

Support for the

Cystinosis Community

At Horizon, we strive to make a powerful difference for patients, their caregivers and clinicians every day.

Founded in 2008 as a startup with only a handful of employees and no office space, Horizon now has over 1,200 employees focused on helping those impacted by rare and rheumatic conditions. Though we've grown, our patient-focused culture is no different today than it was when we started.

You have our commitment to do our best to better the lives of those living with cystinosis, today and into the future.

Our Dedication to People Living with Cystinosis

With support built around the patient, we serve as a partner in their journey, providing each person with access to a range of programs and resources.



Financial support and assistance in place so that, where eligible, cost is not a barrier for patients to receive our medicines¹



Dedicated one-on-one support from a Patient Access Manager (PAM) who can help navigate coverage and what to expect with treatment²



Connection to resources that may help with access to white blood cell (WBC) cystine testing, travel to see a specialist, education and support navigating school, work and life's transitions

Our Support and Engagement with the Community

Through our engagement with the cystinosis community, we go well beyond providing therapies to deliver resources that help address the needs of those living with cystinosis.

22

Horizon-hosted events in 2019 that brought people living with cystinosis together to learn and connect

50+

Industry patient advocacy **groups** supported globally including leading cystinosis and kidney advocacy organizations

50+

Rare disease events sponsored and attended each year by Horizon employees

Our Commitment to Research and Development

We embrace our responsibility to continually discover and deliver new solutions for people living with rare and rheumatic diseases, partly through collaborations with leading industry experts and organizations.



Addressing challenges of managing multiple, different medications, leading to the development of a new form of administering our medicine for cystinosis in 2020



Advancing rare disease research

by supporting **18 research projects** led by academic institutions



through a **\$3 million gift** to Children's National Rare Disease Institute

¹ Available to those prescribed with a Horizon medicine and have opted into the support

² Assumes utilization of Horizon Therapeutics financial support programs

Cystinosis Support and Services

As part of our mission, we strive to improve the experience of living with a rare disease, including through support of many organizations that provide crucial programs and services for people living with cystinosis and other rare diseases.

Financial Support

National Organization for Rare Disorders (NORD)



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



Emergency Relief Program*: supports 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 email to NORD at cystinosis@rarediseases.org.
*costs for medications are not eligible for NORD programs

The Assistance Fund

TAFcares.org

The Assistance Fund is an independent charitable patient assistance organization that provides support for adults and children with rare and chronic diseases. The organization may help provide families with financial and travel assistance.

Good Days

GoodDays.org

Good Days 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.

Community Organizations



Cystinosis Research Network (CRN)
Cystinosis.org



Dialysis Patient Citizens (DPC)
DialysisPatients.org



Cystinosis Research Foundation (CRF)
CystinosisResearch.org



The National Organization for Rare Disorders (NORD)
RareDiseases.org



American Kidney Fund (AKF)
KidneyFund.org



Global Genes
GlobalGenes.org



American Association of Kidney Patients (AAKP) AAKP.org

About Horizon

Horizon is focused on researching, developing and commercializing medicines that address critical needs for people impacted by rare and rheumatic diseases. 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.

If you would like to speak with Horizon about our work – or have ideas you would like to share – please send an email to connect@HorizonTherapeutics.com.