



## Horizon Pharma plc Announces Availability of PROCYSBI™ (cysteamine bitartrate) in Canada

October 26, 2017

DUBLIN, Ireland, Oct. 26, 2017 (GLOBE NEWSWIRE) -- Horizon Pharma plc (NASDAQ:HZNP), a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring and commercializing differentiated and accessible medicines that address unmet medical needs, and its affiliate Horizon Therapeutics Canada, today announced that PROCYSBI™ (cysteamine bitartrate) is now available in Canada. Health Canada issued a Notice of Compliance (NOC) for PROCYSBI in June 2017 for the treatment of nephropathic cystinosis in adults and children two years of age and older.

"We've been honored to collaborate with the dedicated healthcare professionals, advocates, people living with nephropathic cystinosis and their families who have driven research down a path that led to today's announcement," said Eric Mosbrooker, senior vice president, orphan business unit, Horizon Pharma plc. "We're looking forward to our continued work supporting the cystinosis community in Canada."

PROCYSBI is the only delayed release form of cysteamine bitartrate to receive an NOC in Canada for the treatment of nephropathic cystinosis.

"The arrival in Canada of a long acting form of cysteamine taken only twice a day is a significant achievement for people living with cystinosis," said Paul Goodyer, M.D., pediatric nephrologist at the Montreal Children's Hospital. "As a pediatric nephrologist who has cared for many young patients in Quebec over the years, I am delighted to see this important step forward."

Cystinosis is a rare metabolic lysosomal storage disorder that causes toxic accumulation of cystine in all cells, tissues, and organs in the body. Elevated cystine leads to progressive, irreversible tissue damage and multi-organ failure, including kidney failure, blindness and muscle wasting. It is estimated that only about 2,000 people worldwide are currently diagnosed with cystinosis. Nephropathic or "classic infantile" cystinosis is the most common and most severe form of the disease, and is typically diagnosed in infancy and requires lifelong therapy.<sup>1</sup>

"This is a major achievement for the cystinosis community in Canada, providing people living with the disease as well as their families with a new treatment option for a disease that is severe and potentially devastating if left untreated," said Nancy Stack, chair of the board, Cystinosis Research Foundation. "From the earliest days of research evaluating delayed-release cysteamine, the Cystinosis Research Foundation has diligently partnered with academic institutions, industry and families impacted by cystinosis to help bring this medicine forward."

For more information about the availability of PROCYSBI in Canada, including Horizon's patient support programs, please call 1-844-380-7850.

### About PROCYSBI

In Canada, PROCYSBI (cysteamine bitartrate) delayed-release capsules is indicated for the treatment of nephropathic cystinosis.

### Important Safety Information

#### CONTRAINDICATIONS:

- Hypersensitivity to cysteamine bitartrate, any form of cysteamine, any ingredient in the formulation or component in the container.
- Hypersensitivity to penicillamine.

#### WARNINGS AND PRECAUTIONS:

- Ehlers-Danlos-like Syndrome: Skin and bone lesions that resemble clinical findings for Ehlers-Danlos-like syndrome have been reported in patients treated with high doses of immediate-release cysteamine bitartrate or other cysteamine salts.
  - These include molluscoid pseudotumors (purplish hemorrhagic lesions), skin striae, bone lesions (including osteopenia, compression fractures, scoliosis and genu valgum), leg pain, and joint hyperextension.
  - One patient on immediate-release cysteamine bitartrate with serious skin lesions subsequently died of acute cerebral ischemia with marked vasculopathy.
  - Monitor patients for development of skin or bone lesions and interrupt PROCYSBI dosing if patients develop these lesions. PROCYSBI may be restarted at a lower dose under close supervision, then slowly increased to the appropriate therapeutic dose.
- Skin Rash: Severe skin rashes such as erythema multiforme bullosa, Stevens-Johnson Syndrome (SJS), or toxic epidermal necrolysis have been reported in patients receiving immediate-release cysteamine bitartrate. If serious skin rashes develop, permanently discontinue use of PROCYSBI.
- Gastrointestinal Ulcers and Bleeding: Gastrointestinal (GI) ulceration and bleeding have been reported in patients receiving immediate-release cysteamine bitartrate.
  - GI tract symptoms including nausea, vomiting, anorexia and abdominal pain, sometimes severe, have been associated with cysteamine. If severe GI tract symptoms develop, consider decreasing the dose of PROCYSBI.
  - Risk of fibrosing colonopathy: Monitor patients for unusual abdominal symptoms or changes in abdominal symptoms.
- Hepatic Impairment: PROCYSBI has not been studied in patients with hepatic impairment. Close monitoring of the WBC cystine levels is recommended in these patients.
- Central Nervous System Symptoms: Central Nervous System (CNS) symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy have been associated with immediate-release cysteamine.

