

COMMITTED TO THE Cystinosis Community

Horizon Therapeutics Canada is proud to be one of the few companies actively working to make rare disease medicines available in Canada. Our goal is to deliver medicine to Canadians living with some of the most challenging and devastating rare diseases in the world. As Horizon Therapeutics Canada continues to grow, so will our efforts in the cystinosis and rare disease community. We have developed this resource to be transparent about our past, current, and future activities, and to detail the support that is available from Horizon Therapeutics Canada and other organizations.

You have our commitment to do our best to better the lives of Canadians living with cystinosis. We are honoured to work with such a strong community, and will continue to invest in the care of Canadians living with cystinosis today and into the future.



Connection to Rare Disease Support and Services

Horizon provides support to many other organizations that offer a wide range of crucial programs and services for people living with rare diseases, including cystinosis.

NAPRTCS—North American Pediatric Renal Trials and Collaborative Studies (<https://secure.emmes.com/emmesweb/projects/120>)

Horizon provided financial support to develop a comprehensive patient registry to help understand the natural history of cystinosis and explore new approaches that can advance the treatment of the disease.

Global Genes (GlobalGenes.org)

Develops educational resources, programs, and events that unite patients, advocates, and industry experts.

- **Global Genes RARE Patient Advocacy Summit:** The largest rare patient advocate event worldwide
- **Scholarship to the RARE Patient Advocacy Summit:** With the help of generous sponsorships, advocates from all over the globe are afforded travel and lodging scholarships to attend the Summit. E-mail Events@globalgenes.org for scholarship information

The Sing Me a Story Foundation (SingMeAStory.org)

Combines the imaginations of children with the talents of songwriters to create stories and songs that bring joy to all those involved.

The following organizations provide additional information and support for patients who are living with rare diseases like cystinosis:

- Canadian Organization for Rare Disorders (raredisorders.ca)
- National Organization for Rare Disorders (rarediseases.org)
- Rare Disease Information and Resource Centre (French only; rqmo.org)
- Peer 2 Peer Network at Rare Disease Foundation (rarediseasefoundation.org)

Support and Assistance

At Horizon Therapeutics Canada, we are dedicated to providing support for our patients.

As part of our commitment, we offer **TranscendRare™**, a comprehensive program for patients on our cystinosis treatment.



Financial support for eligible patients

- Reimbursement assistance
- Co-pay assistance
- Compassionate use programs



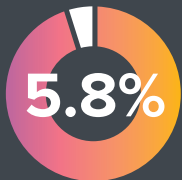
Healthcare services

- Pharmacy counselling and free home deliveries
- Medication re-orders
- Over-the-phone nursing support
- In-home nurse training when necessary
 - Gastrostomy tube use
 - Medication administration



Patient education and management

- Reminder calls about orders to ensure patients are taking medication as prescribed, and to answer any questions they may have



Expected percentage of total Canadian pharmaceutical drug expenditures for **rare disease drugs** in 2018¹

Price Does Not Impact Our Patients

The cost of our medicines is either covered by private insurance, government programs, Horizon patient support and compassionate use programs, or through our co-pay assistance program

\$1 billion

in global patient support provided by Horizon every year since 2016

Community Engagement



50+

patient advocacy group partnerships globally, including leading **cystinosis** and **kidney** advocacy organizations

Research and Development

\$180 million+

invested from 2005–2017 **toward the research, development, and approval** of our cystinosis treatment*



Additional investments were used for **manufacturing and supplying** our cystinosis treatment, and providing **support and resources** for a patient population of **fewer than 100 in Canada**

9 **clinical trials**
INVOLVING:

132 patients living with cystinosis



of patients were Canadian

142 other study volunteers
(110 healthy volunteers for pharmacokinetic and bioequivalence studies and 32 patients with renal insufficiency/failure who did not have cystinosis)

18 research projects

led by academic institutions and other organizations have been funded by Horizon,* **INCLUDING:**



10

cystinosis studies

8

studies exploring our cystinosis treatment **in other rare disease types**

ADVANCING NEW RARE DISEASE OPTIONS

\$225 million

in research and development expenses incurred by Horizon in 2017 for rare and rheumatic diseases

3

clinical research programs exploring **new compounds**

5

clinical research programs exploring **new uses** for our current medicines

* This investment includes Raptor research and development costs that were built into the Horizon Pharma acquisition cost.

If you would like to learn more about the services offered by TranscendRare™, contact us:



Call us at
1-844-380-7850
Monday to Friday, 8 am to 8 pm (EST)



E-mail us at
transcendrare@innomar-strategies.com

Reference:

1. Divino V, et al. Pharmaceutical expenditure on drugs for rare diseases in Canada: a historical (2007–13) and prospective (2014–18) MIDAS sales data analysis. *Orphanet J Rare Dis.* 2016;11(1):68.

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If you would like to speak with Horizon about our work—or have ideas you would like to share—please send an e-mail to connect@horizontherapeutics.com.