



## PROCYSBI AVAILABILITY UPDATE

In June 2017, Horizon Pharma plc, and its affiliate Horizon Therapeutics Canada, announced that Health Canada issued a Notice of Compliance (NOC) for <sup>P</sup>rPROCYSBI™ (cysteamine bitartrate) for the treatment of nephropathic cystinosis in adults and children. Health Canada made this decision based on a review of two clinical trials evaluating PROCYSBI in 62 adults and children living with nephropathic cystinosis. Horizon announced the availability of the medicine in Canada on October 26, 2017.

PROCYSBI is already covered for nephropathic cystinosis patients by many private and some public insurance plans in Canada. Horizon is working with all stakeholders – including healthcare professionals, advocacy organizations, provincial governments and patients and their families – to bring PROCYSBI to nephropathic cystinosis patients throughout Canada.

Horizon is committed to providing access to PROCYSBI as rapidly as possible for all nephropathic cystinosis patients in Canada. Recent public comments have reported greatly exaggerated prices for PROCYSBI. Although dependent on the weight and cystine level for each individual patient, the price is significantly lower than what has been speculated. In setting the price for PROCYSBI, Horizon followed the Patent Medicines Price Review Board's rules and thus has set PROCYSBI's price at the lowest end of comparator countries. This price is immediately available to all nephropathic cystinosis patients whether they have private or public insurance.

Horizon Therapeutics Canada is open to working with the provinces, territories and federal plans, who are committed to a long term funding solution for PROCYSBI, to identify strategies to help support any patient in the interim.

Patients and/or their caregiver interested in PROCYSBI availability should speak with their physician. If the physician decides to prescribe PROCYSBI, they will need to call *TranscendRare* (1-844-380-7850) to obtain a "PROCYSBI Enrollment Form." Horizon's *TranscendRare* program is dedicated to improving the lives of people living with nephropathic cystinosis through a network of services and support, including working with insurance providers and communicating with the physician and/or patient regarding the insurer's decision, next steps and options moving forward.

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