

# Taking Control Of Cystinosis



## THE STEPS YOU TAKE TODAY MATTER TOMORROW

### **What is PROCYSBI?**

PROCYSBI (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules 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.

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

**Please see complete IMPORTANT SAFETY INFORMATION on pages 7 and 8 and click here for the Patient Package Insert.**

**PROCYSBI**<sup>®</sup>  
(cysteamine bitartrate)  
delayed-release capsules  
delayed-release oral granules

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This guide is meant to go along with the information you receive from your health care team. If after consulting this guide you still have questions about PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules or how to take it, contact your doctor, pharmacist, or another health care team member.

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 7 and 8 and [click here for the Patient Package Insert](#).



# CYSTINE DEPLETING THERAPY (CDT) IS CRITICAL

Cystinosis is a lifelong condition, but treatments such as CDT and kidney transplantation have allowed people with cystinosis to live longer. Your doctor will likely start you on a lower dose and increase it over time to help you adjust to treatment. If you're currently taking a CDT, be sure your doctor knows exactly how much you've been prescribed and how much of that you've been taking.

Please see complete [IMPORTANT SAFETY INFORMATION](#) on pages 7 and 8 and [click here for the Patient Package Insert](#).



In a study, cystine levels were  
**65% HIGHER IN PATIENTS WHO  
DELAYED TAKING THEIR NEXT  
DOSE OF CDT BY 3 HOURS**  
compared to those who took their CDT on time

### **CONSISTENT CDT IS THE BEST WAY TO CONTROL CYSTINOSIS**

Taking your medicine on time, every time, even after transplant, is easier said than done. But it's so important for your health. Even brief interruptions in dosing allow a rapid return to toxic levels of cystine. You should be taking all your doses exactly as prescribed. Consistent, continuous control of cystine levels can help limit the damage the disease causes to your body.

#### **USE AND 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.

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 7 and 8 and click here for the **Patient Package Insert**.



# PROCYSBI OFFERS TWICE-DAILY DOSING FOR ALL-DAY LIVING

Taking less than prescribed, or taking what you've been prescribed less often, can have serious long-term consequences. A twice-daily schedule might help.

PROCYSBI is the first and only CDT with 12-hour dosing. It helps reduce and maintain cystine levels with 2 doses in 24 hours, taken every 12 hours at the same time, every day.

## CONSISTENCY IS KEY TO PUTTING YOU IN CHARGE OF YOUR DAY, AND NIGHT.

Since PROCYSBI is different from other CDTs in the way the medicine releases inside the body, your doctor may prescribe a lower starting dose and then adjust the dose. Work with your doctor to get the right dose for you.

