

At Horizon Pharma, our support of the chronic granulomatous disease (CGD) community is part of our overall commitment to people living with rare diseases.

As part of our investment and support, we actively partner with leading professional societies and patient advocacy organizations, volunteer at hundreds of advocacy events each year and are regularly looking for innovative ways to provide new resources.

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

online and in person with other families living with CGD to help **offer tips** from their experience, **answer questions** and **support through life's transitions**



CGD Nurse Educators

available to those who are prescribed our medicine at every step of their journey. These CGD specialists provide **personalized support** and **individual guidance** about CGD and injection training

Community Engagement



CGD Connections™

website and Facebook page co-created with **people living with CGD** and their families provides **education** and **resources**

- Educational fact sheets and animations
- Expert videos
- Career and school resources
- Caregiver support



rare disease events sponsored and attended each year by Horizon employees

patient advocacy group partnerships globally, including leading primary immunodeficiency advocacy organizations



annual events

supported and attended to help **bring people together**

- Jeffrey Modell Foundation Kids Days
- Immune Deficiency Foundation Walks
- Special CGD-specific sessions at Immune Deficiency Foundation retreats and National Conferences



LivingWithCGD.org

a resource made possible through an unrestricted grant to the Immune Deficiency Foundation featuring videos and blog posts from **CGD community members** sharing their **tips, experience** and the **latest news**

Research and Development



FURTHERING EXPERTISE AND DIAGNOSIS

sponsor

the creation of the **Jeffrey Modell Foundation Diagnostic and Research Centers for Primary Immunodeficiencies** at St. Louis Children's Hospital and at the University of Virginia (opening spring 2019) to ensure cutting-edge primary immune deficiency diagnostics are available to more children

support

the development of a **Journal of Pediatric Infectious Disease Society** supplement on understanding CGD and the Clinical Immunology Society's development of a CGD specific educational video series based on the supplement



SUPPORTING EXTERNAL RESEARCH

\$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

\$10 million

in funding for **29 external studies** evaluating our medicine for CGD to help address a range of potential diseases

1,000+

DHR specimen collection kits to aid in CGD diagnosis provided at **no cost** through Horizon support

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.

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clinical research programs exploring **new compounds**

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



Please share your feedback – and any questions – by sending an e-mail to connect@HorizonPharma.com.

Connection to Chronic Granulomatous Disease 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 chronic granulomatous disease and other rare diseases.

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.
- **Diagnostic Support:** May provide assistance for diagnostic testing.

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

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 [@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).