PROCYSBI® (cysteamine bitartrate) Delayed-Release Capsules and Delayed-Release Oral Granules Access Journey

A resource to educate healthcare providers about patient access and coverage

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 pages 12-13 and see Full Prescribing Information.

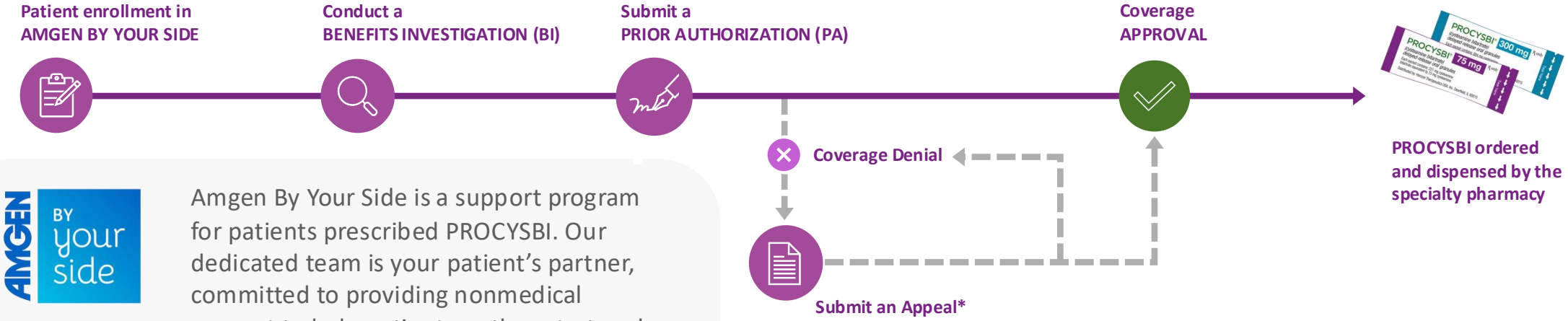
AMGEN Rare Disease

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

PROCYSBI Patient Access Road Map

This resource can help you identify the steps in the access process as well as available resources.

You can navigate through the road map by clicking on areas of the map or by using the navigation bar above. In each section, you will find more detailed information and resources available for that step of the process.



AMGEN BY your side

Amgen By Your Side is a support program for patients prescribed PROCYSBI. Our dedicated team is your patient's partner, committed to providing nonmedical support to help patients as they start and continue on treatment as you prescribe.

Call 1-844-469-4297
Monday–Friday, 9 AM–8 PM ET
www.AmgenByYourSide.com

*Submitting an appeal does not guarantee approval, and this process may need to be repeated.

Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

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Amgen Offers Patients a Range of Support Throughout Their Access and Treatment Journey

Coverage policies may vary, and the team at **Amgen By Your Side** can educate you and your staff about insurance processes, including specific payer requirements and examples.

Amgen By Your Side

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).

Patient Access Liaison (PAL)

The PAL can be a partner, providing nonmedical education to help your patient navigate their unique treatment experience—including information on insurance, financial support options, important appointment-related information, and other patient support services.

Amgen Case Manager

The Case Manager can help healthcare providers to understand their patients' benefits and unique access solutions. A Case Manager assigned to your patients may also be in touch with your office to make sure important insurance information is properly shared.

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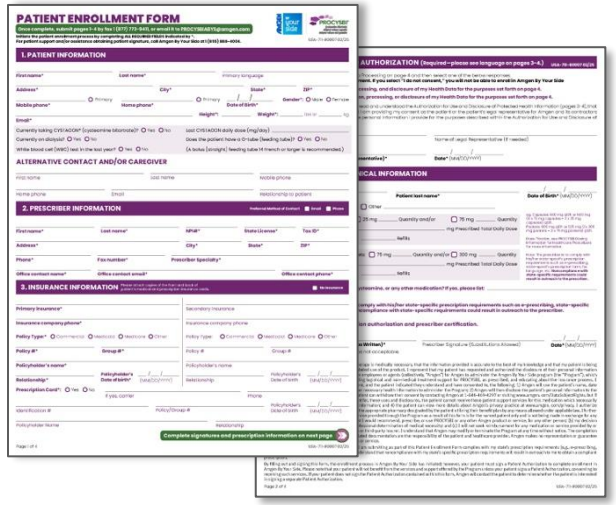
Initiate Patient Enrollment

Many treatments require initial action from the healthcare provider and office staff for patients to get access to PROCYSBI. The **Amgen By Your Side** Team is led by a Patient Access Liaison (PAL). The PAL is a dedicated support partner who helps investigate, explain, and educate on the steps in the treatment experience. They are your patient's point of contact and champion while your patient is accomplishing their treatment goals.