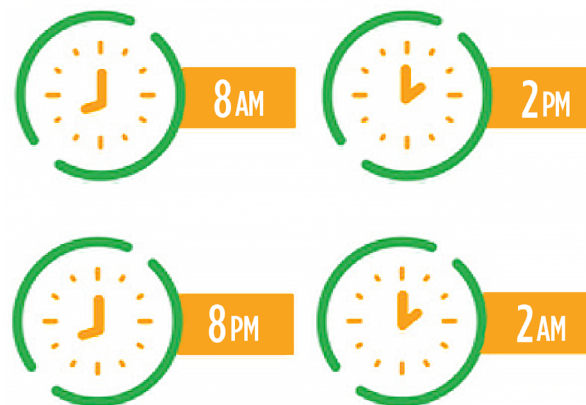
### 2 daily doses, 1 every 12 hours

PROCYSBI  
delayed-release  
capsules and  
delayed-release  
oral granules



### 4 daily doses, 1 every 6 hours

CYSTAGON®  
(cysteamine  
bitartrate)  
Capsules



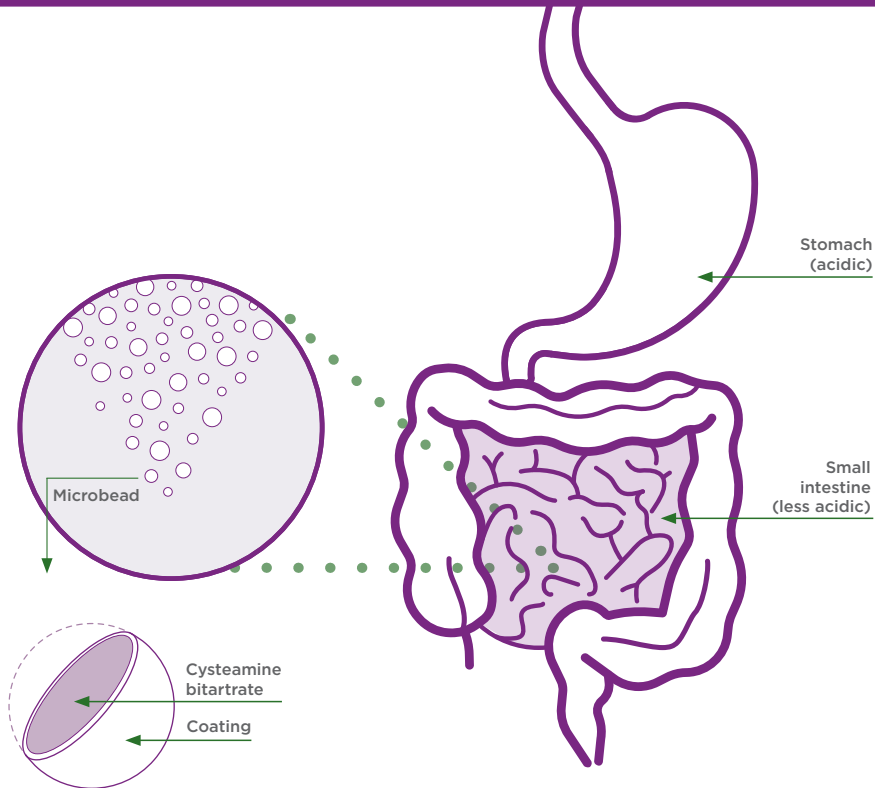
## USE AND IMPORTANT SAFETY INFORMATION

- **Skin rash.** Skin rash is common with cysteamine bitartrate and may sometimes be severe. **Tell your doctor right away if you get a skin rash.** 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.

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# PROCYSBI MICROBEADS MAKE 12-HOUR DOSING POSSIBLE

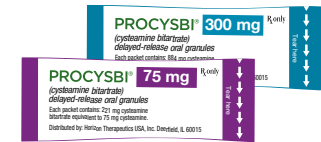


Each microbead granule has a protective coating that surrounds the medicine inside. The protective coating allows it to pass through the acidic environment of the stomach into the less acidic small intestine, where it can begin dissolving slowly to release cysteamine over 12 hours.

## PROCYSBI is available in capsules or packets



Capsules for swallowing whole or mixing with select foods and liquids



Packets for use with G-tube or mixing with select foods and liquids

## USE AND IMPORTANT SAFETY INFORMATION

- **Stomach and bowel (intestinal) problems.** Some people who take other medicines that contain cysteamine bitartrate may develop ulcers and bleeding in their stomach or bowel. People treated with PROCYSBI may also develop abnormal swelling and narrowing of the large bowel which must be treated promptly. **Tell your doctor right away** if you get abdominal pain, bloody or persistent diarrhea, bloating, nausea, vomiting, loss of appetite, vomit blood, poor weight gain or weight loss.

Please see complete [IMPORTANT SAFETY INFORMATION](#) on pages 7 and 8 and click here for the [Patient Package Insert](#).



# AN ACIDIC ENVIRONMENT IN THE STOMACH IS VERY IMPORTANT

If your stomach is not as acidic as it should be, the medicine contained inside the microbeads can be released too soon and could make you feel nauseated. It's important to create an acidic environment in your stomach and take PROCYSBI exactly as directed. If you have any issues once you start taking PROCYSBI, talk to your doctor.

## YES

- Take capsules with water or fruit juice (except grapefruit juice)
- Mix granules with foods like applesauce or berry jelly, which are acidic

## NO

- Alcohol
- High-fat foods too close to the time you take a dose
- Antacids containing bicarbonate or carbonate, 1 hour before or after you take a dose



## USE AND IMPORTANT SAFETY INFORMATION

- **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.

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# HOW TO MAKE THE MOST OF PROCYSBI TREATMENT



Success starts with open and honest dialogue between you and your doctor, so you get the correct dose of PROCYSBI.

- Are you currently taking CDT?
- Are you taking all of it, at the right time, every time?
- Are there things you struggle with or find difficult?

Your doctor can help. Once you start with PROCYSBI, take it the same way each time, as prescribed, once every 12 hours. Regular cystine level testing will help ensure you have the correct dose.

If you have any questions about how to take PROCYSBI, talk to your doctor and see the Full Important Safety Information on pages 7 and 8 and click here for the [Patient Package Insert](#).

## A CHRONIC DISEASE LIKE CYSTINOSIS REQUIRES CONTINUOUS TREATMENT

Continuous treatment of cystinosis is critical for long-term health. Partner with your doctor to find a plan that works for you. The damage caused by toxic levels of cystine cannot be undone, so it's important that you take your medicine on time, every time. You can do it. Establishing a consistent routine is key. Twice-daily dosing may help. Be sure to ask your doctor about the support available to you when needed.

To learn more, visit [PROCYSBI.com](http://PROCYSBI.com)

Please see complete [IMPORTANT SAFETY INFORMATION](#) on pages 7 and 8 and click here for the [Patient Package Insert](#).



# USE AND IMPORTANT SAFETY INFORMATION

## 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. **Tell your doctor right away if you get a skin rash.** 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.
- **Stomach and bowel (intestinal) problems.** Some people who take other medicines that contain cysteamine bitartrate may develop ulcers and bleeding in their stomach or bowel. People treated with PROCYSBI may also develop abnormal swelling and narrowing of the large bowel which must be treated promptly. **Tell your doctor right away** if you get abdominal pain, bloody or persistent diarrhea, bloating, nausea, vomiting, loss of appetite, vomit blood, poor weight gain or weight loss.
- **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.

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

# IMPORTANT SAFETY INFORMATION (CONTINUED)

## **Before taking PROCYSBI, tell your doctor about all your 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.

**Tell your doctor about all the medicines you take**, including prescription and over the counter medicines, vitamins, dietary and herbal supplements. Know the medicines you take. Keep a list of them to show your doctor and pharmacist when you get a new medicine.

## **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?**

### **PROCYSBI can cause serious side effects, including:**

- 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 the possible side effects of PROCYSBI. Call your doctor for medical information about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**For additional IMPORTANT SAFETY INFORMATION, click here for the [Patient Package Insert](#) and discuss with your doctor.**



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