

**Pannie (Panayiota) Trifillis, PhD**  
Global Medical and Scientific Affairs Executive  
(+1 908-249-2760 – [LinkedIn](#), [Email](#))

*I am passionate about discovering, developing and commercializing innovative therapies for rare genetic diseases with a high unmet medical need, and providing access to treatment for every patient in need.*

*In my career, I am thrilled to have launched five rare disease drugs and to have brought two of these drugs all the way, from a concept idea to the patients living with Duchenne Muscular Dystrophy and Spinal Muscular Atrophy.*



I have been dedicated to this mission over the past 25+ years. As the first scientist and employee to join PTC Therapeutics, Inc.'s founding team, I have been fortunate to have my contributions evolve to serve the needs of the company's growth. During my 25-year career at PTC, I have been entrusted to serve in diverse key roles with increasing responsibilities in Research & Development, Strategic Alliance Management and Global Scientific and Medical Affairs. In 2013, standing on the NASDAQ exchange floor witnessing the PTC team's commitment over the previous 15 years, culminating in its IPO, was a humbling moment and a reminder that the work of having our drugs approved and made accessible globally to all patients in need was the next challenge to embrace. That same challenge continues to motivate me to this day; to reach all patients in need of rare disease drugs. Knowing that patients and their families are benefiting from the drugs that I helped bring to them, has been the most rewarding experience in my life thus far.

My proudest contributions to the rare disease community are shepherding not one but two concept ideas and transforming them into first-in-class and best-in-class approved drugs, Translarna™ (ataluren) and Evrysdi® (risdiplam) in 2014 and then again in 2020 for patients with rare diseases. As the first scientist at PTC, I initiated the program that led to the discovery and development of Translarna™ (ataluren), the first approved, oral, innovative, disease-modifying treatment for nonsense mutation Duchenne Muscular Dystrophy (nmDMD), a fatal, rare, progressive, genetic disease.

I subsequently created aperture to market for PTC by establishing and leading alliance management and executing on collaborative partnerships in diverse therapeutic areas including neuromuscular, oncology, central nervous system, cardiovascular and antibacterial diseases while working with partners

in large pharma, biotech and non-profit organizations. In 2020, after 14 years of cumulative efforts in a unique, 3-way collaboration among a non-profit patient advocacy group, the Spinal Muscular Atrophy

Foundation (SMAF), a large pharma, Roche, and PTC Therapeutics, Evrysdi® (risdiplam), an oral, innovative, best-in-class splicing modifier therapy for patients with SMA was approved by the FDA.

As the company looked to increase global awareness among health care professionals and patient advocacy organizations, I shepherded a global outreach and advocacy campaign that led to the successful marketing authorization of Translarna™ by the European Medicines Agency in August of 2014 as well as additional approvals around the world since then. These approvals have granted access to this breakthrough personalized medicine to eligible patients in the European Member States and Iceland, Liechtenstein, Norway, Great Britain, Northern Ireland, Kazakhstan, Israel, Republic of Korea, Belarus, Russia, Brazil, Chile, the Kingdom of Saudi Arabia, and Ukraine most recently.

In the last 5 years, I served as the Vice President and Global Head of Scientific Affairs at PTC where I set the strategy for the company's four functional pillars in Medical Information, Medical Communications, Scientific Publications and Medical Education & Training working cross-functionally within Global Medical Affairs and across the company. In my medical affairs career thus far, I have launched five drugs, namely Translarna™ for nonsense mutation Duchenne Muscular Dystrophy (DMD) ex-US, Emflaza® for DMD in the US, and in Latin America, I launched Tegsedi® for hereditary transthyretin-mediated (hATTR) amyloidosis and Waylivra® for Familial Chylomicronemia Syndrome (FCS) and Familial Partial Lipodystrophy (FPL). Finally in 2022, I was instrumental in a 5th drug launch of Upstaza™ (eladocogene exuparvovec) in Europe, the first ever gene therapy administered intraputaminally in patients with Aromatic L-amino Acid Decarboxylase Deficiency (AADCD) through stereotactic surgery. My tireless efforts and long-lasting relationships working with health care professionals globally have helped countless rare disease patients around the world receive these rare disease drugs and have given a glimpse of hope to them and their families.

In the last few months, I serve as the Senior Vice President, Medical Affairs and Scientific Communications at Rapid Commercialization Partners, a consulting firm focused on optimizing commercialization for clinical-stage biotechnology companies to prepare for future commercial success by building the foundation necessary for growth. Leveraging my unique experience in launching five rare disease drugs and bringing two of these from the bench all the way to the patients as well as my

diverse background in Research & Development, Strategic Alliance Management and Global Scientific and Medical Affairs, I am well poised to provide deep and insightful advice for the successful commercialization of rare disease drugs.

I hold a PhD in Molecular Biology and Human Genetics from the University of Pennsylvania. I am an author of a plethora of peer-reviewed scientific publications and a patent holder, an accomplished speaker in the global arena, and a Fulbright Scholar, fluent in English and Greek. I also hold a Certificate of Achievement in Alliance Management (CAAM) from the Association of Strategic Alliance Professionals. My deep and broad scientific expertise, relationship building skills, positivity and the drive to be resilient and passionate about what I do, have fueled my quest to make a difference in patients' lives with rare genetic diseases and their families.

As part of my volunteer work, I serve as the Vice President and Secretary of the Association of Greek American Professional Women (AGAPW), a charitable and educational organization established in New York City that seeks to expand career opportunities and promote community and leadership-building among Greek American professional women. I also serve on the Board of the Cyprus Children's Fund whose mission is to provide help not only to generationally-affected refugees and enclaved children, but also to children that come from families struggling with economic and medical challenges in Cyprus.

I was born in Famagusta, Cyprus and came to the US as a Fulbright Scholar to attend Wellesley College, MA for my undergraduate studies and then the University of Pennsylvania, PA for my doctoral studies. I am a dual citizen. I am married and a mother of 2 and I reside in New Jersey.