PATIENT ENROLLMENT FORM

1. PATIENT INFORMATION

ALTERNATIVE CONTACT AND/OR CAREGIVER

2. PRESCRIBER INFORMATION

3. INSURANCE INFORMATION

The Patient Enrollment Form (PEF) is the first step for your patients to receive support from Amgen By Your Side. The Amgen By Your Side Team can:

- **Educate** patients on the results of their benefits investigation and review their insurance coverage
- **Help** patients understand potential out-of-pocket costs and financial support options
- **Educate** you and your staff about insurance processes, including specific payer requirements and examples
- **Provide** education to you and your staff about product coding and billing
- **Share** additional resources that may be helpful as your patients get started

DOWNLOAD the PEF to initiate patient enrollment in Amgen By Your Side.

DOWNLOAD the Annotated PEF, a resource that provides details about the PEF.

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Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

Conduct a Benefits Investigation (BI)

Coverage criteria will vary among health plans and a BI will identify requirements specific to PROCYSBI. A Case Manager can help educate you and your patient about the insurance process and accessing treatment.

A BI may help answer questions about:

PROCYSBI coverage

- Is PROCYSBI covered under the medical benefit or pharmacy benefit?

Prior Authorization (PA)

- Will a PA be required for treatment with PROCYSBI, or for a specific formulation (eg, capsules or granule packets)?
- If a PA is not required, is predetermination available?
- What is the process for obtaining a PA or predetermination?
- What information will be required, and how long will the process take?
- How long will the PA remain valid?

DOWNLOAD the PROCYSBI PA Checklist to help your office organize the information that may be needed for a PA.

Benefits coordination

- Does the patient have any other supplemental insurance benefits that would require coordination? Which benefit is primary? Which is secondary?

Patient financial responsibility and out-of-pocket (OOP) costs

- What is the annual deductible amount the patient must meet?
  - Has this amount been met?
  - How much is left?
- What is the patient's co-payment or coinsurance for PROCYSBI?
- Is there a maximum OOP amount that the patient must meet?
  - Has this amount been met?
  - How much is left?

Prescription information

- Is PROCYSBI medically appropriate?
- Is PROCYSBI being prescribed in accordance with generally accepted standards of medical practice?

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Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

Conduct a Benefits Investigation (BI) (cont'd)

Coding and claims submission

A BI can help answer questions, such as:

- What are the specific coding and claims submission requirements for prescribing PROCYSBI in this patient's plan?
- What type of documentation is required?

Reminder: Requirements for coverage will vary among health plans and a BI will identify requirements specific to PROCYSBI, as well as what insurance your patient has.

PROCYSBI ICD-10-CM Code*	
Code	Description
E72.04	Cystinosis

PROCYSBI NDCs		
Code	**Quantity**	**Strength**
Delayed-release capsules		
75987-100-04	Bottle of 60 capsules	25 mg
75987-101-08	Bottle of 250 capsules	75 mg
Delayed-release oral granules		
75987-140-13	60 packets	75 mg
75987-140-14	120 packets	75 mg
75987-145-13	60 packets	300 mg
75987-145-14	120 packets	300 mg

Once the Amgen By Your Side Team completes the BI, you will receive a Summary of Benefits notification, generally within 1 to 2 business days of insurance verification.

*This may not be the only applicable code for coverage, nor does using this code guarantee coverage.

ICD-10-CM, International Classification of Diseases, Tenth Revision, Clinical Modification; NDC, National Drug Code.

Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

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Submit a Prior Authorization (PA)

The PA process allows the health plan to review the reason for treatment with PROCYSBI and to determine if it is medically appropriate. Clinical documentation requirements will vary among health plans.

