Pharmacy Policy Bulletin

Title: Cysteamine-Containing Products

Policy #: Rx.01.136

Application of pharmacy policy is determined by benefits and contracts. Benefits may vary based on product line, group, or contract. Some medications may be subject to precertification, age, quantity, or formulary restrictions (ie limits on non-preferred drugs). Individual member benefits must be verified.

This pharmacy policy document describes the status of pharmaceutical information and/or technology at the time the document was developed. Since that time, new information relating to drug efficacy, interactions, contraindications, dosage, administration routes, safety, or FDA approval may have changed. This Pharmacy Policy will be regularly updated as scientific and medical literature becomes available. This information may include new FDA-approved indications, withdrawals, or other FDA alerts. This type of information is relevant not only when considering whether this policy should be updated, but also when applying it to current requests for coverage.

Members are advised to use participating pharmacies in order to receive the highest level of benefits.

Intent:

The intent of this policy is to communicate the medical necessity criteria for **cysteamine Hydrochloride (Cystaran®/Cystadrops®)** and **cysteamine bitartrate (Cystagon®/ Procysbi®)** as provided under the member's prescription drug benefit.

Description:

Cystinosis is a rare, genetic disorder in which the amino acid cystine accumulates in tissues and organs, most commonly the kidneys and eyes. Treatment with cysteamine should be initiated as soon as the diagnosis is made to preserve kidney function, prevent hypothyroidism, and improve growth in affected children. Orally administered cysteamine does not reach the cornea, thus ophthalmic administration is necessary for accumulation of corneal cystine crystals.

Cysteamine acts as a cystine-depleting agent by converting cystine to cysteine and cysteine-cysteamine mixed disulfides. The result is a reduction in cystine crystals.

Cysteamine hydrochloride (Cystaran®/Cystadrops®) is indicated for the treatment of corneal cystine crystal accumulation in adults and children with cystinosis.

Cysteamine bitartrate (Cystagon®/Procysbi®) is indicated for the treatment of nephropathic cystinosis in adult and children.

Policy:

Cystinosis

INITIAL CRITERIA: Cysteamine hydrochloride (Cystaran®/Cystadrops®) Ophthalmic Solution is approved when BOTH of the following are met:

1. Diagnosis of cystinosis; AND
2. Member has corneal cystine crystal accumulation

Initial authorization duration: 2 years

REAUTHORIZATION CRITERIA Cysteamine hydrochloride (Cystaran®, Cystadrops®) is re-approved when there is documentation of positive clinical response to therapy.

Reauthorization duration: 2 years

Nephrotic Cystinosis

INITIAL CRITERIA: Cysteamine bitartrate (Cystagon®) is approved when there is a diagnosis of nephrotic cystinosis.

Initial authorization: 2 years

REAUTHORIZATION CRITERIA Cysteamine bitartrate (Cystagon®) is re-approved when there is documentation of positive clinical response to therapy.

Reauthorization duration: 2 years

INITIAL CRITERIA: Cysteamine bitartrate (Procysbi®) is approved when ALL of the following are met:

1. Diagnosis of nephrotic cystinosis; AND
2. Member is 1 year of age or older; AND
3. Inadequate response or titration from cysteamine bitartrate immediate release capsules (Cystagon®)

Initial Authorization duration : 2 years

REAUTHORIZATION CRITERIA: Cysteamine bitartrate (Procysbi®) is re-approved when there is documentation of positive clinical response to therapy.

Reauthorization duration: 2 years

Black Box Warning as shown in the drug Prescribing Information:

N/A

Guidelines:

Refer to the specific manufacturer's prescribing information for administration and dosage details and any applicable Black Box warnings.

BENEFIT APPLICATION

Subject to the terms and conditions of the applicable benefit contract, the applicable drug(s) identified in this policy is (are) covered under the prescription drug benefits of the Company's products when the medical necessity criteria listed in this pharmacy policy are met. Any services that are experimental/investigational or cosmetic are benefit contract exclusions for all products of the Company.

References:

Cystadrops® [package insert] Lebanon, NJ. Recordati Rare Diseases Inc. August 2020. Available from: https://www.cystadrops.com/wp-content/uploads/cystadrops-prescribing-information.pdf. Accessed January 14, 2022.

Cystagon® [package insert]. Morgantown WV. Mylan Pharmaceuticals Inc. Jan 2019. Available from: https://www.accessdata.fda.gov/drugsatfda_docs/label/2007/020392s010lbl.pdf. Accessed January 14, 2022.

Cystanosis. National Organization for Rare Disorders. Available at: http://rarediseases.org/rare-diseases/cystinosis/. Accessed January 14, 2022.

Cystaran® [package insert]. Amityville NY. Sigma-Tau Pharmaceuticals, Inc. April 2020. Available from: https://www.accessdata.fda.gov/drugsatfda_docs/label/2012/200740s000lbl.pdf. Accessed January 14, 2022.

Niaudet P. Cystinosis. UpToDate. February 2020. Available at: https://www.uptodate.com/contents/cystinosis?source=search_result&search=cystinosis&selectedTitle=1~31. Accessed January 14, 2022.

Procysbi® [package insert]. Novato CA. Raptor Pharmaceuticals Inc. February 2020. Available from: https://www.accessdata.fda.gov/drugsatfda_docs/label/2015/203389s010lbl.pdf. Accessed January 14, 2022.

Applicable Drugs:

Inclusion of a drug in this table does not imply coverage. Eligibility, benefits, limitations, exclusions, precertification/referral requirements, provider contracts, and Company policies apply.

Brand Name	Generic Name
Cystagon®	Cysteamine Bitartrate
Cystaran®/Cystadrops®	Cysteamine Hydrochloride

Procysbi®	Cysteamine Bitartrate

Cross References:

Off-Label Use Rx. 01.33

Quantity Level Limits for Pharmaceuticals Covered Under the Prescription Drug Benefit Rx.01.76

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