



## **Horizon Therapeutics Canada Statement Regarding PMPRB Notice of Hearing**

Horizon Therapeutics Canada is deeply committed to the nephropathic cystinosis and rare disease community in Canada. We have collaborated with nephropathic cystinosis advocates, healthcare professionals and Canadian regulatory and reimbursement authorities, and invested significant resources to make PROCYSBI® (cysteamine bitartrate) delayed-release capsules available in Canada. PROCYSBI was approved after a priority review by Health Canada in 2017, providing an important advance for the approximately 100 people in Canada living with nephropathic cystinosis, a progressive, extremely severe, genetic disease, characterized by kidney and other vital organ damage or failure, and premature death.

Given the seriousness of this disease and the advance PROCYSBI represents, Horizon has ensured that all patients prescribed PROCYSBI are able to receive the medicine regardless of their insurance coverage or ability to pay. No patient in Canada has to pay out of pocket for PROCYSBI. Horizon has ensured that Canadian patients are fully covered through private insurance, provincial and federal drug plans, and supplemented by Horizon's patient support program that covers co-payments and provides free PROCYSBI to patients with no drug plan coverage. Furthermore, Horizon has signed agreements for PROCYSBI with provincial and federal authorities to ensure value and cost certainty for public drug plans across Canada.

On January 16<sup>th</sup>, 2019, the Patented Medicines Pricing Review Board (PMPRB) issued Horizon Therapeutics Canada a notice of hearing investigating the Canadian price of PROCYSBI. This notice of hearing was issued despite established provincial and federal pricing agreements and Horizon's commitment to maintain its patient support program that ensures Canadian patients have access to PROCYSBI regardless of their insurance coverage or ability to pay. PMPRB rejected Horizon's proposed list price, ignoring its own guidelines and demanding a price reduction that would bring the Canadian price of PROCYSBI to a small fraction of the lowest price in the world, thereby threatening the continued availability of PROCYSBI in Canada. Horizon has made significant investments to develop PROCYSBI, secure Health Canada approval, maintain quality, safety and availability, and support all Canadian patients. We have always supported patients having choice, and encourage the distributor of immediate-release cysteamine to commit to the nephropathic cystinosis community in Canada and invest in the established safety, efficacy, quality, regulatory and reimbursement processes.

Recently released proposed changes to PMPRB's guidelines target innovative medicines that serve some of the most vulnerable and smallest patient populations in the country, despite the cost of these medicines representing a very small percentage of the overall Canada drug budget. There will be severe ramifications for the future availability of innovative therapies for people living with rare diseases in Canada if PMPRB forces biopharmaceutical companies to reduce prices to a level where investments are never recouped.

Horizon stands by its commitment to the nephropathic cystinosis community, its investment to bring PROCYSBI to market, and the innovative advancement the medicine has brought to patients. We will continue to make PROCYSBI available as we work through this process for the benefit of people living with nephropathic cystinosis and their families across Canada.