

At Horizon Pharma, our support of the urea cycle disorder (UCD) community is part of our overall commitment to people living with rare diseases.

We actively partner with leading professional societies and patient advocacy organizations to support people living with UCDs and their families. If you or your loved one live with a UCD, we want you to be aware of all of the resources available to you from Horizon and other organizations that Horizon supports.

## Support and Assistance

### Patient Support

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

\* Assumes utilization of Horizon Pharma financial support programs

### Connection

to help with travel to **see a specialist, support through life's transitions** (changing schools, transition to adult care, changing employment), and more

### Low-Protein Diet

tools to help manage your UCD, including **local cooking classes, online recipes** and a **cookbook**

## Community Engagement



**10+**

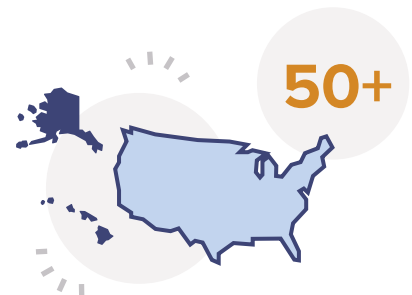
**low-protein cooking classes** specifically designed for people living with UCDs held throughout the U.S. each year



UCD in  
COMMON

Website and Facebook page co-created with **people living with UCDs** and their families provides **education** and **resources**

- Educational fact sheets and animations
- Expert videos
- Low-protein recipes
- School resources



**rare disease events** sponsored and attended each year by Horizon employees

**patient advocacy group partnerships** globally, including leading UCD advocacy organizations

# Research and Development



## OUR UREA CYCLE DISORDER MEDICINE

**12** clinical trials

INVOLVING

**140**

UCD patients

**114**

hepatic patients

**170**

healthy patients

Horizon has continued to invest in UCD research and development, **conducting studies in young children – including newborns.**



## SUPPORTING EXTERNAL RESEARCH

**6 UCD-related research projects**

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

**\$3 million**

dollar gift to **Children's National Rare Disease Institute** to create *Horizon Pharma Clinical Care Endowment* – established 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.

**4**

clinical research programs exploring **new compounds**

**3**

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

**Clinical trials** are research studies performed in people that are aimed at evaluating whether a medicine is safe and effective. Less than 12 percent of medicines that enter the clinical trial phase of study result in an approved medicine.

# Connection to Urea Cycle Disorder and 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 urea cycle disorders and other rare diseases.

### 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:

- **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@rarediseases.org](mailto:orphan@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.

### 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@HorizonPharma.com](mailto: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](http://www.horizonpharma.com). Follow [@HZNPplc](https://twitter.com/HZNPplc) on Twitter, like us on [Facebook](https://www.facebook.com/HorizonPharma) or explore career opportunities on [LinkedIn](https://www.linkedin.com/company/horizon-pharma).