

Why PROCYSBI?

**BECAUSE LIFE IS WHAT HAPPENS IN BETWEEN**

**8:00 AM**

**8:00 PM**

**You are more than your meds.**  
PROCYSBI works to control cystine levels continuously over 12 hours.  
Take PROCYSBI every 12 hours, at times that work for you.

#### 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.

#### Who should not take PROCYSBI?

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

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and click here for the Patient Package Insert.**



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

*“Eden’s biggest passion is art. She loves painting and drawing. And she just got a camera, so she’s really excited about trying photography next.”*

—Nicolai, Eden’s father

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 18 and 19, and [click here](#) for the Patient Package Insert.



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# WHAT IS CYSTINOSIS?

Cystinosis is a rare, genetic condition that affects about 500 to 600 people in the United States and an estimated 2,000 worldwide.

## In people with cystinosis:

- An amino acid called **cystine** builds up in the body's cells because the cells are unable to remove it
- When cystine builds up, it forms crystals within cells
- Crystals eventually lead to damage to the organs—including the kidneys, eyes, liver, muscles, pancreas, and brain
- This damage cannot be reversed, but it may be delayed or prevented

## Nephropathic cystinosis

"Nephropathic" means damaging to the kidneys. Nephropathic cystinosis causes severe damage to the kidneys and other organs. It is the most common and severe form of cystinosis, making up 95% of all cases, and symptoms usually appear within the first year of life.

Nephropathic cystinosis generally affects boys more than girls. People of all races and ethnic backgrounds can be affected. However, it is most common in individuals who are of European descent.

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# WHAT IS FANCONI SYNDROME?

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Over time, nephropathic cystinosis causes damage to the kidneys. **This damage can lead to Fanconi syndrome, a progressive disorder that prevents the kidneys from absorbing nutrients and filtering out the body's waste.**

In people with Fanconi syndrome, nutrients that would normally be absorbed pass through the kidneys and leave the body in pee (urine). Fanconi syndrome is managed with fluids, electrolytes, and vitamin supplements, but damage worsens over time.

## Symptoms of Fanconi syndrome include:

- Excessive thirst and peeing (urination)
- Reduced appetite
- Weight loss
- Slow growth
- Softening or weakening of bones (rickets)

Symptoms of Fanconi syndrome can emerge in children as young as 6 months old. **In many cases, it's the presence of Fanconi syndrome that causes doctors to suspect and diagnose nephropathic cystinosis.**

