

In partnership with



ST. JOHN'S
UNIVERSITY

College of Pharmacy
and Health Sciences

PTC Therapeutics, Inc. - Pharmaceutical Industry

PharmD Fellowship Program

Global Regulatory Affairs | 2023-2025



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MESSAGE FROM OUR CEO

When I founded PTC nearly 25 years ago, I wanted our ideal work environment to be one where great people with great minds collaborate to accomplish big and hard goals. Where employees are as much family as they are colleagues, where nothing is taken at face value and rigorous debate pushes us to be more innovative. Every day, I consider it a privilege to work alongside such talented and committed people. We are dedicated to using groundbreaking science and new technologies to find innovative ways to treat rare diseases so that patients and their families have more moments together.

Stuart W. Peltz, Ph.D.
Founder and Chief Executive Officer
PTC Therapeutics, Inc.

RARE DISEASES AT PTC

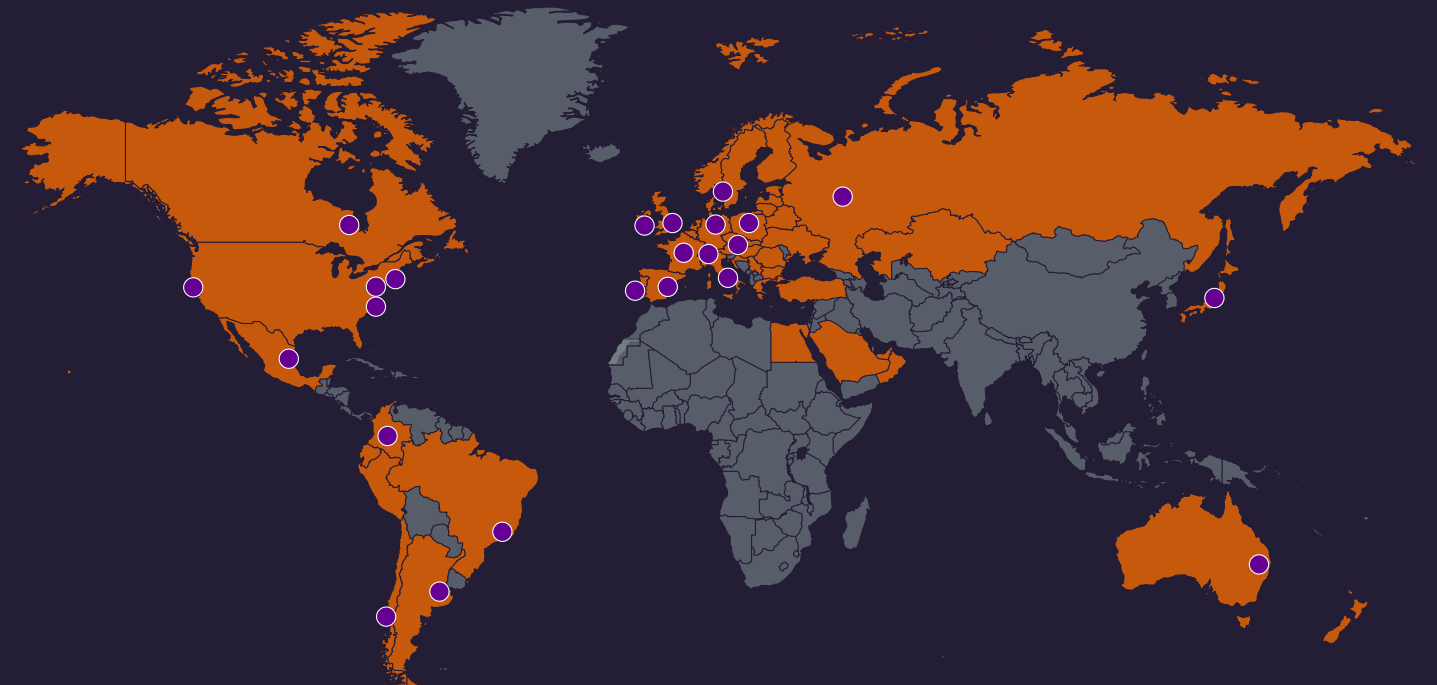
Who We Are

MISSION

Our mission is to provide access to best-in-class treatments for rare disease patients. This mission underpins our focus on the discovery, development and global commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. As a science-driven, global biopharmaceutical company, we combine our strong clinical and scientific expertise with dedication to using groundbreaking science and new technologies to find innovative ways to treat these diseases, which can give patients and their families more moments.

GLOBAL FOOTPRINT

Since 1998, PTC has been a biopharmaceutical company focused on innovative small molecule and gene therapies for rare genetic disorders. We operate offices and/or research labs in more than 20 countries and occupy a business footprint in more than 50 countries around the world. We also enjoy strong commercial performance and a robust capital position.



