
measured by moments
PTC THERAPEUTICS™



Our Commitment

Make every day count

At PTC, patients are at the center of everything we do. We have the opportunity to support patients and families living with rare disorders through their journey. We know that every day matters and we are committed to making a difference.

Our Science

Our scientists are finding new ways to regulate biology to control disease

We have several scientific research platforms focused on modulating protein expression within the cell that we believe have the potential to address many rare genetic disorders.

Our People

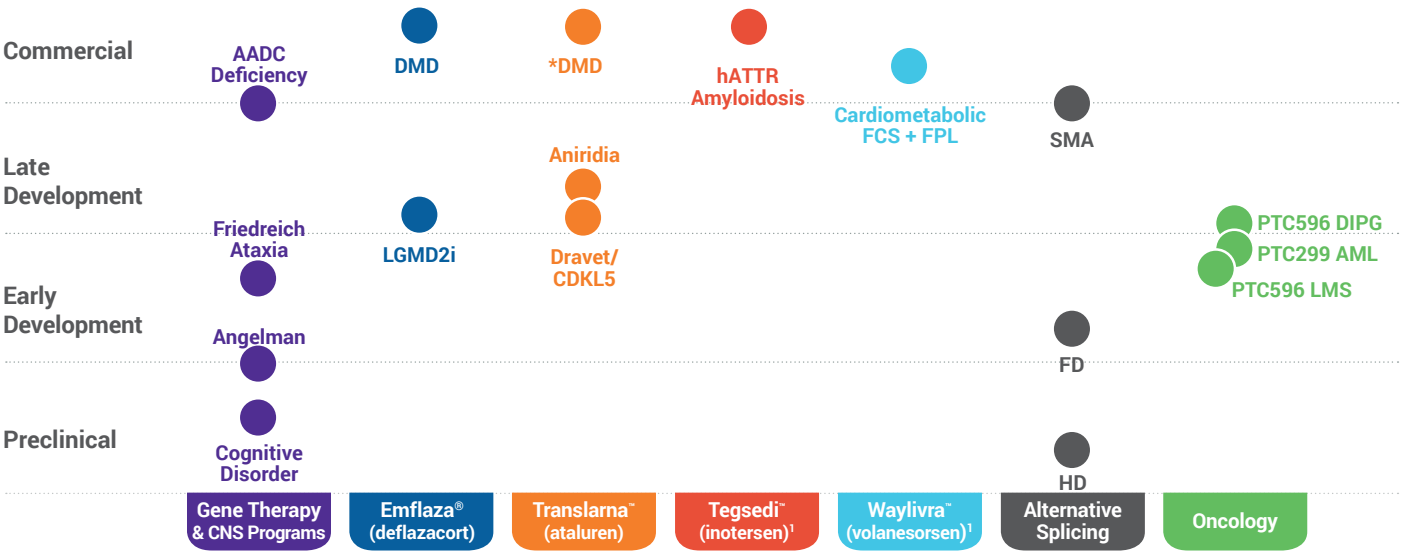
Care for each other, our community, and for the needs of our patients

At PTC, we are looking at drug discovery and development in a whole new light, bringing new technologies and approaches to developing medicines for patients living with rare disorders and cancer. We strive every day to be better than we were the day before.

At PTC Therapeutics, it is our mission to provide access to best-in-class treatments for patients who have an unmet need.

PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically-differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

Our Pipeline



*MA requires annual renewal following reassessment by the European Medicines Agency (EMA), confirmatory study 041 for conditional approval ongoing

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Note: Cardiometabolic diseases due to elevated triglyceride level: FCS = Familial Chylomicronemia Syndrome

A Scientist's Story

ANU BHATTACHARYYA, DIRECTOR OF BIOLOGY, PTC

Just because a collaboration ends doesn't mean a scientist stops thinking about it, and Anu Bhattacharyya couldn't stop thinking about the brain and the diseases that affect it.

She wanted to know more about the mechanisms that lead to neurological disorders, so she invested in an atlas of the brain and studied it to learn more about the organ's different regions and why they break down in some people.

The more she learned, the stronger her passion grew. She started to wonder whether she could harness her passion into something that might help people afflicted with Huntington's disease, a fatal genetic disorder that causes the progressive breakdown of nerve cells in the brain. It deteriorates a person's physical and mental abilities during their prime adult years and has no cure.

"This is a very exciting time for emerging research in the Huntington's disease community, and PTC is committed to following the science to discover and develop novel small molecule treatments for patients with rare diseases," Bhattacharyya said.

She built a business case that she believed would prove that a Huntington's program could fit into PTC's business goals and strategy of innovation, and, eventually, its splicing platform.

"Huntington's disease is a rare genetic disorder so it falls right into PTC's sweet spot," she said. "One appealing feature of Huntington's disease is that it's a monogenic disorder and the key culprit is a single expansion mutation in the huntingtin gene. That makes it an easier problem to tackle; we know the key cause of the disease, so targeting the expression of huntingtin is the most direct and efficient way of targeting Huntington's disease."

"This is a very exciting time for emerging research in the Huntington's disease community, and PTC is committed to following the science to discover and develop novel small molecule treatments for patients with rare diseases."

-Anu Bhattacharyya

With that thought in mind and a business case in hand, she walked into her boss' office to pitch her idea, confident that it was the right thing to do. He agreed.

Go for it, he told her.

She got support from PTC's leadership team even though resources were scarce and the company didn't have a Huntington's program. Combine that endorsement with the unmet medical need and the result is a recipe that led to PTC's Huntington's disease program, which recently announced a research collaboration with the CHDI Foundation.