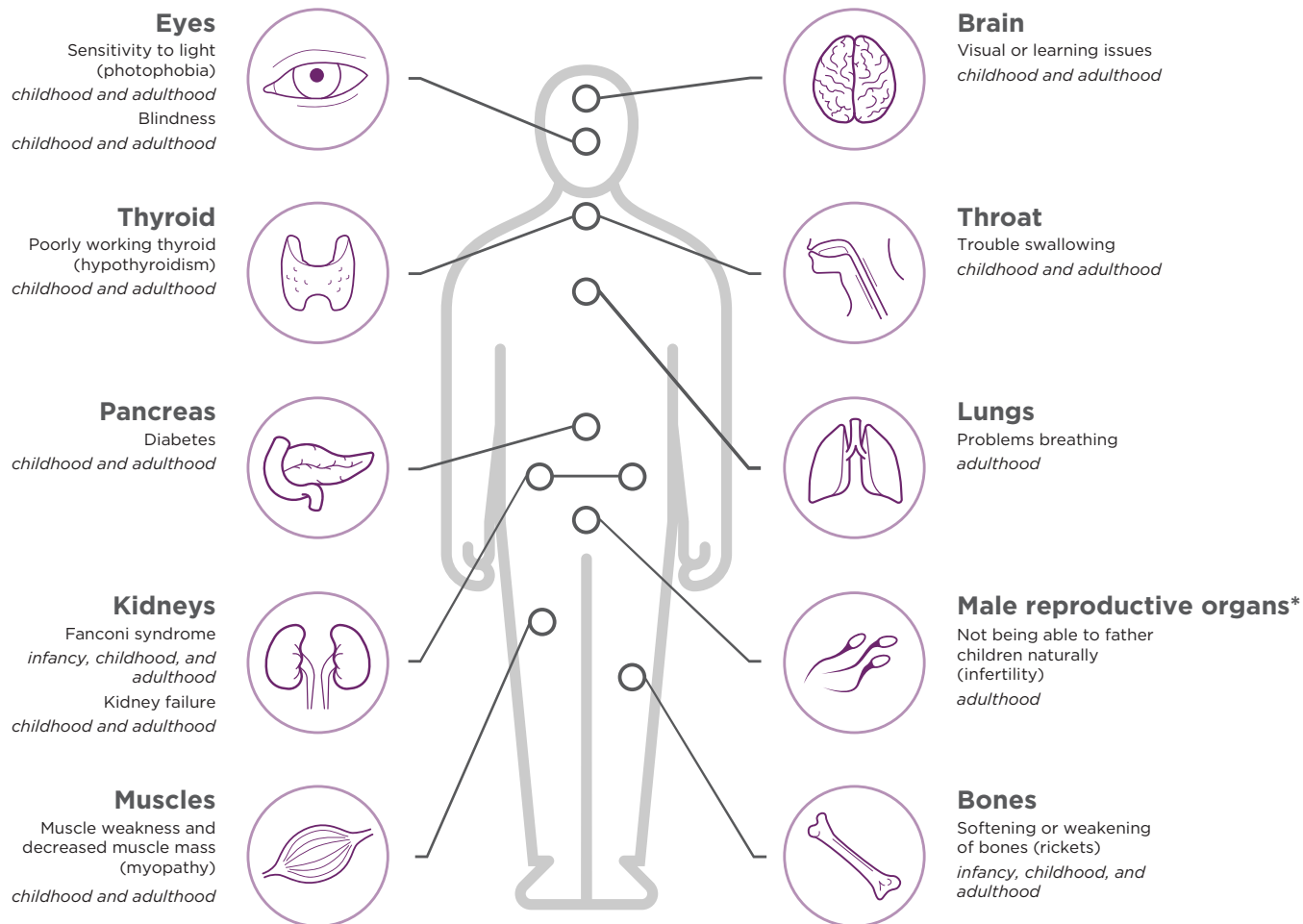
Eventually, the damage caused by cystinosis causes the kidneys to fail completely, which can be treated only with dialysis (an artificial means of getting rid of the body's waste) and, ultimately, a kidney transplant.

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and [click here for the Patient Package Insert.](#)**



# HOW CYSTINOSIS AFFECTS THE BODY

- Cystinosis can affect nearly every organ of the body.
- Treatment can prevent or slow some effects, but
- existing damage cannot be reversed. (See page 7
- for more information about treatment.)



\*Women of childbearing age with cystinosis may become pregnant. If your loved one is pregnant, planning on becoming pregnant, or breastfeeding, talk with your doctor about appropriate treatments.

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 18 and 19, and [click here for the Patient Package Insert](#).

# HOW CYSTINOSIS IS MANAGED

A key part of cystinosis management is keeping cystine levels low. There are medicines called cystine-depleting therapies, or CDTs, that can help.

CDTs lower cystine levels. When taken as prescribed, they help remove cystine from the cells. That is why it's important that your loved one take a CDT on time and as prescribed, even if he or she looks or feels fine.

**One medical study showed that people who took their CDT on time had significantly lower average cystine levels compared with people who took their medicine late (at 9 hours vs 6 hours).**

The following CDTs are approved by the US Food and Drug Administration (FDA):

- 2 CDTs, taken by mouth, remove cystine from the body
- 1 CDT, in eyedrop form, removes cystine from the eyes
  - CDTs taken by mouth do not remove cystine from the eyes. Eyedrops are the only way to remove cystine from the eyes



**Treats the body**



**Treats the eyes**

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and [click here for the Patient Package Insert](#).**

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





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# WHAT IS PROCYSBI?