OUR PEOPLE

From our beginnings 24 years ago, and as a founder-led startup in New Jersey, we have grown to be a global company with more than 1,300 employees. We take great pride in our corporate culture. Our employees share our sense of purpose and our goal of bringing life-changing therapies to patients in need. We support the growth of our employees through regular use of the Gallup® Q12 survey to measure employee engagement and other company-wide analytic and coaching tools to assess each employee's strengths.

The Employee Resource Groups meet regularly and interact with our CEO and Executive Committee. We also host monthly programs to accentuate our diverse culture, each managed by a member of the Executive Committee.

We have built a strong talent management program for leadership training throughout the organization. Our programs support employees with training and mentorship to become the next generation of company leaders.

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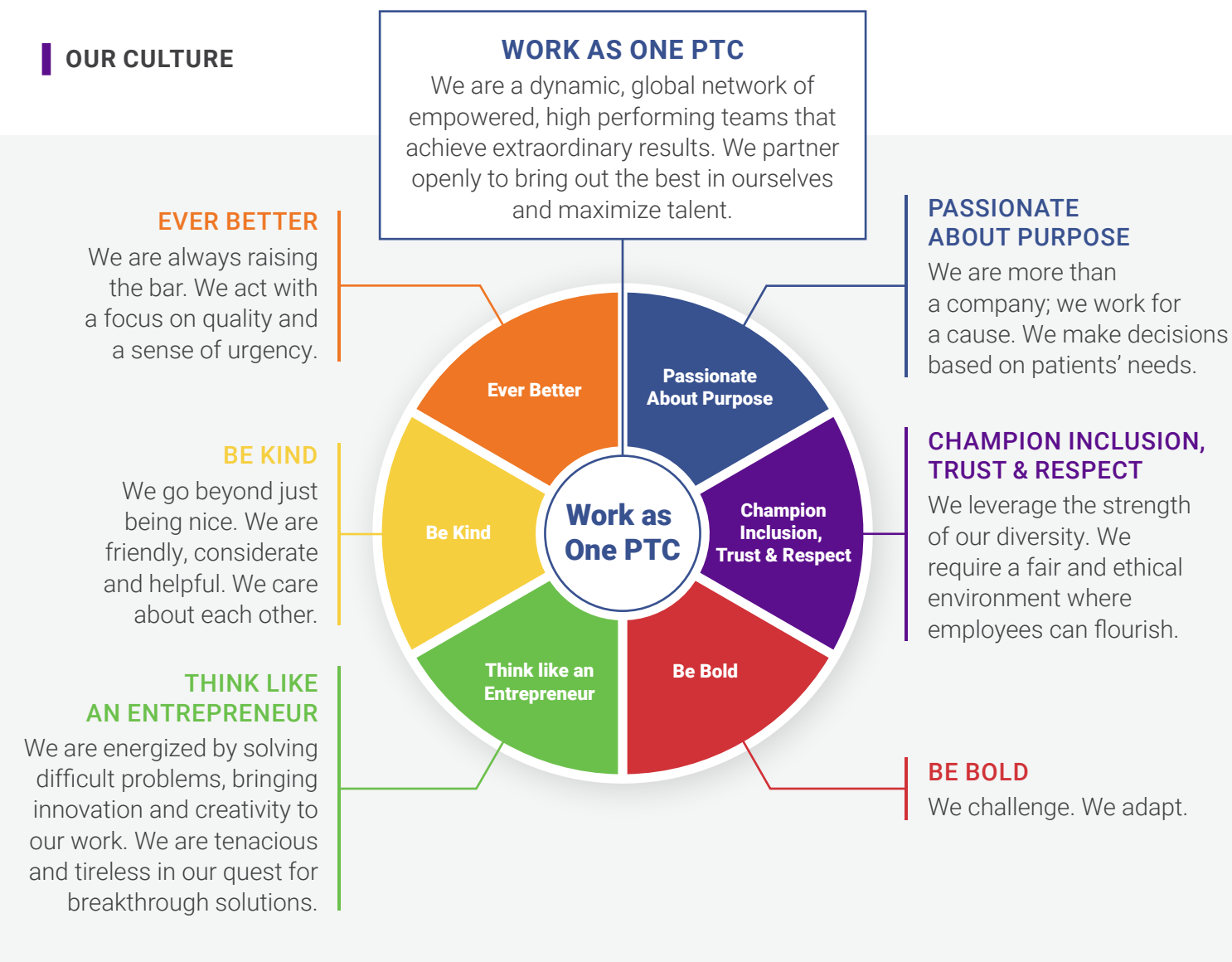
We always consider hiring the best person or team member for a role. I get inspired when we have the opportunity to partner and create new programs where we can bring in phenomenal talent from Colleges and Universities and develop them to peak levels of performance, as they become emerging talent and great future leaders within our company. We now have a phenomenal opportunity to partner with St. John's University faculty and PharmD students to participate in a Regulatory Affairs Fellowship Program. This is a win/win situation for the students, University and PTC Therapeutics!



