

Reference number
2095-A

## SPECIALTY GUIDELINE MANAGEMENT

### PROCYSBI (cysteamine bitartrate delayed-release)

#### POLICY

##### I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

##### FDA-Approved Indication

Procysbi is indicated for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older.

All other indications are considered experimental/investigational and not medically necessary.

##### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- A. Initial requests: assay detecting increase cystine concentration in leukocytes or genetic testing results supporting diagnosis.
- B. Continuation requests: lab results or chart notes documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for serum creatinine, calculated creatinine clearance, leukocyte cystine concentration, or maintained growth [height]).

##### III. CRITERIA FOR INITIAL APPROVAL

##### **Nephropathic cystinosis**

Authorization of 12 months may be granted for treatment of nephropathic cystinosis when all of the following criteria are met:

- A. Diagnosis of cystinosis was confirmed by the presence of increased cystine concentration in leukocytes or by genetic testing; and
- B. Member is 1 year of age or older; and
- C. Member will not use Procysbi in combination with Cystagon.

##### IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for nephropathic cystinosis who are responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for serum creatinine, calculated creatinine clearance, leukocyte cystine concentration, or maintained growth [height]).

##### V. REFERENCES

1. Procysbi [package insert]. Novato, CA: Raptor Pharmaceuticals Inc.; February 2020.
2. Ivanova E, De Leo MG, De Matteis MA, Levchenko E. Cystinosis: clinical presentation, pathogenesis, and treatment. *Pediatr Endocrinol Rev.* 2014;12(1):176-184.