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**PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules is the first and only CDT with 12-hour dosing.**

**This means PROCYSBI is taken every 12 hours (2 times in 24 hours).** For example, your loved one might take PROCYSBI at 7:00 AM and again at 7:00 PM every day.

## Clinical trials with PROCYSBI

In clinical trials, people with nephropathic cystinosis who took PROCYSBI every 12 hours as directed saw the following results:

- **PROCYSBI controlled cystine levels** for a full 12 hours
- **PROCYSBI maintained kidney function** during 24 months or more of treatment
- The **safety and effectiveness of PROCYSBI were established** in children 1 year of age and older; cystine levels were maintained through the 18 months of the trial
- **PROCYSBI improved weight and height** in children older than 1 year and younger than 6 years (comparisons were made based on the change in weight and height from day 1 to 12 months and 18 months of the trial)

## 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.

## Who should not take PROCYSBI?

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

## 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.

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and click here for the Patient Package Insert.**



# HOW PROCYSBI IS DESIGNED TO WORK

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A protective coating helps PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules provide 12-hour continuous cystine control.

PROCYSBI granules, also called “microbeads,” are made of medicine called cysteamine bitartrate surrounded by this protective coating.

**The protective coating on PROCYSBI microbeads helps to control where and how the medicine is released.**

For PROCYSBI to work properly, the microbeads must release medicine *only* in the small intestine. The coated microbeads allow the medicine to pass through the stomach and into the small intestine without dissolving. Once in the small intestine, the coating begins to dissolve and the microbeads release medicine gradually, which allows PROCYSBI to control cystine continuously over 12 hours.

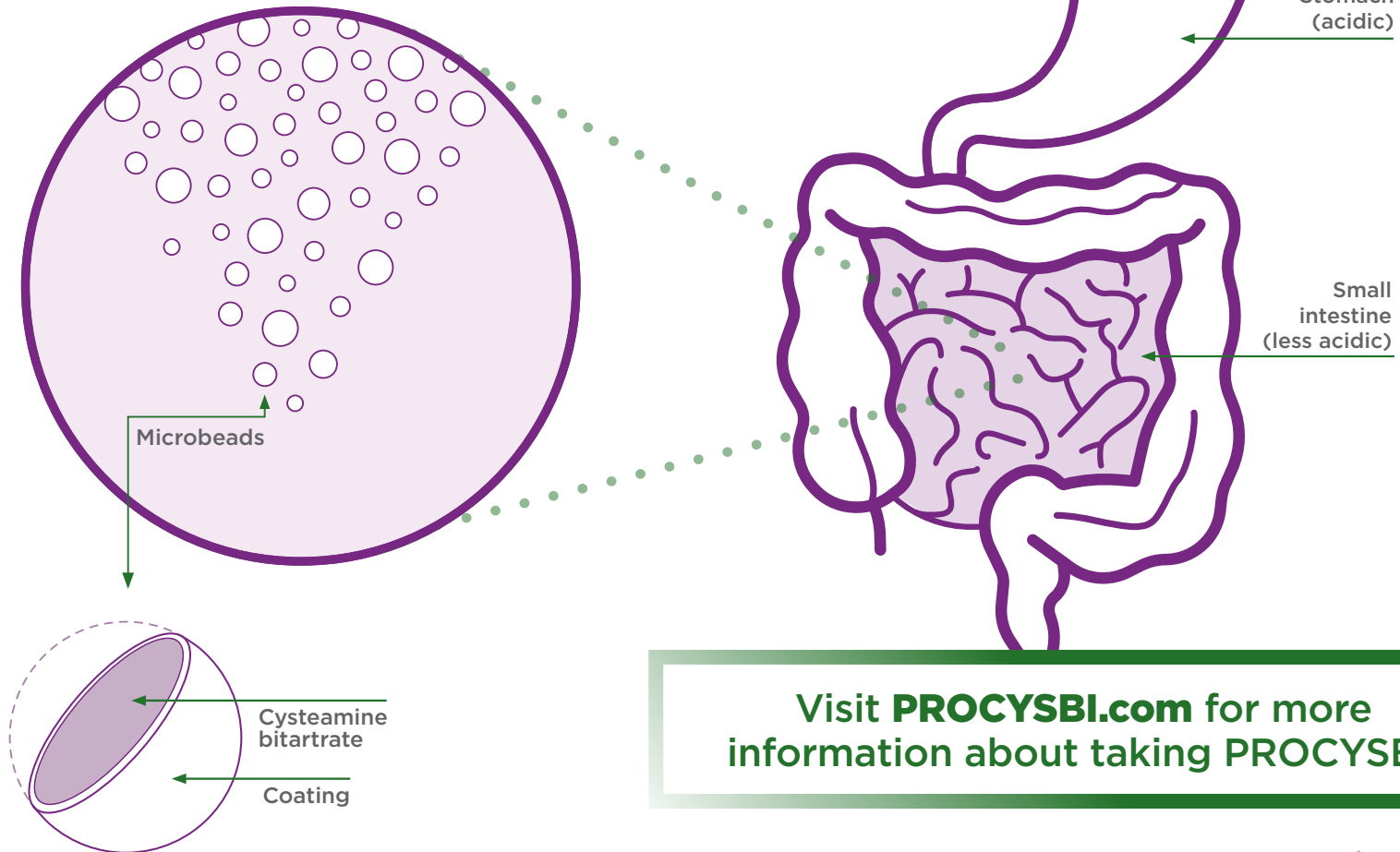
## What should I avoid while taking PROCYSBI?

