



**HORIZON**

# Horizon at a Glance



More than  
1,000 employees



Focused on rare patient  
populations



Our medicines treat rare and  
rheumatic conditions

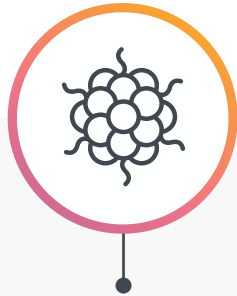


Personally invested in  
our patients wherever  
they are in their journey



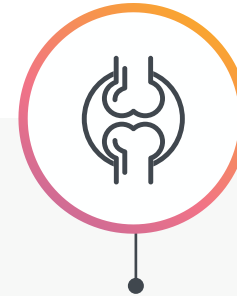
Corporate headquarters in Dublin,  
Ireland, with U.S. operations  
in Lake Forest, Illinois

# We are Bringing Forward Medicines That Address Unmet Needs in Both Rare and Rheumatic Disease



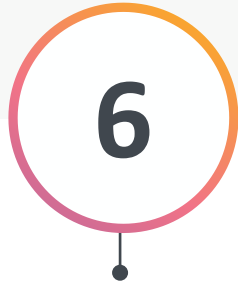
## RARE DISEASE

Urea Cycle Disorder, Cystinosis, Chronic Granulomatous Disease, Severe Malignant Osteopetrosis, Thyroid Eye Disease

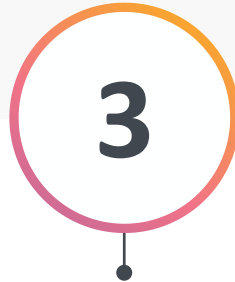


## RHEUMATIC DISEASE

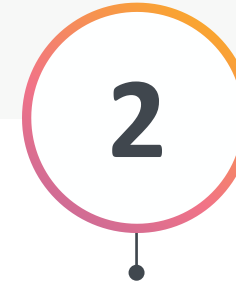
Osteoarthritis, Rheumatoid Arthritis, Polymyalgia Rheumatica, Ankylosing Spondylitis, Uncontrolled Gout



Out of 10 medicines for the treatment of **RARE DISEASES**



New clinical research programs for **DEVELOPMENT STAGE MEDICINES**



Research programs **EXPLORE THE FURTHER USE OF OUR CURRENT MEDICINES**

# Building a Robust Rare Disease Pipeline

MEDICINE / PROGRAM	DESCRIPTION	PRE-CLINICAL	PHASE 1	PHASE 2	PHASE 3	PHASE 3b / 4
KRYSTEXXA®	<ul style="list-style-type: none"> <li>MIRROR immunomodulation study: KRYSTEXXA + methotrexate</li> </ul>	.....●				
KRYSTEXXA	<ul style="list-style-type: none"> <li>Study in kidney transplant patients with uncontrolled gout<sup>(1)</sup></li> </ul>	.....●				
PROCYSBI	<ul style="list-style-type: none"> <li>NDA submitted for delayed-release oral granules in packets</li> </ul>	.....●				
Teprotumumab <sup>(2)</sup>	<ul style="list-style-type: none"> <li>OPTIC trial: Phase 3 (complete)</li> <li>OPTIC-X trial: Phase 3 extension</li> </ul>	.....●	.....●			
HZN-003	<ul style="list-style-type: none"> <li>Optimized uricase and optimized PEGylation for uncontrolled gout</li> </ul>	.....●				
HZN-007 <sup>(3)</sup>	<ul style="list-style-type: none"> <li>Optimized uricase and PASylation for uncontrolled gout</li> </ul>	.....●				
HemoShear Gout Discovery Collaboration	<ul style="list-style-type: none"> <li>Exploration of novel approaches to treating gout</li> </ul>	.....●				

(1) Planned study, expected to begin in the second half of 2019.

(2) Teprotumumab is a fully human monoclonal antibody (mAb) IGF-1R inhibitor in development for active thyroid eye disease (TED). Teprotumumab is an investigational candidate and its safety and efficacy have not been established.

(3) Being developed under a collaboration agreement with XL Protein.

MIRROR: Methotrexate to Increase Response Rates in Patients with Uncontrolled GOut Receiving KRYSTEXXA.

OPTIC: Treatment of Graves' Orbitopathy (Thyroid Eye Disease) to Reduce Proptosis with Teprotumumab Infusions in a Randomized, Placebo-Controlled, Clinical Study.



#RARE is  
My Mission

#RARE is...  
finding harmony  
while facing  
instability!

#RARE is...  
Unique and  
Complicated

# Heart of Horizon: Commitment Beyond Our Medicines

#RAREis...



