

PTC Therapeutics, Inc. Pharmaceutical Industry

PharmD Fellowship Program

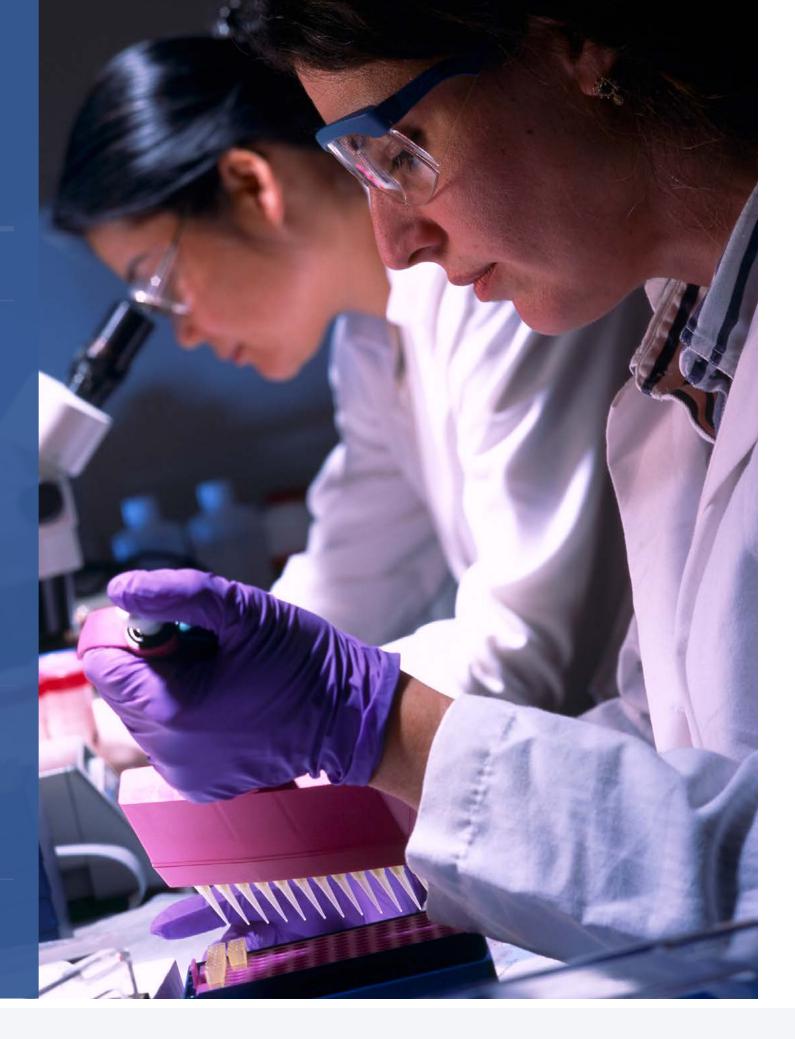
Global Regulatory Affairs

2024-2026



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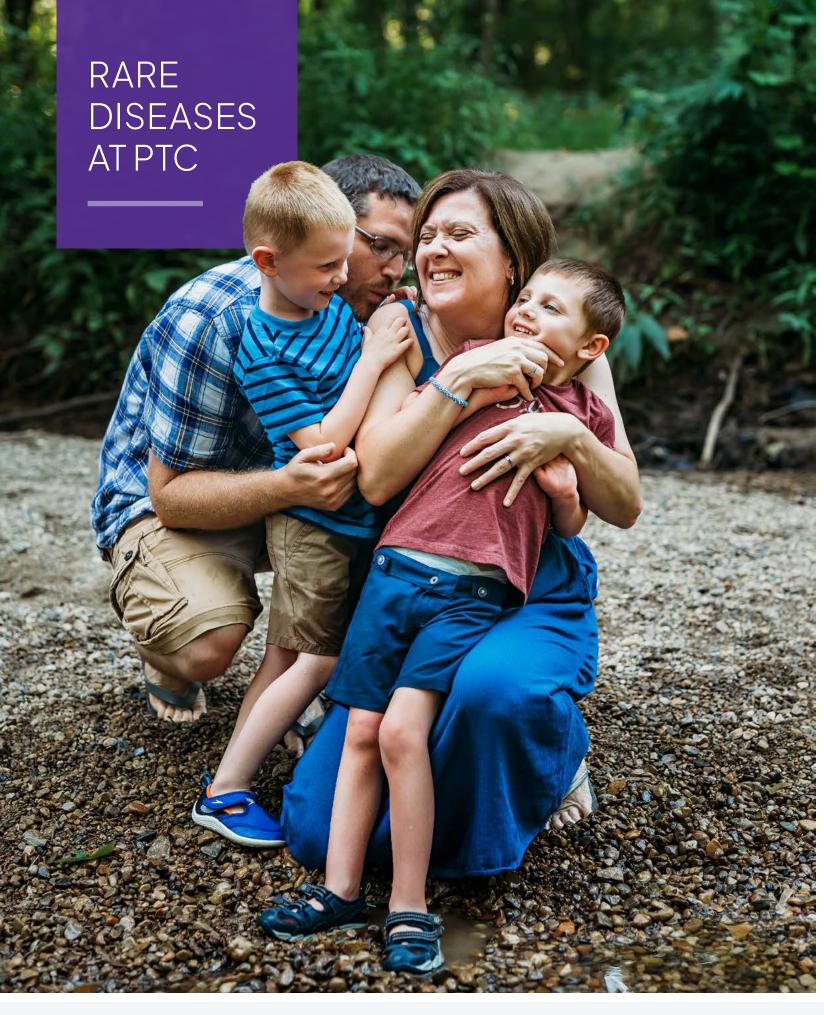




# MESSAGE FROM OUR CEO

It is an incredibly exciting time to be at PTC where we strive to achieve the impossible through our unwavering commitment to deliver transformative therapies to patients living with rare diseases. Our passion for our purpose drives us to think differently about solutions and work collaboratively as One PTC. For 25 years, we have been harnessing our scientific platforms, drug development expertise and robust commercial infrastructure to transform lives and deliver more moments that matter for patients and their families around the world.