- 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

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and [click here for the Patient Package Insert](#).**



PROCYSBI microbeads move through the stomach to the small intestine.



Visit **PROCYSBI.com** for more information about taking PROCYSBI.

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 18 and 19, and click here for the Patient Package Insert.

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

# TWO OPTIONS FOR PROCYSBI

PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules is available in capsules *and* in tear-open packets. **Both contain the same PROCYSBI microbeads that provide 12 hours of continuous cystine control.**

## Capsules



Capsules may be swallowed whole with select liquids, or opened and the microbeads mixed with select foods or liquids.

**PROCYSBI in capsules may be a good option for people who prefer to swallow whole capsules.**

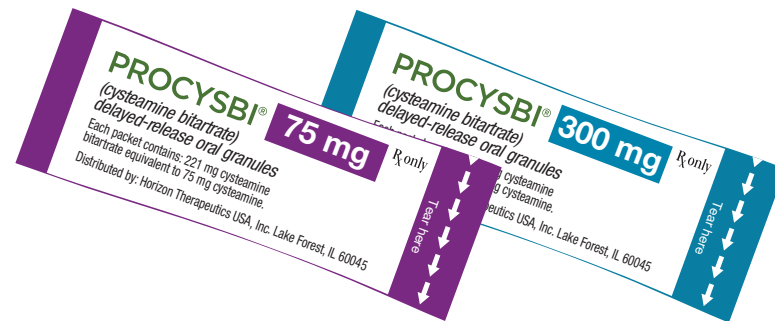
### Who should not take PROCYSBI?

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

**Talk to your doctor about which option is best for your loved one: capsules or packets. With PROCYSBI, there's a choice!**

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and click here for the Patient Package Insert.**

## Packets



Packets should be opened and the microbeads mixed with select foods or liquids.

**PROCYSBI in packets may be a good option for people who:**

- Have trouble swallowing
- Take PROCYSBI via a gastrostomy tube (G-tube)
- Are taking the time to open individual capsules at every dose





# HOW TO GET THE MOST OUT OF TREATMENT WITH PROCYSBI

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Your loved one doesn't need to be defined by nephropathic cystinosis. Cystinosis can be treated with PROCYSBI—the first and only CDT with 12-hour dosing.