Everette Brown

Executive Director, Global Talent Acquisition & Strategy

OUR CULTURE



BY THE NUMBERS



Investing in Our People



Clifton Strengths

Employee Resource Groups

Individual Development Plan

Herrmann Brain Dominance Instrument

What We Do

THE SCIENCE

We commit ourselves to pushing the envelope of what is possible. We seek to expand our pipeline through our innovative technology platforms and through strategic collaborations. Our mission is to bring innovative treatments to patients so that they can enjoy more moments with the ones they love.

OUR DIFFERENT MOAs

Nonsense Readthrough	Alternative Splicing
Transcript Regulators	Bio-e Electron Transfer
Gene Therapy	

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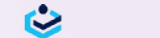







For over the past 25 years, PTC has been dedicated to using pioneering science to develop transformative therapies for patients with rare disorders worldwide. We have a robust pipeline built on a number of platforms, including small molecules and biologics.

We look forward to your joining our passionate, purpose-driven, and entrepreneurial team and helping to make a difference for patients in need of effective therapies.”

Matthew Klein, MD, MS, FACS
Chief Operating Officer



SCIENTIFIC PLATFORMS and RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology	Virology
<div><div>COMMERCIAL</div></div> <div><div><div>Emflaza[®] (deflazacort) 8 mg / 15 mg / 30 mg / 36 mg tablets 22.75 mg/mL oral suspension</div></div><div><div><div>Tegsedi[®] (inotersen) injection 250 mg/15 mL</div></div><div><div><div>waylivra[®] (volanesorsen) injection 100 mg/15 mL</div></div><div><div><div>translarna[™] ataluren</div></div><div><div><div>Evrysdi[®] risdiplam</div></div><div><div><div>Upstaza[™] (eladocagene exuparvovec)</div></div></div></div></div></div></div></div>									
<div><div>CLINICAL</div></div> <div></div> <div></div> <div></div> <div>US Ataluren</div> <div></div> <div>PTC518 HD</div> <div></div> <div>Vatiquinone MDAS</div> <div>Vatiquinone FA</div> <div>PTC857 ALS</div> <div>PTC923 PKU</div> <div></div> <div>Unesbulint DIPG</div> <div>Unesbulin LMS</div> <div>Emvododstat AML</div> <div>Emvododstat COVID-19</div>									
<div><div>RESEARCH</div></div> <div></div> <div></div> <div>2 Undisclosed</div> <div>SCA-3</div> <div>MAP-Tau</div> <div>8 Undisclosed</div> <div>FA</div> <div>Angelman</div> <div>IRDs</div> <div>IRDs</div> <div>3 Undisclosed</div> <div></div> <div>3 Undisclosed</div> <div></div>									

IAADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich’s ataxia; ALS, amyotrophic lateral sclerosis; HD, Huntington’s disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.
*Upstaza is currently in development in the US

Potential registrational studies

Early-stage programs

Why We Do it

OUR PATIENT FOCUS

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.

We continue to invest both internally and externally in cutting-edge research programs to search for treatments for patients suffering from diseases with little to no treatment options. PTC is also supporting external research programs for next generation rare disease therapies.

PTC is committed to working with and supporting patient advocacy groups, which are incredibly important assets to rare disease patients and their families. We have forged approximately 200 relationships with patient advocacy groups around the world, and we have committed significant funding to support patients through their patient advocacy groups with our STRIVE Awards and various unsolicited grant programs.



