



## **RATIONALE FOR INCLUSION IN PA PROGRAM**

### **Background**

Procysbi, is designed to treat nephropathic cystinosis, the most common form of a disease known as cystinosis, in which toxic levels of cystine, a naturally occurring amino acid, build up in the body's cells and organs. Cystinosis may lead to slow body growth and small stature, weak bones and developing and worsening kidney failure. There are three types of cystinosis, the most severe being nephropathic cystinosis, which severely damages the kidneys. The drug works by lowering cystine levels, potentially delaying kidney and other damage (1).

### **Regulatory Status**

FDA-approved indication: Procysbi is a cysteine-depleting agent indicated for the management of nephropathic cystinosis in adults and pediatric patients 1 year of age and older (1).

Procysbi should be prescribed by a physician experienced in management of nephropathic cystinosis. Goal of therapy is to maintain a white blood cell (WBC) cystine level  $< 1 \text{ nmol } \frac{1}{2} \text{ cystine/mg protein}$  or a plasma cysteamine concentration  $> 0.1 \text{ mg/L}$  (1).

Patients should have their WBC cystine levels and/or plasma cysteamine concentration measured in 2 weeks, and quarterly for 6 months then twice yearly at a minimum. If the plasma cysteamine is  $> 0.1 \text{ mg/L}$ , but the WBC cystine is  $> 1.0 \text{ nmol } \frac{1}{2} \text{ cystine/mg protein}$ , the physician is advised to investigate the following parameters: adherence to dosing interval, adherence to medication, or the relationship between administration of Procysbi and fasted/fed state (1).

The use of Procysbi delayed-release capsules is contraindicated in patients who are hypersensitive to penicillamine (1).

Interrupt Procysbi if patients develop skin or bone lesions. Cysteamine has been associated with reversible leukopenia and elevated alkaline phosphatase levels. Therefore, blood counts and alkaline phosphatase levels should be monitored (1).

Patients receiving immediate-release cysteamine bitartrate have reported central nervous system symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy. Patients have



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## **PROCYSBI (cysteamine bitartrate)**

also reported gastrointestinal ulceration and bleeding. Monitor patients and an adjustment in dose may be necessary (1).

Safety and efficacy in pediatric patients under the age of 1 year have not been established (1).

### **Summary**

Procysbi is a cystine depleting agent indicated for the management of nephropathic cystinosis in adults and children ages 1 year and older. Procysbi should be prescribed by a physician experienced in management of nephropathic cystinosis. Cystagon is the current standard of care. Procysbi is contraindicated in patients who are hypersensitive to penicillamine. Safety and efficacy in pediatric patients under the age of 1 year have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Procysbi while maintaining optimal therapeutic outcomes.

### **References**

1. Procysbi [package insert]. Lake Forest, IL: Horizon Pharma USA, Inc.; February 2022.