## Reminders about taking PROCYSBI

- **Take PROCYSBI every 12 hours (2 times every 24 hours)** at the same time every day
- Work with your loved one's healthcare team to **schedule regular cystine level testing**
- **Take only as directed**, since food and drinks can affect how the medicine in PROCYSBI is released
- **Do not eat for at least 2 hours before** taking PROCYSBI and **at least 30 minutes after** taking it
  - If your loved one can't take PROCYSBI without eating, it may be taken with **no more than 1/2 cup (4 oz) of food** up to 1 hour before or after taking PROCYSBI
- **Avoid high-fat foods** (such as avocados, cheese, and nuts) right before and after dosing
- **Take PROCYSBI the same way each time**

If you have questions about how to take PROCYSBI, contact your loved one's doctor or pharmacist, or review the accompanying Patient Package Insert located in the pocket of this brochure.

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and [click here for the Patient Package Insert](#).**

## Tell your doctor and pharmacist:

- About all the medicines your loved one is taking, including:
  - Medicines prescribed by a doctor
  - Medicines available over the counter
  - Vitamins and herbal supplements
  - Medicines that contain ingredients with “bicarbonate” or “carbonate” in their name, which decrease the acid in the stomach
- Any time your loved one begins taking a new medicine, as some medicines may cause the active ingredient in PROCYSBI to release before it reaches the small intestine. If this happens, PROCYSBI may act differently in the body than intended

**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.



# HOW TO KNOW IF TREATMENT IS WORKING

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Your loved one's doctor will order white blood cell (WBC) cystine level tests to measure **the amount of cystine** in your loved one's white blood cells.

## WBC cystine level tests help the doctor determine:

- The dose of CDT that is right for your loved one
- How well the medicine is working to manage your loved one's cystine levels
- When and how much the dose should be adjusted, including in growing children

Having cystine levels tested 1 to 2 times a year for adults and 4 times a year for children is a critical part of a cystinosis management plan.

*"I stay adherent to my medicine because I want to stay healthy and just live my life. It's worth it to be adherent. It's worth it to be healthy."*

—Adult with cystinosis

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 18 and 19, and [click here for the Patient Package Insert](#).



# TYPES OF WBC CYSTINE LEVEL TESTS

There are 2 types of WBC cystine level tests that measure different types of white blood cells: granulocytes and mixed leukocytes. Because the tests measure different types of white blood cells, **they have different target cystine levels.**

TYPE OF TEST	TESTING INSTITUTION	TEST-SPECIFIC TARGET CYSTINE LEVEL
Granulocytes	University of California San Diego	Less than <b>1.9</b> nmol ½ cystine/mg protein
Mixed leukocytes	Baylor Genetics	Less than <b>1.0</b> nmol ½ cystine/mg protein

It’s important to **have the same test every time** so your loved one’s doctor can compare the results over time.

Cystine levels should be tested just before the next dose of CDT, because that’s when cystine will approach its highest levels. Talk to your loved one’s doctor about the best day and time to schedule a WBC cystine level test.

Please see complete **IMPORTANT SAFETY INFORMATION** on pages 18 and 19, and [click here for the Patient Package Insert.](#)







*“We chose PROCYSBI for Eden just because of our daily routine. Now she only takes her CDT twice a day, every 12 hours, which allows her more time to do the things she wants to do.”*

*—Nicolai, Eden’s father*

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# SUPPORT FOR YOU AND YOUR FAMILY



Horizon By Your Side is a patient support program dedicated to improving the lives of people with cystinosis by providing ongoing individualized support and education. Your Patient Access Liaison (PAL) provides you with dedicated, one-on-one support. They work directly with you to answer non-medical, logistical questions and provide support once you have been prescribed PROCYSBI and you have completed enrollment.

These comprehensive services are free of charge and built around 3 components: Connect, Coordinate, and Champion.