# We Participate, Listen and Learn with Our Disease Communities



+50



+1,000

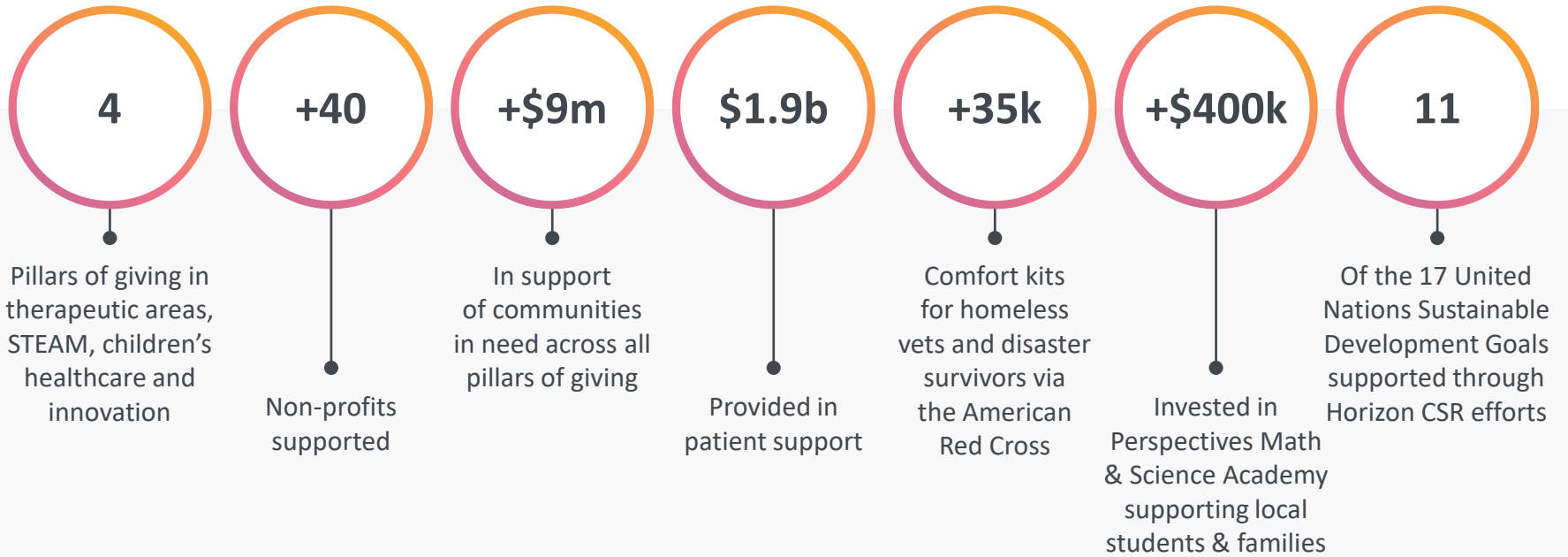


Patient advocacy group partnerships globally

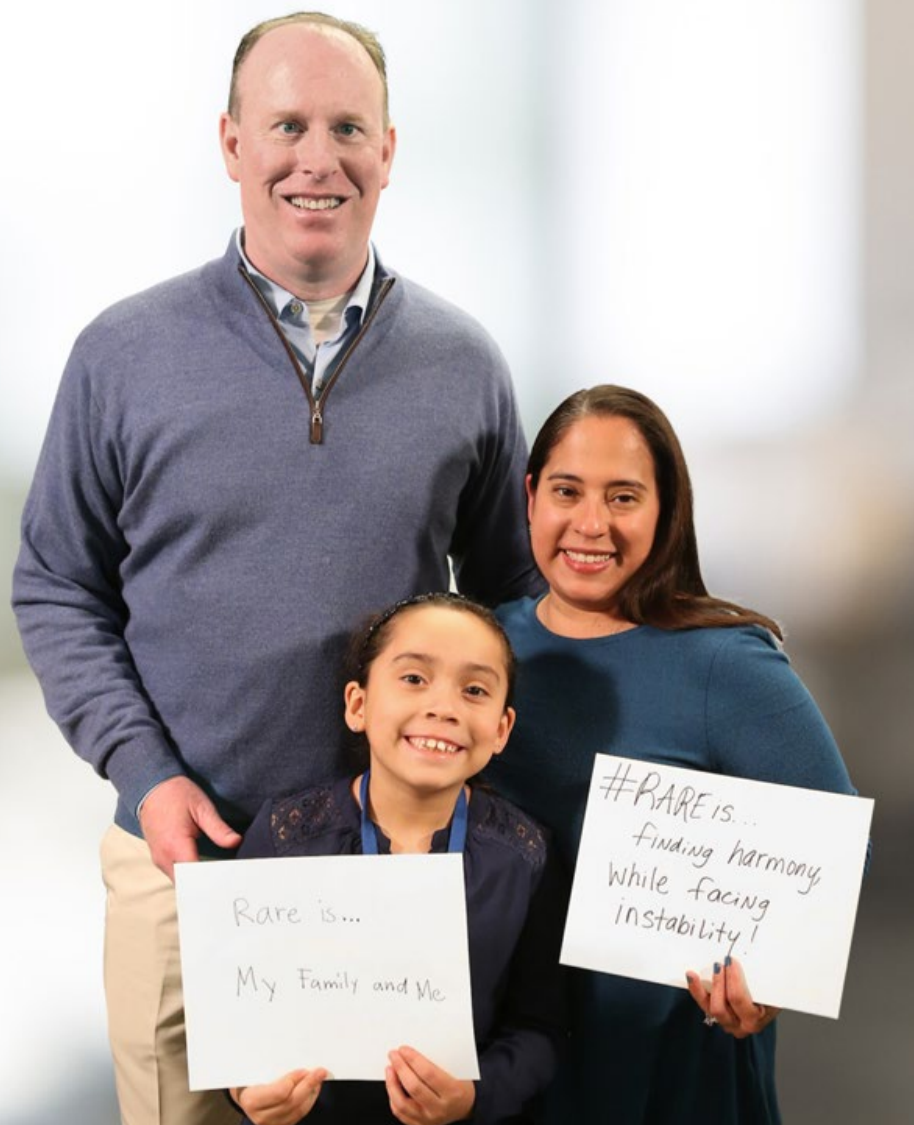
Employees take part in hundreds of advocacy events around the world



# We Create Change in Our Community: Annual Impact







“Going above and beyond for patients is what we do every day. It is at the core of our company culture and reflects my own experience as a patient.”

**Timothy Walbert**

Chairman, President and CEO

Working At Horizon Isn't Just A Job. For Me, It's Personal.





Thank you