## PTC'S GENE THERAPY CENTER OF EXCELLENCE

Gene therapy holds tremendous promise for some of the most debilitating and intractable rare, genetic disorders. PTC's 220,000 square foot state-of-the-art Center of Excellence for gene therapy development, testing and manufacturing in Hopewell, New Jersey, 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.

220,000

square foot state-of-the-art research and biologics facility to support gene therapy production and foster innovation The Hopewell team is part of PTC's global team of more than

1,200

## talented and dedicated employees

working to bring transformative new treatments to those living with rare disorders.

## MANUFACTURING ACTIVITIES

Through the Gene Therapy Center of Excellence, PTC offers world class plasmid DNA and AAV manufacturing facilities, services and technology to valued partners.





**Process Development** 



**Analytical Development** 



**Clinical Phase AAV Gene Therapy Manufacturing** 



**Drug Product Manufacturing** 



**Plasmid Manufacturing** 



**Quality Control** 



**Quality Assurance** 

## GENE THERAPY PRODUCTION AT PTC



PTC has a robust gene therapy pipeline for central nervous system disorders. The company's lead indication is in aromatic L-amino acid decarboxylase (AADC) deficiency, an ultrarare, highly morbid and often fatal genetic neurotransmitter disorder, as well as other preclinical programs in Friedreich Ataxia (FA) and Angelman syndrome, among others.

PTC's in-house manufacturing and R&D capabilities ensure maximum control over quality, capacity and supply, and seamless transition from clinical to commercial development.

PTC is at the forefront of a new and transformative era of gene therapy. We are committed to the development and manufacturing of gene therapies so that these transformative treatments can get to patients as quickly and efficiently as possible.