Common criteria that a health plan policy may require for a PA:

Diagnosis information:

- Diagnosis/ICD-10-CM codes
- Diagnosis confirmed by 1 or more of the following methods:
  - White blood cell 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

Treatment history:

- Note any and all previous medications, including name, dosage, and dates/duration of treatment
- Note if the patient has experienced inadequate response of treatment OR intolerance to use of Cystagon® (cysteamine bitartrate)
- Note attestation of inability to swallow capsules or if gastrostomy tube (G-tube) placement is required, if requesting granules
- For patients already taking PROCYSBI, provide documentation showing clinical improvement
- Note any consultations with a specialist (eg, nephrologist, urologist, or endocrinologist)

Including a letter of medical necessity with a PA is important and may help avoid delays.

Your office may need to connect with the referring physician to gather the clinical documentation required to complete the PA. The dedicated Case Manager has the local expertise to provide education about PA, medical exception, or appeal processes.

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

Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

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Submit a Prior Authorization (PA) (cont'd)

The PA process allows the health plan to review the reason for the requested therapy and to determine medical appropriateness. Clinical documentation requirements will vary among health plans. When submitting a PA for PROCYSBI, be sure to:

- **Submit the PA directly to the health plan or by using an electronic PA system, such as CoverMyMeds®**
- **Thoroughly complete every section of the PA form and review the medical policy carefully, as each health plan may have unique requirements**
- **Provide supporting documentation, including but not limited to:**
  - Medical records
  - Diagnosis confirmed by one of the following methods: white blood cell cystine concentration count above the upper limit of normal reference range, genetic test confirming mutation of the *CTNS* gene, or demonstration of cystine corneal crystals by slit-lamp examination
  - Chart notes
  - Publications and references
  - A letter of medical necessity
- **Inquire about how long the process will take once necessary documents have been submitted**

DOWNLOAD the PA Checklist for reminders and recommendations for submitting a PA.

DOWNLOAD the Letter of Medical Necessity Template and print it out on your office letterhead.

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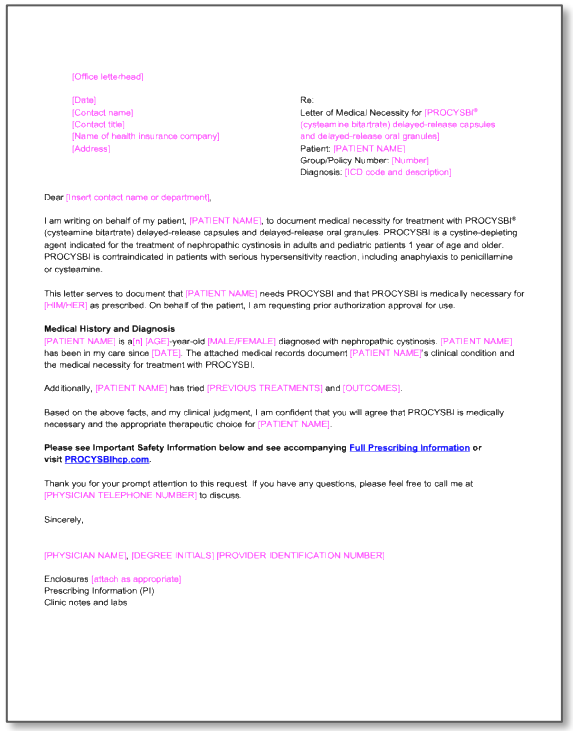
CTNS, cystinosin, lysosomal cystine transporter.

Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

Submit a Prior Authorization (PA) (cont'd)

Writing a letter of medical necessity

A patient-specific letter of medical necessity explains the physician's rationale and clinical decision-making in choosing PROCYSBI.



[Office letterhead]

[Date]
[Contact name]
[Contact title]
[Name of health insurance company]
[Address]

Re:
Letter of Medical Necessity for [PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules]
Patient: [PATIENT NAME]
Group/Policy Number: [Number]
Diagnosis: [ICD code and description]

Dear [Insert contact name or department],

I am writing on behalf of my patient, [PATIENT NAME], to document medical necessity for treatment with PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules. PROCYSBI is a cystine-depleting agent indicated for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older. PROCYSBI is contraindicated in patients with serious hypersensitivity reaction, including anaphylaxis to penicillamine or cysteamine.

