

We have created this resource to be transparent about our past, current and future activities in the cystinosis and rare disease community. We've also detailed support for the community that is available from Horizon and other organizations.

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

Support and Assistance

Patient support

and assistance in place so that where eligible patients are able to receive our medicines **regardless of their ability to pay***

* Assumes utilization of Horizon Pharma financial support programs

Connection

to resources that may help with access to **white blood cell (WBC) cystine testing**, travel to see a specialist, education and **support through life's transitions** (changing schools, transition to adult care, changing employment) and more

Community Engagement



17

Horizon-hosted events in 2017 that brought together people living with cystinosis to learn and connect



50+

rare disease events sponsored and attended each year by Horizon employees



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 and development** of our medicine for nephropathic cystinosis**

9 clinical trials

INVOLVING

132 Patients living with cystinosis

142 Other study volunteers (110 healthy volunteers for pharmacokinetic and bioequivalence studies and 32 patients with renal insufficiency/failure who do 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 Horizon's nephropathic cystinosis medicine in **other rare disease types**

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

ADVANCING NEW RARE DISEASE OPTIONS

\$225 million

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

*** Includes costs associated with the acquisition of River Vision Development Corp.

3

clinical research programs exploring **new compounds**

5

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

Resources

Connection to Rare Disease Support and Services

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

National Organization for Rare Disorders (RareDiseases.org)

Provides many resources for people living with rare diseases, their families and other advocates, including but not limited to:

- **Emergency Relief Support:** May offer small grants to families or individuals in need of financial support when faced with limited resources to pay for unexpected or emergency expenses.
- **Medical Foods and Supplements:** May provide assistance with high out-of-pocket costs associated with the purchase of medical foods and supplements.
- **Diagnostic Support:** May provide assistance for diagnostic testing.

For information about each of the above programs, send an e-mail to orphan@rare diseases.org.

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.

Miracle Flights (MiracleFlights.org)

Provides financial assistance to low-income children for commercial air travel to obtain special medical care.

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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.

Good Days (GoodDays.org)

Works to improve the health and quality of life of patients with chronic disease, cancer, or other life-altering conditions. The organization may help provide families with financial and travel assistance.

The HealthWell Foundation (HealthWellFoundation.org)

Independent non-profit dedicated to reducing financial barriers to care for underinsured Americans with chronic and life-altering medical conditions. HealthWell offers a financial lifeline to adults and children who desperately need critical medical treatments but can't afford them by assisting with their cost-sharing obligations.



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@HorizonPharma.com.

About Horizon Pharma plc

Horizon Pharma plc is focused on researching, developing and commercializing innovative medicines that address unmet treatment needs for rare and rheumatic diseases. By fostering a growing pipeline of medicines in development and exploring all potential uses for currently marketed medicines, we strive to make a powerful difference for patients, their caregivers and physicians. For us, it's personal: by living up to our own potential, we are helping others live up to theirs. For more information, please visit www.horizonpharma.com. Follow @HZNPlc on Twitter, like us on Facebook or explore career opportunities on LinkedIn.