

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 applicable, patients are able to receive our medicines **regardless of their ability to pay\***

\* Assumes utilization of Horizon 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)*

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



## 18 research projects

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

INVOLVING

**10** cystinosis studies

**8** studies exploring Horizon's nephropathic cystinosis medicine in **other rare disease types**

## \$3 million

dollar gift to **Children's National Rare Disease Institute** to establish an expert care team in rare disease

**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.

**2**

clinical research programs exploring **new compounds**

**3**

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

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# 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](http://RareDiseases.org))

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

- **Co-pay Program\***: assists eligible individuals who have health insurance to cover health insurance deductibles, copayments and coinsurance costs associated with the care of cystinosis.
- **Medical Assistance Program\***: assists eligible individuals who are uninsured or for whom coverage has been denied, with out-of-pocket costs for medical expenses such as medical visits, laboratory and diagnostic testing, physician prescribed medical foods & supplements, travel to and from a disease-related medical appointment and other expenses for the care of cystinosis.
- **Emergency Relief Program**: May assist eligible individuals and/or families who need financial support when faced with limited resources to pay for unexpected or emergency non-medical expenses.

For information about each of the above programs, send an e-mail to [cystinosis@rarediseases.org](mailto:cystinosis@rarediseases.org).

### Global Genes ([GlobalGenes.org](http://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](mailto:events@globalgenes.org) for scholarship information.

\*costs for medications are not eligible for NORD programs.

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### Miracle Flights ([MiracleFlights.org](http://MiracleFlights.org))

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

### The Sing Me a Story Foundation ([SingMeAStory.org](http://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](http://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](http://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@HorizonTherapeutics.com](mailto:connect@HorizonTherapeutics.com).

### About Horizon

Horizon is focused on researching, developing and commercializing medicines that address critical needs for people impacted by rare and rheumatic diseases. Our pipeline is purposeful: we apply scientific expertise and courage to bring clinically meaningful therapies to patients. We believe science and compassion must work together to transform lives. For more information on how we go to incredible lengths to impact lives, please visit [www.horizontherapeutics.com](http://www.horizontherapeutics.com), follow us @HorizonNews on Twitter, like us on Facebook or explore career opportunities on LinkedIn.