- Carefully evaluate and monitor patients who develop CNS symptoms. Interrupt medication or adjust the dose as necessary for patients with severe symptoms or with symptoms that persist or progress.
- Leukopenia and/or Elevated Alkaline Phosphatase Levels: Cysteamine has been associated with reversible leukopenia and elevated alkaline phosphatase levels. Monitor white blood cell counts and alkaline phosphatase levels. If tests values remain abnormal, consider adjusting or discontinuing the drug until values revert to normal.
- Gastric pH: Caution while using drugs that increase gastric pH. More frequent monitoring of WBC cystine concentration is recommended.
- Oral cysteamine: Oral cysteamine does not prevent eye deposition of cystine crystals. Continue use of cysteamine ophthalmic solution if required.
- Renal disease: Monitor WBC cystine levels in patients with severe or end stage renal disease.
- Benign Intracranial Hypertension: Benign intracranial hypertension (pseudotumor cerebri; PTC) and/or papilledema has been reported in patients receiving immediate-release cysteamine bitartrate treatment.
  - Monitor patients for signs and symptoms of PTC, including headache, tinnitus, dizziness, nausea, diplopia, blurry vision, loss of vision, pain behind the eye or pain with eye movement. If signs/symptoms persist, interrupt dosing or decrease the dose and refer the patient to an ophthalmologist. If the diagnosis is confirmed, permanently discontinue use of PROCYSBI.
- Monitoring and Laboratory Tests: Monitor WBC cystine levels to assess PROCYSBI treatment.

#### USE IN SPECIFIC POPULATIONS:

*Women of child bearing potential:* Before starting PROCYSBI, pregnancy status should be confirmed. Patients should be advised of the potential risk to a fetus and the importance of ensuring adequate contraception while taking PROCYSBI.

*Lactation:* Breastfeeding is not recommended while taking PROCYSBI.

Please consult the [product monograph](#) for important information relating to adverse reactions, drug interactions, and dosing information which has not been discussed in this press release.

#### About Horizon Pharma plc

Horizon Pharma plc is a biopharmaceutical company focused on improving patients' lives by identifying, developing, acquiring and commercializing differentiated and accessible medicines that address unmet medical needs. The Company markets 11 medicines through its orphan, rheumatology and primary care business units. For more information, please visit [www.horizonpharma.com](http://www.horizonpharma.com). Follow [@HZNPplc](#) on Twitter, like us on [Facebook](#), or view careers on our [LinkedIn](#) page.

#### Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential of PROCYSBI to treat patients with nephropathic cystinosis. These forward-looking statements are based on management expectations and assumptions as of the date of this press release, and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include whether PROCYSBI will be successfully commercialized in Canada and whether patients are willing to use PROCYSBI to treat nephropathic cystinosis, as well as those factors described in Horizon Pharma's filings with the United States Securities and Exchange Commission, including those factors discussed under the caption "Risk Factors" in those filings. Forward-looking statements speak only as of the date of this press release and Horizon Pharma does not undertake any obligation to update or revise these statements, except as may be required by law.

#### Contacts:

Tina Ventura  
Senior Vice President, Investor Relations  
[Investor-relations@horizonpharma.com](mailto:Investor-relations@horizonpharma.com)

Ruth Venning  
Executive Director, Investor Relations  
[Investor-relations@horizonpharma.com](mailto:Investor-relations@horizonpharma.com)

#### U.S. Media Contact:

Matt Flesch  
Executive Director, Product Communications  
[media@horizonpharma.com](mailto:media@horizonpharma.com)

#### Ireland Media Contact:

Ray Gordon  
Gordon MRM  
[ray@gordonmrm.ie](mailto:ray@gordonmrm.ie)

#### References:

1. Cystinosis Research Foundation. "About cystinosis." Available at <http://www.cystinosisresearch.org/About-Cystinosis/>. Accessed Oct. 25, 2017.

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