeal Guidelines

Some health plans have short appeal periods, so it is important to contact the health plan to find out its deadline for submitting an appeal. Be sure to inquire about the number of appeals permitted (some plans allow only 1) and the mailing address or fax number to which the appeal should be sent. You may also need to schedule a peer-to-peer consultation.

Contact the Review Department

The denial letter may include a telephone number for the review department. If so, the prescribing physician should call for further clarification about the denial. The reviewer may agree with the rationale and approve treatment during the call; if so, the appeal process is complete.

Compose the Appeal and Schedule a Consultation

The health plan will tell you what supporting documentation is needed. You may also need to schedule a peer-to-peer consultation.

Provide Additional Supporting Documentation

It is important to determine each plan's appeal requirements, as they may vary according to the payer. The appeal package should include all relevant medical documentation, including clinical notes and related test results, as well as any newly available information related to the patient's condition. The Amgen By Your Side team works directly with patients to answer nonmedical logistical questions and to provide information about insurance processes and treatment access.

Follow Up as Needed

Contact the health plan to learn about the appeal review timeline. Though some plans may respond within 7 days, most health plans respond within 30 to 60 days of receipt of the appeal package.

Maintain Complete Records

Retain a copy of all documentation submitted with the patient's appeal and record all subsequent communications made to the patient's health plan, including the date and the name of the person contacted.

NOTE: As a reminder, do not send patient medical records to Amgen.

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.

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INDICATION and IMPORTANT SAFETY INFORMATION

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CONTRAINDICATIONS

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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 Full Prescribing Information.

AMGEN Rare Disease

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