Matthew B. Klein, MD, Chief Executive Officer **PTC Therapeutics** 



#### Who We Are

#### MISSION

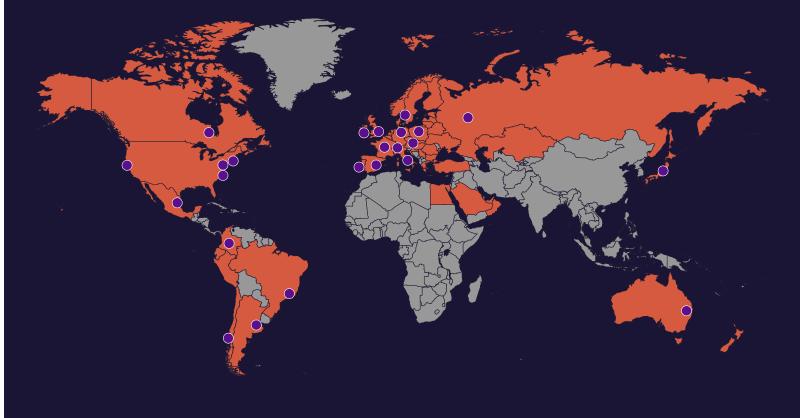
PTC is committed to providing access to best-in-class treatments for patients with little to no treatment options.

This mission underpins our focus on the discovery, development, and global commercialization of clinically differentiated medicines for those with rare diseases.

As a science-driven, global pharmaceutical company, we combine clinical and scientific expertise with groundbreaking science and technology. We work hard to provide broad access to our life-changing treatments. What drives us is our passion to provide patients with more moments with their families.

