

Clinical Policy: Cysteamine (Cystagon, Procysbi)

Reference Number: CP.PHAR.155

Effective Date: 02/16

Last Review Date: 02/17

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

The intent of the criteria is to ensure that patients follow selection elements established by Centene® clinical policy for cysteamine bitartrate (Cystagon®, Procysbi®).

Policy/Criteria

It is the policy of health plans affiliated with Centene Corporation® that Cystagon and Procysbi are **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Nephropathic Cystinosis (must meet all):

1. Diagnosis of nephropathic cystinosis confirmed by any of the following:
 - a. Increased leukocyte cystine concentration (normal concentration: <0.2 nmol half-cystine/mg protein);
 - b. Cystinosis, lysosomal cystine transporter gene mutation;
 - c. Corneal crystals on slit lamp examination;
2. Member does not have a known hypersensitivity to penicillamine or cysteamine.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to CP.PHAR.57 - Global Biopharm Policy.

II. Continued Approval

A. Nephropathic Cystinosis (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met all initial approval criteria.
2. Member is responding positively to therapy;
3. Member does not have any of the following reasons to discontinue:
 - a. Known hypersensitivity to penicillamine or cysteamine;
 - b. If Procysbi, either of the following:
 - i. Development of severe skin rash;
 - ii. Diagnosis of benign intracranial hypertension (pseudotumor cerebri).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2):

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy; or
2. Refer to CP.PHAR.57 - Global Biopharm Policy.

Background

Description/Mechanism of Action:

Cystinosis is an autosomal recessive inborn error of metabolism in which the transport of cystine out of lysosomes is abnormal; in the nephropathic form, accumulation of cystine and formation of crystals damage various organs, especially the kidney, leading to renal tubular Fanconi Syndrome and progressive glomerular failure, with end stage renal failure by the end of the first decade of life. In four studies of cystinosis patients before cysteamine was available, renal death (need for transplant or dialysis) occurred at median age of less than 10 years. Patients with cystinosis also experience growth failure, rickets, and photophobia due to cystine deposits in the cornea. With time most organs are damaged, including the retina, muscles and central nervous system. Cysteamine is an aminothiols that participates within lysosomes in a thiol-disulfide interchange reaction converting cystine into cysteine and cysteine-cysteamine mixed disulfide, both of which can exit the lysosome in patients with cystinosis.

Formulations:

- Cystagon (cysteamine bitartrate): Capsules for oral use
 - 50 or 150 mg/capsule
- Procysbi (cysteamine bitartrate): Capsules for oral use
 - 25 or 75 mg/capsule (delayed-release)

FDA Approved Indications:

Cystagon and Procysbi (cysteamine bitartrate) are cystine depleting agents with the following indications:

- Cystagon is an oral capsule formulation indicated for the management of nephropathic cystinosis in children and adults.
- Procysbi is a delayed-release oral capsule formulation indicated for the treatment of nephropathic cystinosis in adult and pediatric patients 2 years of age and older.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date
Policy split from CP.PHAR.48 LSD Policy converted to new template	01/16	02/16
Age restriction removed. Additional diagnostic criteria added.	12/16	02/17

Reviews, Revisions, and Approvals	Date	Approval Date
Reasons to discontinue added to continuation criteria. Positive response to therapy added. Background section converted to new template.		

References

1. Cystagon prescribing information. Morgantown, WV: Mylan Pharmaceuticals, Inc.; June 2007. Available at www.accessdata.fda.gov/drugsatfda_docs/label/2007/020392s010lbl.pdf. Accessed December 15, 2016.
2. Procysbi prescribing information. Novato, CA: Raptor Pharmaceuticals, Inc.; August 2015. Available at <http://www.procysbi.com/wp-content/uploads/2016/05/Prescribing-Info-Mar-2016.pdf>. Accessed December 15, 2016.
3. Kleta R, Kaskel F, Dohil R, et al. First NIH/Office of Rare Diseases conference on cystinosis: past, present, and future. *Pediatr Nephrol.* 2005; 20: 452-454.
4. Bendavid C, Kleta R, Long R, et al. FISH diagnosis of the common 57-kb deletion in CTNS causing cystinosis. *Hum Genet.* November 2004; 115(6): 501-514.

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

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Note: For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

Note: For Medicare members, to ensure consistency with the Medicare National Coverage Determinations (NCD) and Local Coverage Determinations (LCD), all applicable NCDs, LCDs, and Medicare Coverage Articles should be reviewed prior to applying the criteria set forth in this clinical policy. Refer to the CMS website at <http://www.cms.gov> for additional information.

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