

PROCYSBI Oral Granules in Packets: Frequently Asked Questions

This document is intended as a guide for questions that may arise about the U.S. FDA approval of PROCYSBI (cysteamine bitartrate) delayed-release oral granules in packets.

PROCYSBI is a prescription medicine used to treat nephropathic cystinosis in adults and children 1 year of age and older. It is not known if PROCYSBI is safe and effective in children under 1 year of age. Please see Important Safety Information on pages 2 and 3 and click here for [Patient Package Insert](#).

Note: If you choose to share or post these FAQs, please share the full document and not select pieces of it, in order to comply with regulatory guidelines.

Q1: Are the oral granules in packets a new or different type of PROCYSBI?

No. The same granules, also called microbeads, inside PROCYSBI capsules are in the tear-open packets. PROCYSBI oral granules in packets is not a new medicine, it is a new dosage form (a new means of packaging).

Q2: How should I switch from PROCYSBI capsules to oral granules in tear-open packets?

If you and your cystinosis healthcare team decide that the oral granules in tear-open packets are the right option, they can guide you through the switch.

Q3: What strengths are the oral granules in tear-open packets available in?

PROCYSBI oral granules in tear-open packets are available in 75 mg and 300 mg dosage strength options. The strength options are based on input from families and physicians in the cystinosis community and extensive research to best address the needs.

Q4: How do I adjust my dosage for PROCYSBI capsules to accommodate the new strengths of the oral granules in tear-open packets?

We recommend talking with your physician about how to make the appropriate adjustments for the strengths if needed. If you and your cystinosis healthcare team decide that oral granules in tear-open packets are the right option, they can guide you through the switch.

Q5: Will Horizon continue to have the 25mg and 75 mg PROCYSBI capsules available?

Yes, both the 25mg and 75mg PROCYSBI capsules will continue to be available. Both the capsules and the tear-open packets contain the same PROCYSBI granules, also called microbeads. Having PROCYSBI capsules and oral granules in tear-open packets will allow patients to select which option is best for them.

Q6: Are the oral granules in tear-open packets the same price as the capsules?

Yes, the price for both PROCYSBI capsules and the oral granules in tear-open packets is based on the dosage prescribed. The oral granules in packets is simply a new means of packaging.

Horizon remains committed to ensuring patients prescribed our medicines, including PROCYSBI oral granules in tear-open packets, have access. We have support and assistance in place so that eligible patients are able to receive our medicines regardless of their ability to pay.

Q7: Is it possible to take the oral granules in tear-open packets with food?

Yes, the same steps required when taking the contents of the PROCYSBI capsule with food are required when taking the oral granules in tear-open packets. The packets must be opened and mixed with select high acidity foods or liquids identified in the full prescribing information. Mix the packets with no more than 1/2 cup (4 oz) of certain foods. Avoid high-fat foods (such as avocados, cheese, and nuts) right before and after you take PROCYSBI. Please see [Instructions for Use](#) for additional information.

Q8: Are the oral granules in packets approved/available outside of the United States?

No, PROCYSBI oral granules in tear-open packets was approved by the FDA and this approval only applies to the United States.

Q9: How do you open the PROCYSBI oral granules in packets?

Each packet has a dotted (perforated) line where they should be torn open. Please remember that oral granules should **not** be stored in open packets. Please see Instructions for Use (<https://www.hzndocs.com/PROCYSBI-Instructions-for-Use.pdf>) for additional information.

Q10: Do the packets provide protection for the granules/microbeads?

The packets provide the needed protection of the granules/microbeads. Horizon maintains a rigorous testing and evaluation process to ensure the consistency of our medicines.

Horizon will continue to participate, listen and learn from those living with or caring for someone with cystinosis to understand the needs and invest in resources and development to address them as appropriate. If there are additional needs from the community regarding our cystinosis medication, please feel free to contact us at any time: connect@horizontherapeutics.com.

USE and IMPORTANT SAFETY INFORMATION

What is PROCYSBI?