## Connect

- Connect you to others living with rare diseases via live events and online resources
- Provide tools and resources to help you manage day-to-day challenges
- Introduce you to advocacy groups to provide more support and inform you of events in your area

## Coordinate

- Help you understand your coverage and provide information about financial assistance programs
- Assist you in connecting with your specialty pharmacy

## Champion

- Serve as a dedicated personal resource and the main point of contact for your ongoing non-medical logistical needs
- Provide you with education and answer your non-clinical questions
- Help you through changes that may impact your loved one's treatment

To learn more about the resources and tools provided by Horizon By Your Side:

- **Visit the Savings and Support page on PROCYSBI.com**
- **Call 1-855-888-4004 to speak with a PAL**
- **Email [HPSPRO@horizontherapeutics.com](mailto:HPSPRO@horizontherapeutics.com)**

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# USE AND IMPORTANT SAFETY INFORMATION

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## What is PROCYSBI?

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## 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.



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# IMPORTANT SAFETY INFORMATION (CONTINUED)

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## 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 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.**



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## CYSTINOSIS RESOURCES AND SUPPORT

### CONNECT WITH THE CYSTINOSIS COMMUNITY

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Wherever you and your loved one are in the cystinosis journey, we want you to know that there are others in the cystinosis community who can help you feel less alone. The resources below, which are unaffiliated with Horizon, can help you connect with advocacy groups, support programs, and online communities.

#### Cystinosis Research Foundation (CRF)

**CystinosisResearch.org**

**949-223-7610**

CRF supports research that aims to improve the quality of life of patients with cystinosis, find better treatments, and ultimately find a cure.

#### Cystinosis Research Network (CRN)

**Cystinosis.org**

**866-276-3669**

CRN is an all-volunteer, nonprofit organization dedicated to supporting and advocating for research, providing family assistance, and educating the public and medical communities about cystinosis.

#### Global Genes

**GlobalGenes.org**

**949-248-RARE (7273)**

Global Genes is a nonprofit advocacy organization for patients and families fighting rare and genetic diseases, including cystinosis. Its mission is to eliminate the challenges of rare disease.

#### The National Organization for Rare Disorders (NORD)

**RareDiseases.org**

**203-744-0100**

NORD is a patient advocacy organization dedicated to people with rare diseases and the groups that help them. NORD provides patients and families with advocacy information, assistance programs, and connections to patient organizations.

*These websites are run by independent third parties, and Horizon is not responsible for their content.*

**Please see complete IMPORTANT SAFETY INFORMATION on pages 18 and 19, and click here for the Patient Package Insert.**





## DOCTOR DISCUSSION GUIDE

### PREPARE FOR A CONVERSATION WITH YOUR LOVED ONE'S DOCTOR

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If you're interested in learning more about PROCYSBI® (cysteamine bitartrate) delayed-release capsules and delayed-release oral granules, you may want to schedule an appointment with your loved one's doctor. Bring this discussion guide with you to help make the most of your conversation about PROCYSBI.

#### USE and IMPORTANT SAFETY INFORMATION

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- **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.**

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**Information about your loved one's nephropathic cystinosis:**

**Diagnosis year:** \_\_\_\_\_

**Current cystine-depleting therapies (CDTs), including dose:**

\_\_\_\_\_

**Does your loved one:**

☐ have trouble swallowing    ☐ take medicine via a gastrostomy tube (G-tube)

**If you checked yes to one or both, ask whether PROCYSBI packets might be the right choice for your loved one.**

**Kidney transplant:** yes | no    **At what age?** \_\_\_\_\_

**If your loved one takes a CDT, about how many doses are generally missed, skipped, or taken late each day? (It's okay to be honest with the doctor; talking can help):** 1 dose | 2 or more doses | none

**Reason(s) doses are missed, skipped, or taken late (if applicable):**

\_\_\_\_\_

**Date of your loved one's last cystine level test:**

\_\_\_\_\_

**Which type of cystine level test did your loved one have? (Circle one.)**

Mixed leukocytes (target: less than 1.0 nmol ½ cystine/mg protein)

Granulocytes (target: less than 1.9 nmol ½ cystine/mg protein)

**What was your loved one's cystine level when last tested?**

\_\_\_\_\_





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