"When I started to build the rationale for the discovery and development of this program, I didn't know the Huntington's research and patient community," Bhattacharyya said. "Now, our relationships are strong and our discovery program is showing great promise to deliver an oral medication for Huntington's disease. That is what can be accomplished at PTC."

Signature Programs

STRIVE

PTC's STRIVE (Strategies to Realize Innovation, Vision and Empowerment) grant award program for Duchenne provides funds to patient advocacy organizations to develop unique and collaborative programs that will make meaningful contributions to the rare disorder community by increasing awareness, diagnosis, education, or fostering development of future patient advocates.

Since 2015, the program has supported 14 global organizations that are finding innovative ways to improve patient care, like the Little Steps Association, a STRIVE winner. The non-profit organization, founded in Israel by parents of children with Duchenne and Becker muscular dystrophy, used its STRIVE funds for its assistant dog program, Friends for Life.

The program uses trained dogs to help young boys with Duchenne with daily tasks so they can be more independent. The dogs help remove shoes and socks, pull clothes from the wardrobe, and assist with walking up stairs. The feedback from the families to date has been tremendous. Not only do the dogs help lessen some of the burden of Duchenne on the families, they've also become the patient's loyal friend, helping them to feel less isolated because of their condition.

PTC CARES™

With almost 20 years of experience in patient services, Amy Stets has seen just about everything. So, it struck her as odd when a colleague told her about a family's on-going frustration with an insurance company's approval process for a PTC medicine approved in the U.S. for the treatment of Duchenne muscular dystrophy.

"I was concerned that this family was taking on too much of the burden, especially when PTC Cares is designed to shoulder those burdens," Amy said, who is PTC's Director of Patient Services.

Through PTC Cares, a PTC program that is only available in the U.S., Amy and her team help families with the U.S. insurance approval process. They remove financial barriers, when possible, and link families to educational and community resources in the U.S. that can help Americans who live with a son with Duchenne muscular dystrophy.

If the process hits a snag, Amy and her team investigate.

"We are especially committed to the patient part of the equation," Amy said. "That's what makes PTC so special."

'PTC Cares' is a U.S. only program

Amy Stets

PTC Therapeutics in Gene Therapy

PTC is focused on discovering, developing, and commercializing transformative therapies for patients living with rare, genetic disorders. Our desire is to change the course of rare diseases by addressing the underlying causes, not just the symptoms.

For more than 20 years we have been relentlessly pursuing innovative, technologies and strategic partnerships that can help solve some of the most challenging unmet needs of rare disease patients who have limited, or no treatment options. This is driving our vision to be a leading, fully-integrated biotech company with multiple technology platforms.

Gene therapy is one technology that holds tremendous promise for some of the most debilitating and intractable rare, genetic diseases. Genetic diseases are caused when a defective gene stops the body from effectively making a protein critical for the body to function normally. Through gene therapy, we intend to replace or repair the defective gene, creating the possibility of a one-time, life-transforming therapy.

In August 2018, through an acquisition, PTC added a cutting-edge gene therapy pipeline platform focused on rare, neurological disorders, including the most advanced program in development for central nervous system (CNS) gene therapy in the world. Our broad gene therapy pipeline comprises three early and late stage programs for rare disorders, including Aromatic L-Amino Acid Decarboxylase (AADC) Deficiency, Friedreich Ataxia (FA) and Angelman Syndrome.

A major benefit of our gene therapy approach will be the use of targeted micro-dosing directly to specific areas of the CNS involved in the disorder. This should reduce the risk of immunogenicity and of unintended, off-target effects from the inserted gene, maximize efficacy, and durability, and allow for efficient, scalable manufacturing.

PIONEERING CNS GENE THERAPY

Our most advanced gene therapy, PTC-AADC (eladocagene exuparvovec), which addresses a high unmet medical need in AADC Deficiency, is expected to be submitted to the US Food and Drug Administration (FDA) in 2019. AADC Deficiency is a devastating disorder that causes profound neurological and developmental failure at a very young age, caused by a defect in the DDC gene. With deficient AADC enzyme - a critical protein in the brain - the body is unable to produce sufficient dopamine for normal development and function.

PTC-AADC, an adeno-associated virus (AAV)-based gene therapy, is intended to restore the dopamine production

Children with severe AADC Deficiency, who have been treated with a single dose of PTC-AADC, have demonstrated sustained, clinically meaningful, and statistically significant improvement in their physical ability. They also have achieved developmental milestones not normally achieved in the normal course of AADC Deficiency, including head control, sitting and standing. To date, 26 patients have been treated since 2010.

Our gene therapy pipeline also includes the most advanced program in development for the underlying cause of FA, a devastating, neurodegenerative disease that can progressively rob patients of their ability to walk, speak, see and hear. FA is caused by a single defect in the FXN gene, which results in the loss of frataxin protein. Frataxin is critical for the function of many proteins, particularly those involved in energy supply to cells.

Preclinical results in animal models have shown very promising increases of frataxin in the brain with our AAV-based gene therapy, PTC-FA. Phase I/II clinical trials are due to commence in 2019 with a view to filing for FDA approval in 2019.

Additionally, we will be advancing PTC-AS into clinical trials in the next few years. PTC-AS targets Angelman Syndrome, a rare, genetic, neurodevelopmental disorder characterized by severe intellectual and developmental delays.

Rapid, scientific innovation is critical for patients with rare diseases. As a leader in the rare disease gene therapy space, we are in a position to optimally treat more rare disorders and accelerate the process of bringing life-transforming therapies to patients worldwide.

Everyone has a different definition of progress. For more than two decades, we've measured our progress researching rare disease in moments. Smiling ones and crying ones. Moments spent with our boys' families and ones with their friends. We know that every step forward comes after several steps backward, because we've lived it—whether spending time with families in their homes or with our scientists researching in our labs.

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