This letter serves to document that [PATIENT NAME] needs PROCYSBI and that PROCYSBI is medically necessary for [HIM/HER] as prescribed. On behalf of the patient, I am requesting prior authorization approval for use.

Medical History and Diagnosis
[PATIENT NAME] is a[n] [AGE]-year-old [MALE/FEMALE] diagnosed with nephropathic cystinosis. [PATIENT NAME] has been in my care since [DATE]. The attached medical records document [PATIENT NAME]'s clinical condition and the medical necessity for treatment with PROCYSBI.

Additionally, [PATIENT NAME] has tried [PREVIOUS TREATMENTS] and [OUTCOMES].

Based on the above facts, and my clinical judgment, I am confident that you will agree that PROCYSBI is medically necessary and the appropriate therapeutic choice for [PATIENT NAME].

Please see Important Safety Information below and see accompanying Full Prescribing Information or visit PROCYSBIhcp.com.

Thank you for your prompt attention to this request. If you have any questions, please feel free to call me at [PHYSICIAN TELEPHONE NUMBER] to discuss.

Sincerely,

[PHYSICIAN NAME], [DEGREE INITIALS] [PROVIDER IDENTIFICATION NUMBER]

Enclosures [attach as appropriate]
Prescribing Information (PI)
Clinic notes and labs

The Letter of Medical Necessity Template for PROCYSBI can be customized based on your patient's medical history and demographic information. The template can help your office craft the letter and highlight the medical necessity for your patient.

NOTE: Some health plans may have specific forms that must be completed in order to document medical necessity.

- **Check with the health plan to identify specific documentation that needs to be submitted with a letter of medical necessity**
- **Provide relevant medical information and attach the patient's medical records and/or supporting documents for health plans to review**
- **Include a copy of the Full Prescribing Information**

DOWNLOAD the Letter of Medical Necessity Template and print it out on your office letterhead.

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Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

Submit an Appeal

An appeal letter may be needed if a PA for PROCYSBI is denied. When writing an appeal letter, ensure that you address the specific details of the denial reason(s), refer to the letter of denial for specific language regarding the reason(s) for denial, and address any concerns that are patient-specific.

Supplemental documentation may include:

- Relevant clinical notes for your patient
- Recent test results
- Supporting scientific publications/journal articles
- A summary of your recommendation at the end of the letter
- A letter of medical necessity

Make sure you match the exact language from the denial letter.

It is imperative to address the specifics of the denial in the appeal letter.

Before you submit your appeal, make sure to:

- Check for any incomplete or missing information, as this is a common reason for denial
- Schedule a peer-to-peer meeting with the health plan
- Contact a Case Manager to learn about additional resources and next steps in the process

If a letter of medical necessity was not submitted with the PA, consider including it with the appeal letter.

Contact the health plan to learn about the appeal review timeline. Once you have submitted the letter, along with any supporting documentation, most health plans will review and decide on coverage within approximately:

72 HOURS
for urgent care

30 DAYS
for nonurgent care

60 DAYS
for services already provided

To initiate an expedited appeal, contact your patient's health plan to confirm its instructions for expedited requests.

DOWNLOAD the Appeal Letter Template to help your office draft an appeal letter.

DOWNLOAD the Payer Appeal Letter Checklist and Tips to help you through the appeal process.

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PA, prior authorization.

Please see Important Safety Information on pages 12-13 and see Full Prescribing Information.

Best Practices to Maintain Throughout the Access Journey

DOCUMENT
Keep a record of policy requirements, which may vary considerably among different health plans.

IDENTIFY
Ensure smooth transactions among the provider, health plan, and patient by identifying each health plan's policy early on.

KNOW
Health plan policies provide clarity for patients on their coverage and OOP expenses.

CONTACT
Our Case Managers are ready to assist you with your questions.

AMGEN BY your side

Our dedicated team is your patient's partner, committed to providing nonmedical support to help patients as they start and continue on treatment as prescribed.

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OOP, out-of-pocket.

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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.

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Please see additional Important Safety Information on page 13 and Full Prescribing Information.

IMPORTANT SAFETY INFORMATION (cont'd)

WARNINGS AND PRECAUTIONS (cont'd)

- **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.

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