We provide resources and support to patients and their families throughout the life of their journey with rare disease. We work with advocacy groups around the world, raising awareness and addressing the needs of the patient communities PTC serves.

Through collaboration and partnership, we provide information, education, and support to empower individuals and their families affected by rare disease. Developing relationships among these patient, patient family and caregiver groups is vital throughout the drug discovery and development process, in order that we ensure patient benefit.

GLOBAL REGULATORY AFFAIRS FELLOWSHIP INFORMATION

Actively Recruiting: 1 Fellow 2023-2025

PTC is offering a 2-year PharmD Fellowship in Global Regulatory Affairs (GRA) - Strategy. This position will be remote full-time.

YEAR 1

An opportunity to gain specialized and in-depth training in Regulatory Affairs over a period of 12 months.

Rotate through:

- Regulatory Strategy
- International Regulatory Affairs
- Regulatory - CMC
- Regulatory Operations and Writing

YEAR 2

The remaining 12 months of the program will be spent on an elective.

Electives will last approximately three months and will be customized based on the fellow's interests and company business needs.

Elective options include, but are not limited to:

Internal

- Clinical Pharmacology
- Translational Medicine
- Clinical Development
- Clinical Operations
- Pharmacovigilance
- Project Management
- Regulatory Affairs
- Statistics

External

- Food and Drug Administration



Overall, the structured program will provide a cumulative set of skills that will help facilitate a smooth transition to a successful career after the fellowship. Fellows will establish a strong foundation of experience and knowledge to lead a successful career within the pharmaceutical industry, Food and Drug Administration (FDA), or Contract Research Organizations (CROs)

Fellowship Objectives

- Serve as the primary liaison between PTC, CROs, and Health Authorities worldwide (e.g., the US FDA)
- Provide strategic input and tactical support to expedite development, submissions, and regulatory approvals
- Participate in the development of global regulatory strategies supporting development, approval, and maintenance of drugs and biologics
- Participate in Health Authority (HA) interactions and assess impact of HA feedback
- Submit and maintain regulatory applications including INDs, CTAs, NDA/BLAs
- Work with matrix team members (R&D and Non-R&D) to identify solutions that meet regulatory requirements as well as commercial objectives
- Provide support to the life-cycle management activities

Meet the Global Regulatory Affairs Leadership Team

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The PharmD Industry Fellowship program at PTC comes at one of the most exciting times in our history. Together with our passionate focus on Rare Diseases and our cutting-edge pipeline in gene and small molecule splicing technology, we have a tremendous opportunity to provide our fellows with in-depth and broad range of regulatory experiences to meet the future needs of patients in a complex and ever-evolving healthcare environment.

