Cystinosis Agents
Cystaran (cysteamine 0.44% ophthalmic solution)
Cystadrops (cysteamine 0.37% ophthalmic solution)
Procysbi (cysteamine delayed-release capsule, granule)
Effective 01/01/2023

Plan	☒ MassHealth (UPPL) ☐Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit (NLX)		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Specialty Medications**		
	All Plans	Phone: 866-814-5506	Fax: 866-249-6155
	Non-Specialty Medications		
	MassHealth	Phone: 877-433-7643	Fax: 866-255-7569
	Commercial	Phone: 800-294-5979	Fax: 888-836-0730
	Exchange	Phone: 855-582-2022	Fax: 855-245-2134
	Medical Specialty Medications (NLX)		
	All Plans	Phone: 844-345-2803	Fax: 844-851-0882
Exceptions	N/A		

Overview

Procysbi® (cysteamine delayed-release) capsules and granules are cystine-depleting agents indicated for the treatment of nephropathic cystinosis in adults and children ages one year and older.[1]

Cystaran® (cysteamine 0.44% ophthalmic solution) is an ophthalmic solution indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis.[4] A new formulation, Cystadrops® (cysteamine 0.37% ophthalmic solution) offers four times daily dosing compared to every-hour dosing (during waking hours) with Cystaran® (cysteamine 0.44% ophthalmic solution).[5]

No PA	Drugs that require PA
Cystagon® (cysteamine immediate-release capsule)	Cystadrops® (cysteamine 0.37% ophthalmic solution)
	Cystaran® (cysteamine 0.44% ophthalmic solution)
	Procysbi® (cysteamine delayed-release) capsule
	Procysbi® (cysteamine delayed-release) granule

Coverage Guidelines

Authorization may be reviewed on a case by case basis for members who are new to the plan currently receiving treatment with requested medication excluding when the product is obtained as samples or via manufacturer's patient assistance programs.

OR

Authorization may be granted for members when all the following criteria are met, and documentation is provided:

Corneal cystine crystal accumulation due to cystinosis
Cystadrops® (cysteamine 0.37% ophthalmic solution)
Cystaran® (cysteamine 0.44% ophthalmic solution)
Prescriber provides documentation of **ALL** of the following:

1. Appropriate diagnosis
2. Appropriate dosing
3. Prescriber is a nephrologist or ophthalmologist

Nephropathic Cystinosis
Procysbi® (cysteamine delayed-release) capsules
Procysbi® (cysteamine delayed-release) granules
Prescriber provides documentation of **ALL** of the following:

1. Appropriate diagnosis
2. Appropriate dose (weight, height, or BSA required)*
3. Prescriber is a nephrologist
4. Physician documented of an inadequate response or adverse reaction to Cystagon® (cysteamine immediate-release capsule)
5. If the request if for **Procysbi**® granules, compelling clinical rationale for use of the requested formulation

**If 75 mg dose is requested, ensure dose consolidation and use of 75 mg capsule instead of three 25 mg capsules*

Continuation of Therapy
For **Cystadrops**®, **Cystaran**®, and **Procysbi**® capsules: Reauthorization by physician will infer a positive response to therapy.

For **Procysbi**® granules: Reauthorization requires physician documentation of continued necessity of dosage form.

Limitations

1. Authorizations will be approved for 12 months.

Drug	Dosing
Cystadrops® (cysteamine 0.37% ophthalmic solution) Ophthalmic solution: 5 mL bottle Bottle must be discarded 7 days after opening	Cystinosis: One drop in each eye, four times a day during waking hours.

Cystaran® (cysteamine 0.44% ophthalmic solution) Ophthalmic solution: 15 mL bottle (300 drops)	Cystinosis: Ophthalmic drops: One drop in each eye every waking hour Bottle must be discarded after one week of use.
Procysbi® (cysteamine delayed-release) Capsules: 25 mg 75 mg Granules: 75 mg 300 mg	Cystinosis: Capsules and granules: initial, 1/6 to 1/4 of the maintenance dose; maintenance,1.3 g/m^2 of BSA per day in two divided doses given every 12 hours; maximum, 1.95 g/m^2/day The dose should be raised gradually over four to six weeks to help reduce the incidence of adverse events.

References

1. Procysbi® [package insert]. Deerfield (IL): Horizon Pharma USA, Inc.; 2021 Mar.
2. FDA approves Procysbi for rare genetic condition [press release on the Internet]. Rockville (MD): Food and Drug Administration (US): 2013 Apr 30 [cited 2021 Jul 18]. Available from: https://www.fiercebiotech.com/biotech/fda-approves-procysbi-for-rare-genetic-condition.
3. Langman CB, Greenbaum LA, Sarwal M, Grimm P, Niaduet P, Deschenes G, et al. A randomized controlled crossover trial with delayed-release cysteamine bitartrate in nephropathic cystinosis: effectiveness on white blood cell cystine levels and comparison of safety. Clin J Am Soc Nephrol. 2012;1-9.
4. Cystaran® [package insert]. Gaithersburg (MD): Leadiant Biosciences, Inc.; 2020 Apr.
5. Cystadrops® [prescribing information]. Lebanon (NJ): Recordati Rare Diseases Inc.; 2020 Aug.
6. Niaudet P. Cystinosis. In Basow DS (Ed). UpToDate [database on the Internet]. Waltham (MA): UpToDate; 2019 [cited 2021 Jul 18]. Available from: http://www.utdol.com/utd/index.do.

Review History

09/21/2022 – Reviewed and Created for Sept P&T; matched MH UPPL.

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