#### **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 25 years ago, and as a founder-led startup in New Jersey, we have grown to be a global company with over 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 ED&I 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.

# 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, MBA Executive Director, Global Talent Acquisition & Strategy

# OUR CULTURE WORK AS ONE PTC We are a dynamic, global network of empowered, high performing teams that

achieve extraordinary results. We partner

openly to bring out the best in ourselves

and maximize talent.

Work as

**One PTC** 

**Ever Better** 

Think like an

Entrepreneur

Be Kind

**Passionate** 

**About Purpose** 

Be Bold

**Trust & Respect** 

#### EVER BETTER |

We are always raising the bar. We act with a focus on quality and a sense of urgency.

#### **BEKIND**

We go beyond just being nice. We are friendly, considerate and helpful. We care about each other.

# THINK LIKE AN ENTREPRENEUR

We are energized by solving difficult problems, bringing innovation and creativity to our work. We are tenacious and tireless in our quest for breakthrough solutions.

#### PASSIONATE ABOUT PURPOSE

We are more than a company; we work for a cause. We make decisions based on patients' needs.

# CHAMPION INCLUSION, TRUST & RESPECT

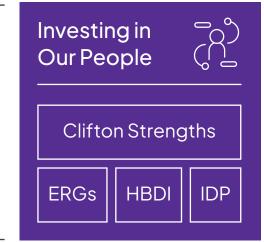
We leverage the strength of our diversity. We require a fair and ethical environment where employees can flourish.

#### BEBOLD

We challenge. We adapt.

#### BY THE NUMBERS





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#### What We Do

#### OUR SCIENCE

We have been a pioneer in the development of breakthrough rare disease therapies, delivering a number of firsts for patients who previously had no other treatment options. We're using our biotechnology platforms and expertise in translating science to target the underlying causes of rare diseases, with an ambitious goal of developing at least one new, life-changing therapy every two to three years.



Nonsense Supression

Alternative Splicing

Bio-e Electron Transfer



For over 25 years PTC has been at the forefront of pioneering science to develop novel therapeutics for rare diseases with high unmet medical need. With a growing pipeline of innovative drug candidates developed from a core set of scientific platforms, PTC looks forward to bringing hope to a whole new generation of patients.



We look forward to you joining this great team of passionate professionals with an amazing can do spirit who transform the lives of patients.

Lee Golden, MD Chief Medical Officer

#### **OUR PIPELINE**

Commercial >















Neurology >



Metabolism >



Oncology >

### Research

MAP-Tau

SCA-3

10 Undisclosed

 $2\, Undisclosed$ 

3 Undisclosed

## **Development**

PTC518 HD

Vatiquinone FA

Utreloxastat ALS

Sepiapterin PKU

Unesbulin LMS

Unesbulin DIPG

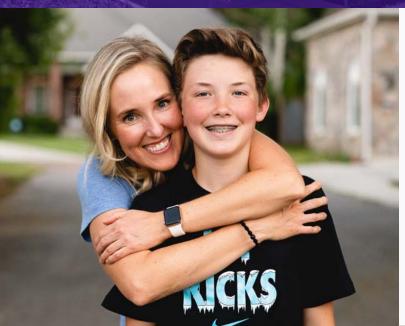
#### **Our Commitment**

PATIENTS ARE OUR PRIORITY

Over the last 25 years, everything we've done, every study, every experiment, every investment is made first and foremost to change the course of rare diseases for the lives of our patients.







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.

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.



