

TRANSLATING SCIENCE Transforming Lives



PTC THERAPEUTICS

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living with rare disorders.

PTC has been committed to rare diseases since inception in 1998. PTC achieved historic scientific breakthroughs and delivered a number of life-changing therapies to patients that address multiple therapeutic areas with high unmet need. We remain focused on scientific research and development efforts to deliver the next wave of innovative PTC products for patients.



+25 YEARS OF COMMITMENT TO PATIENTS

A LEGACY OF INNOVATION

PIONEER IN RARE DISEASE

+20 OFFICES WORLDWIDE

~ 1000 EMPLOYEES

WE ARE COMMITTED TO PROVIDING TREATMENTS FOR PEOPLE LIVING WITH RARE DISEASES

We are focused on therapeutic areas with high unmet medical need and we are at the forefront of transforming science into new therapies. Therapeutic areas include Neurology and Metabolism.

- Aromatic L-amino Acid Decarboxylase (AADC) Deficiency
- Duchenne Muscular Dystrophy (DMD)
- Friedreich's Ataxia (FA)
- Phenylketonuria (PKU)

Our research efforts are focused on two scientific platforms—splicing and inflammation and ferroptosis—where PTC has unique expertise to discover and advance to the clinic innovative therapies.

Splicing

Technology that helps find small molecules that modify alternative splicing of genes. This technology focuses on changing mRNA splicing, which is a process that affects how proteins are made in the body.

Therapeutic areas include Spinal Muscular Atrophy, Huntington's Disease.

Inflammation & Ferroptosis

Targeting oxidative stress and inflammation pathways to treat central nervous system (CNS) diseases

Therapeutic areas include Friedreich's ataxia.

We partner with
+200
patient advocacy
groups globally



WE ARE COMMITTED TO PROVIDING ACCESS TO PTC MEDICINES

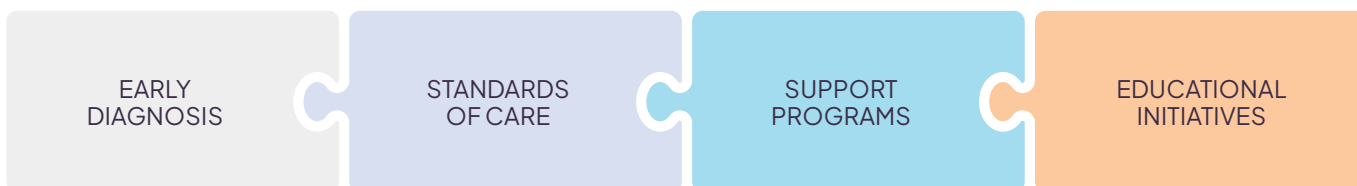
We collaborate with stakeholders to advance access to our therapies across geographies.



Our medicines are reaching patients across **+50** countries

WE ARE COMMITTED TO IMPROVING THE LIVES OF PEOPLE LIVING WITH RARE DISEASES

We partner with and support patients and their families throughout their entire journey.



WE ARE ON A MISSION TO TRANSFORM LIVES



Date of preparation:
February 2025