PROCYSBI (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules is a prescription medicine used to treat a medical condition called nephropathic cystinosis in adults and children 1 year of age and older. It is not known if PROCYSBI is safe and effective in children under 1 year of age.

IMPORTANT SAFETY INFORMATION

What is the most important safety information I should know about PROCYSBI?

PROCYSBI can cause serious side effects, including:

- **Skin, bone, and joint problems.** People treated with high doses of cysteamine bitartrate may develop abnormal changes of their skin and bones, such as stretch marks, bone injuries (such as fractures), bone deformities, and joint problems. Check your skin while taking PROCYSBI. Tell your doctor if you notice any skin changes or problems with your bones or joints. Your doctor will check you for these problems.
- **Skin rash.** Skin rash is common with cysteamine bitartrate and may sometimes be severe. Your dose of PROCYSBI may need to be decreased until the rash goes away. If the rash is severe, your doctor may tell you to stop taking PROCYSBI. **Tell your doctor right away if you get a skin rash.**

- **Stomach and bowel (intestinal) problems.** Some people who take other medicines that contain cysteamine bitartrate develop ulcers and bleeding in their stomach or bowel. **Tell your doctor right away** if you get stomach-area pain, nausea, vomiting, loss of appetite, or vomit blood.
- **Central nervous system symptoms.** Some people who take other medicines that contain cysteamine bitartrate develop seizures, depression, and become very sleepy. The medicine may affect how your brain is working (encephalopathy). Tell your doctor right away if you develop any of these symptoms.
- **Low white blood cell count and certain abnormal liver function blood tests.** Your doctor should check you for these problems.
- **Benign intracranial hypertension** (pseudotumor cerebri) has happened in some people who take immediate-release cysteamine bitartrate. This is a condition where there is high pressure in the fluid around the brain. Your doctor should do eye examinations to find and treat this problem early. **Tell your doctor right away if you develop any of the following symptoms while taking PROCYSBI:** headache, buzzing or "whooshing" sound in the ear, dizziness, nausea, double vision, blurry vision, loss of vision, pain behind the eye, or pain with eye movement.

Who should not take PROCYSBI?

Do not take PROCYSBI if you are allergic to penicillamine or cysteamine.

What should I tell my doctor before taking PROCYSBI?

Tell your doctor if you have any other medical conditions, including if you:

- drink alcohol.
- have a skin rash or bone problems.
- have or have had stomach or bowel (intestinal) problems including ulcers or bleeding.
- have a history of seizures, lack of energy, unusual sleepiness, depression, or changes in your ability to think clearly.
- have liver or blood problems.
- are pregnant or plan to become pregnant. It is not known if PROCYSBI will harm your unborn baby. Tell your doctor right away if you think that you are pregnant. Talk with your doctor about the benefits and risks of taking PROCYSBI during pregnancy.
- are breastfeeding or plan to breastfeed. You should not breastfeed during treatment with PROCYSBI. Talk with your doctor about the best way to feed your baby if you take PROCYSBI.

What should I avoid while taking PROCYSBI?

- Do not drive or operate machinery until you know how PROCYSBI affects you. PROCYSBI can make you sleepy or less alert than normal.
- Do not drink alcohol if you take PROCYSBI. Drinking alcohol while taking PROCYSBI may change how PROCYSBI works and may cause an increase in the amount of PROCYSBI in your blood that may cause serious side effects.

What are the possible side effects of PROCYSBI?

- See **"What is the most important information I should know about PROCYSBI?"**

The most common side effects of PROCYSBI include: vomiting, nausea, stomach (abdominal) pain, pink eye, diarrhea, cold, tiredness, flu, headache, problems with body salts or electrolytes, infection of ear, nose or throat, joint pain.

These are not all of the possible side effects of PROCYSBI. Call your doctor for medical information about side effects.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit <http://www.fda.gov/medwatch> or call 1-800-FDA-1088.

For additional important safety information, click here for the [Patient Package Insert](#) and discuss with your doctor.