CADTH

CADTH Drug Implementation Advice

CYSTEAMINE DELAYED-RELEASE (PROCYSBI)

(HORIZON PHARMA IRELAND LTD.)

Indication: Treatment of nephropathic cystinosis

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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.

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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 cystinosin, 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 cystinosin, 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 cystinosin, 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 cystinosin 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.