Our commitment to the fellowship program is to train the regulatory leaders of tomorrow by providing a solid drug development foundation with the skills necessary to make impactful decisions, provide strategic regulatory insights and finding creative solutions to challenging issues to be “ever better” in all we do.”



Doreen V. Morgan
PharmD, M.S. Vice President,
Head of Global Regulatory Strategy,
Preceptor-Program Director



Murad Husain
SVP, Global Regulatory Affairs

“

Our Global Regulatory Affairs group is thrilled to welcome PharmD fellows. Every moment of our time here at PTC is aimed at extending life's moments for patients with rare disease and their loved ones. The PTC GRA PharmD Fellow will have the unique opportunity to develop an understanding of regulatory strategy in parallel with helping shape the future of rare disease treatments.

St. John's University College of Pharmacy and Health Sciences provides undergraduate, graduate, and postdoctoral programs focused on global impact, which cultivates the tools necessary to find solutions to complicated health care issues. Located in Queens, New York, St. John's commitment to excellence and renowned faculty inspire students to practice patient-centered care and shape the future of health care. St. John's University will empower diverse learners with quality education for life. Through innovative teaching, research, and service we will foster rational, spirited inquiry and intelligent reflection.

Our student-centered approach will be shaped by a caring, energized, nimble culture. Enlivened by our distinctive mission, our graduates will excel in the competencies and values required for leadership and service in a rapidly evolving global community. As a Catholic and Vincentian university, we will be known worldwide for addressing issues of poverty and social justice.



Application Process

ELIGIBILITY:

Potential applicants for the St. John's University/ PTC Therapeutics program must meet the following criteria:

- Be a graduate of an Accreditation Council for Pharmacy Education (ACPE) accredited Doctor of Pharmacy Program before beginning this fellowship in July 2023
- Possess a genuine interest in a career in the pharmaceutical industry
- Be eligible to work in the United States

HOW TO APPLY:

Please electronically send all required items and correspondence to:

stjfellowships@stjohns.edu

- Deadline of October 21, 2022, for Letter of Intent and Curriculum Vitae (CV)
- Deadline of October 28, 2022, for Three Letters of Recommendation and College Transcript

SCHEDULING AN INTERVIEW:

Virtual Interviews will be provided on a rolling basis upon submission of a Letter of Intent and CV beginning October 5th, 2022.



FOR ANY ADDITIONAL INFORMATION OR QUESTIONS:

Visit: www.stjohns.edu/resources/places/pharmaceutical-industry-fellowship-programs

Email: stjfellowships@stjohns.edu

FELLOWS AT ST. JOHN'S

The fellow will be a full-time St. John's University employee. A competitive stipend will be provided, as well as options for comprehensive health, dental, and vision insurance. Each fellow is responsible for completing semester-long teaching requirements at the University and an additional fellowship research project that may include original research, or the development, enhancement, or evaluation of some aspect of operations or patient care services.

UPON FELLOWSHIP COMPLETION

The fellow will receive a Certificate of Completion.

ADDITIONAL FELLOWSHIP OPPORTUNITIES

- The fellow may apply for an affiliate faculty appointment within St. John's University College of Pharmacy and Health Sciences
- Opportunity to teach on campus and receive a teaching certificate
- Precept St. John's University Doctor of Pharmacy Candidates
- Perform collaborative research with St. John's faculty
- Enroll with tuition remittance for Graduate Degree programs (Master's or PhD degrees)