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Horizon Pharma plc Announces Health Canada Approval of PROCYSBI™ (Cysteamine Delayed-Release Capsules) for the Treatment of Nephropathic Cystinosis

DUBLIN, Ireland, June 19, 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 Health Canada has issued a Notice of Compliance (NOC) for PROCYSBI™ (cysteamine delayed-release capsules) for the treatment of nephropathic cystinosis in adults and children 2 years of age and older.

"For children diagnosed with nephropathic cystinosis, treatment with cystine-depleting therapy should be started as soon as possible to avoid the serious and potentially life-threatening impact on tissues and organs," said Durhane Wong-Rieger, president and chief executive officer, Canadian Organization for Rare Disorders (CORD). "Prior to PROCYSBI there was no approved therapy in Canada for treating cystinosis. We are especially pleased that Health Canada has granted very timely approval of PROCYSBI, the delayed-release form of the therapy, which will help assure patients avoid toxic build-up of cystine in the cells."

PROCYSBI is the only cystine-depleting agent approved in Canada for the treatment of nephropathic cystinosis. It is a delayed-release form of cysteamine bitartrate that works by continuously reducing the toxic concentration of cystine in the cells to limit or prevent the damage too much cystine can cause to cells, tissue and organs.

"The Cystinosis Research Foundation is proud to have funded every bench and early clinical trial that led to the discovery of PROCYSBI," said Nancy Stack, chair of the board, Cystinosis Research Foundation. "The approval of PROCYSBI in Canada is a milestone achievement for the Canadian cystinosis community."

Cystinosis is a rare, life-threatening 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, muscle wasting and premature death. 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.[1]

"The Health Canada approval would not have been possible without the dedication of the entire nephropathic cystinosis community, particularly the people who participated in the PROCYSBI clinical studies, their families, as well as advocacy organizations and healthcare professionals," said Jared Rhines, group vice president and general manager, Canada, LATAM and APAC, Horizon Pharma plc. "We look forward to making PROCYSBI available to Canadians living with this extremely challenging disease."

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. Follow @HZNPplc on Twitter or view careers on our LinkedIn page.

Forward Looking Statement
This press release contains forward-looking statements, including statements regarding the future availability of PROCYSBI in Canada and 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 and sufficiently available in Canada, 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.

References:

1. Cystinosis Research Foundation. "About cystinosis." Available at http://www.cystinosisresearch.org/About-Cystinosis/. Accessed June 16, 2017.

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