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

To learn more about PTC, please find us here:

www.ptcbio.com







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# GLOBAL REGULATORY AFFAIRS **FELLOWSHIP INFORMATION**

**Actively Recruiting: 1 Fellow 2024-2026** 

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



#### YEAR1

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 3 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**

- Support the primary liaison between PTC, CROs, and Health Authorities worldwide (e.g. the US Food and Drug Administration)
- Provide strategic input and tactical support to expedite the development, submission and regulatory approval
- Participate in the development of global regulatory strategies supporting development, approval, and maintenance of drugs and biologics

- Participate in Health Authority (HA) interactions and assessing 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

## **Resources for Development and Support**

- Connect with PTC leadership
- Open-door culture and networking opportunities
- Strong mentorship
- Monthly learning and development workshops

- External industry conferences
- Soft-skills development
- ERGs (Employee Resource Groups)
- Personal development (CliftonStrengths) and psychometric assessments (HBDI)
- Wellbeing and selfcare sessions

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## Meet the Global Regulatory Affairs (GRA) Leadership Team



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



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 such as, PTC's 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.



Murad Husain, RPh 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.

#### CURRENT GLOBAL REGULATORY AFFAIRS FELLOW

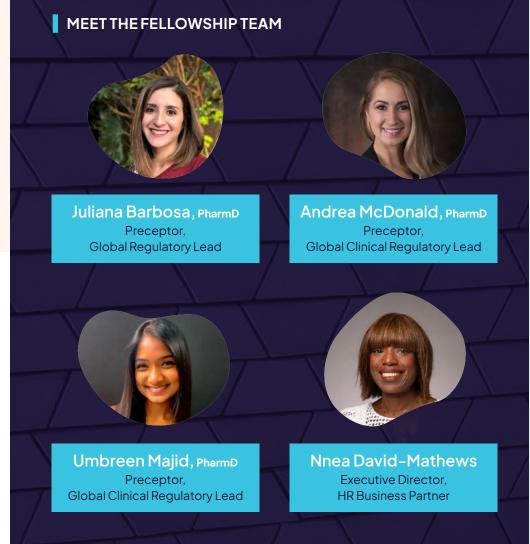


Tarik Brakta, PharmD SJU Fellow, Global Regulatory Affairs



This Global Regulatory Affairs (GRA) fellowship has allowed me to embark on a transformative journey. It has given me the tools to confidently navigate both the national and international regulatory spaces. I am learning how to contribute towards the approval process of new drugs worldwide and help make a difference for patients with rare diseases. This fellowship is guiding me toward a future where I thrive in regulatory excellence, leading the way for innovative therapies that can potentially transform the lives of many throughout the world.





The PTC Global Regulatory Affairs Fellowship team is dedicated to fostering the professional growth and success of fellows as they embark on their two-year journey. From structured learning opportunities and tailored mentorship to hands-on project collaboration the GRA Fellowship team is committed to nurturing a rich environment where fellows can thrive, learn, and contribute meaningfully to innovative research and innovation.

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## St. John's University

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.

#### 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)



# **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 2024
- 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:

# Dr. William M. Maidhof: stjfellowships@stjohns.edu

- Deadline of October 20<sup>th</sup>, 2023, for Letter of Intent and Curriculum Vitae (CV)
- Deadline of October 27<sup>th</sup>, 2023, for Three Letters of Recommendation and College Transcript

#### SCHEDULING AN INTERVIEW:

Virtual Interviews will be provided upon submission of a Letter of Intent and CV beginning October 24th, 2023.

#### FOR ANY ADDITIONAL INFORMATION OR QUESTIONS:

Visit: <a href="www.stjohns.edu/resources/places/pharmaceutical-industry-fellowship-programs">www.stjohns.edu/resources/places/pharmaceutical-industry-fellowship-programs</a>
Email: <a href="mailto:stjfellowships@stjohns.edu">stjfellowships@stjohns.edu</a>

#### Alliance of Industry Fellowship Associates Fellowship Offers

Recognizing that the choice of a Post-Doctoral Industry Fellowship is an important decision, AIFA exists to promote a common aspect of each of our program's cultures by supporting a consensus first offer date of December 13, 2023 for all fellowship candidates.



We hope that other academic and non-academic Fellowship Programs will respect this timeline to allow for best program fit for candidates.

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