

CADTH Drug Implementation Advice

CYSTEAMINE DELAYED-RELEASE (PROCYSBI)

(HORIZON PHARMA IRELAND LTD.)

Indication: Treatment of nephropathic cystinosis

Service Line: CADTH Drug Implementation Advice
Version: Final
Publication Date: July 2019
Report Length: 4 Pages

Disclaimer: The information in this document is intended to help Canadian health care decision-makers, health care professionals, health systems leaders, and policy-makers make well-informed decisions and thereby improve the quality of health care services. While patients and others may access this document, the document is made available for informational purposes only and no representations or warranties are made with respect to its fitness for any particular purpose. The information in this document should not be used as a substitute for professional medical advice or as a substitute for the application of clinical judgment in respect of the care of a particular patient or other professional judgment in any decision-making process. The Canadian Agency for Drugs and Technologies in Health (CADTH) does not endorse any information, drugs, therapies, treatments, products, processes, or services.

While care has been taken to ensure that the information prepared by CADTH in this document is accurate, complete, and up-to-date as at the applicable date the material was first published by CADTH, CADTH does not make any guarantees to that effect. CADTH does not guarantee and is not responsible for the quality, currency, propriety, accuracy, or reasonableness of any statements, information, or conclusions contained in any third-party materials used in preparing this document. The views and opinions of third parties published in this document do not necessarily state or reflect those of CADTH.

CADTH is not responsible for any errors, omissions, injury, loss, or damage arising from or relating to the use (or misuse) of any information, statements, or conclusions contained in or implied by the contents of this document or any of the source materials.

This document may contain links to third-party websites. CADTH does not have control over the content of such sites. Use of third-party sites is governed by the third-party website owners' own terms and conditions set out for such sites. CADTH does not make any guarantee with respect to any information contained on such third-party sites and CADTH is not responsible for any injury, loss, or damage suffered as a result of using such third-party sites. CADTH has no responsibility for the collection, use, and disclosure of personal information by third-party sites.

Subject to the aforementioned limitations, the views expressed herein do not necessarily reflect the views of Health Canada, Canada's provincial or territorial governments, other CADTH funders, or any third-party supplier of information.

This document is prepared and intended for use in the context of the Canadian health care system. The use of this document outside of Canada is done so at the user's own risk.

This disclaimer and any questions or matters of any nature arising from or relating to the content or use (or misuse) of this document will be governed by and interpreted in accordance with the laws of the Province of Ontario and the laws of Canada applicable therein, and all proceedings shall be subject to the exclusive jurisdiction of the courts of the Province of Ontario, Canada.

The copyright and other intellectual property rights in this document are owned by CADTH and its licensors. These rights are protected by the *Canadian Copyright Act* and other national and international laws and agreements. Users are permitted to make copies of this document for non-commercial purposes only, provided it is not modified when reproduced and appropriate credit is given to CADTH and its licensors.

About CADTH: CADTH is an independent, not-for-profit organization responsible for providing Canada's health care decision-makers with objective evidence to help make informed decisions about the optimal use of drugs, medical devices, diagnostics, and procedures in our health care system.

Funding: CADTH receives funding from Canada's federal, provincial, and territorial governments, with the exception of Quebec.

Drug	Cysteamine delayed-release (Procysbi)
Indication	Treatment of nephropathic cystinosis
Dosage Form	Delayed-release 25 mg and 75 mg capsules
NOC Date	June 13, 2017
Manufacturer	Horizon Pharma Ireland Lid.
Date the CDEC Recommendation Issued	January 24, 2018

Background

Based on the review of delayed-release cysteamine (Procysbi) through the CADTH Common Drug Review (CDR), the CADTH Canadian Drug Expert Committee (CDEC) issued the following reimbursement recommendation:

CDEC Recommendation for Cysteamine Delayed-Release (Procysbi)

The CADTH Canadian Drug Expert Committee (CDEC) recommends that delayed-release cysteamine be reimbursed for treatment of nephropathic cystinosis, if the following criterion and conditions are met:

Criterion:

For use in patients with an established diagnosis of infantile nephropathic cystinosis with documented cystinosis, lysosomal cystine transporter gene mutation.

Conditions:

- The patient is under the care of a physician with experience in the diagnosis and management of cystinosis.
- Significant reduction in price.

Implementation Issue

Since the delayed-release cysteamine (Procysbi) recommendation was issued, CADTH has received feedback from the CDR-participating drug plans that the requirement of “*documented cystinosis, lysosomal cystine transporter gene mutation*” is an implementation challenge for the drug plans as the genetic test is not currently part of routine clinical practice. Therefore, the CDR-participating drug plans have requested that CADTH provide implementation advice regarding an appropriate criterion for the diagnosis of infantile nephropathic cystinosis.

Consultation Process

CADTH convened a panel of three clinical experts with experience in treating patients with cystinosis. CADTH staff prepared a summary of the input provided by the panel, with input from the panellists. CDR-participating drug plans and the manufacturer of Procysbi were given the opportunity to comment on the draft document.

Objective

The objective of this Implementation Advice is to propose a criterion for demonstrating a diagnosis of infantile nephropathic cystinosis that can be used for implementing the delayed-release cysteamine (Procysbi) CDEC recommendation.

Implementation Advice

The proposed criterion for demonstrating a diagnosis of infantile nephropathic cystinosis for the purposes of delayed-release cysteamine reimbursement by the CDR-participating drug plans is shown in Table 1. A summary of the relevant clinical panel input is provided below.

Table 1: Criterion for the Diagnosis of Infantile Nephropathic Cystinosis

Current Criterion in the Cysteamine Delayed-Release (Procysbi) CDEC Recommendation
For use in patients with an established diagnosis of infantile nephropathic cystinosis with documented cystinosis, lysosomal cystine transporter gene mutation.
Proposed Criterion for Implementation of the Cysteamine Delayed-Release (Procysbi) CDEC Recommendation
For use in patients with an established diagnosis of infantile nephropathic cystinosis with documented high levels of leukocyte cystine.

CDEC = CADTH Canadian Drug Expert Committee.

Summary of Clinical Panel Input Regarding the Diagnosis of Nephropathic Cystinosis

The panellists agreed that a demonstration of high levels of leukocyte cystine following classic presentation of nephropathic cystinosis is sufficient to diagnose the disease. Genetic testing for mutations of the gene that encodes cystinosis may be useful for early diagnosis in infant siblings of affected cystinosis patients. However, it was noted that genetic confirmation is not required to diagnose nephropathic cystinosis and it may not be readily available in all jurisdictions, unlike the test for leukocyte cystine.