



# PTC Therapeutics 2021 Corporate Responsibility

PTC's commitment to ESG focuses on five key areas: our patients, our people, our community, our values and the environment.



# Table of Contents

## **Who We Are**

Our Values & Mission Statement  
PTC Expectations  
Culture Statement  
Awards & Recognition

## **Our Patients**

R&D / Innovation  
Products & Pipeline  
Clinical Trials  
Access to Health Care/Medicine

## **Our People**

Talent Planning  
Global Onboarding  
Benefits & Wellbeing  
Learning & Development  
Workplace/Employee Safety & COVID-19 Response

## **Our Culture & Community**

Commitment  
Global Equality, Diversity & Inclusion  
Global Employee Outreach  
Talent Pipeline Program

## **Ethics & Compliance**

Compliance Program  
Code of Business Conduct & Ethics  
Privacy & Cybersecurity

## **Environment**

Green Operations  
Sustainability Efforts

# Who We Are





As a growing global company operating in over 50 countries, our mission remains focused on bringing more moments to our patients living with rare diseases and their loved ones. Our approach to corporate social responsibility is rooted in our commitment to patient advocacy, access to medicines, and advancing science.

### PTC Culture Statement

Our culture is to make every day count; to care for each other, our community, and for needs of our patients; to be critical, not cynical, of what we do, but not of each other; we strive every day to be better than we were the day before; we want to be part of an important cause and strive to achieve great accomplishments. We are more fearful of not daring to do great things than of failing.

### Awards & Recognition

PTC is proud to have been recognized for our strong culture and scientific innovation

- Gallup CliftonStrengths Award
- PTC Brazil certified as a "Great Place to Work"
- Recognized by EuropaBio as one of the top companies with a scientific breakthrough in last 25 years
- Evrysdi®(risdiplam) received multiple awards that recognize it as one of the most innovative therapies of 2021 and for its life-saving impact
  - 2022 Drug Discovery of the Year award by British Pharmacological Society
  - 2021 Drug Discovery Prize by the Society for Medicines Research and Pharmaceutical Technology Sector Excellence award

### PTC Expectations

How we operate as a #OnePTC company:



# Our Patients





Since our founding in 1998, PTC has concentrated primarily on discovering, developing treatments for patients living with rare disorders utilizing our expertise in RNA biology. As we have grown, our technology platforms have expanded from this foundational scientific expertise to include multiple approaches including gene therapy.

### Our therapeutic areas encompass genetic disorders, oncology, and virology.

**Genetic Disorders.** Most genetic disorders are quite rare and affect one person in every several thousands or millions. We are committed to discovering and developing therapeutics for patients living with genetic disorders and are currently focused in the areas of:

- AADC Deficiency
- Duchenne Muscular Dystrophy
- Huntington's Disease
- Angelman Syndrome
- Familial Chylomicronemia Syndrome (FCS)
- Friedreichs Ataxia
- Hereditary Transthyretin (hATTR) Amyloidosis
- Mitochondrial Disease Associated Seizures
- Phenylketonuria (PKU)
- Spinal Muscular Atrophy (SMA)

**Oncology.** PTC is developing innovative cancer treatments involving enzyme and protein inhibition therapies in these areas:

- Acute Myeloid Leukemia (AML)
- Leiomyosarcoma (LMS)
- Diffuse Intrinsic Pontine Glioma (DIPG)



Hopewell Ribbon Cutting Ceremony



**Virology.** PTC wanted to part of the solution to help the world develop treatments to address the COVID-19 pandemic and looked within its pipeline to determine whether it had potential therapies to treat COVID-19 patients. Emvododstat, a dihydroorotate dehydrogenase (DHODH) inhibitor, was shown to be a potential treatment. This treatment has the potential to be taken outside the hospital setting, thereby reducing the burden coronavirus has placed on health care institutions. Emvododstat is currently in ongoing clinical trials.

### Patient Advocacy

PTC was founded with the mission of discovering, developing and commercializing therapies for diseases with high unmet medical need. Today, we have multiple products on the market that are making a difference in the lives of patients and their families, and many more product candidates in our pipeline.

PTC's commitment to patient advocacy is a foundational hallmark of our company, as we seek to build and sustain rare disease community relationships. Working with patients, families, those affected by rare disease, and non-profit organizations allows our patient relations team to better understand the patient experience and share those insights across PTC throughout the drug discovery and development process.

Today, we have partnerships with over 200 global patient advocacy groups to support patients with rare diseases.



*Hopewell Ribbon Cutting Ceremony*



## Research & Development / Innovation

PTC has made significant investments to expand its research and development programs over the last year. Gene therapy holds tremendous promise for some of the most debilitating and intractable rare, genetic disorders. Last year we commemorated the official opening of our 220,000-square-foot Center of Excellence for gene therapy development, testing and manufacturing in Hopewell, New Jersey. The state-of-the-art research and biologics facility is perfectly equipped to handle process development (PD) and good manufacturing practice (GMP) of plasmids and adeno-associated virus (AAV) vectors, essential to PTC's robust rare disease gene therapy pipeline.

We are proud of our roughly \$20 million investment at the site, in addition to the significant growth we've made to our research space and employee headcount, as part of our commitment to bring transformative new treatments to patients living with rare disease.



*Caption here*



*Caption here*



*Caption here*





# Stats & Figures

220k 220,000 sq ft state-of-the-art research facility to support gene therapy production and foster innovation

XX% Grew our research workforce by XX%

66% 66%\* cumulative investment in R&D

*\* As a percentage of Business Operation Expenses (R&D + SG&A)*



*PTC Therapeutics in Hopewell*