Pannie (Panayiota) Trifillis, PhD

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(Email, LinkedIn, 2024 Marquis Who is Who)

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

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 lab concept idea through clinical trials to the patients living with Duchenne Muscular Dystrophy and Spinal Muscular Atrophy.

I have been dedicated to this mission for over 3 decades. As the first scientist and employee to join PTC Therapeutics, Inc., I have been fortunate to have my contributions evolve to serve the needs of the company. Over my 25-year career at PTC, I was entrusted to serve in diverse key roles with increasing responsibilities across Research & Development, Strategic Alliance Management and Global Scientific and Medical Affairs. In 2013, while standing on the NASDAQ floor as our team celebrated PTC's IPO, it was an emotional moment and a reminder for me that the next challenge was achieving access to our therapies for patients globally. Knowing that patients and families benefit from the medicines I helped discover and develop has been the most rewarding experience of my life.

My proudest contributions to the rare disease community are shepherding not one, but two innovative ideas into first-in-class, best-in-class approved medicines: Translarna™ (ataluren) and Evrysdi® (risdiplam) for patients with rare diseases. As PTC's first scientist, I launched the program that led to the discovery and development of Translarna™. In August of 2014, I led an awareness and advocacy campaign that culminated in the EMA approval of Translarna™, the first approved, innovative, disease-modifying therapy for nonsense mutation Duchenne Muscular Dystrophy (nmDMD), a rare, progressive and fatal genetic condition. The EMA approval of Translarna was followed by additional approvals globally.

Subsequently, I opened the market for PTC by establishing and leading the Alliance Management department and executing on collaborative partnerships in therapeutic areas including neuromuscular, oncology, neurology, cardiovascular, and anti-infective diseases - with pharma, biotech, and nonprofit partners. 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 and subsequently by many global markets.

From 2018 to 2023, I served as Vice President and Head of Global Scientific Affairs at PTC, defining the strategy across the company's four core pillars: Medical Information, Medical Communications, Publications, and Medical Education & Training. In my medical affairs career at PTC, I launched five drugs: Translarna™ for nmDMD (ex-US), Emflaza® for DMD (US), and Tegsedi® for hereditary transthyretin amyloidosis (hATTR), and Waylivra® for Familial Chylomicronemia Syndrome (FCS) and Partial Lipodystrophy (FPL) in Latin America. And I also played a pivotal role in the EU launch of Upstaza™ (eladocagene exuparvovec), the first gene therapy delivered locally through surgery in the brain of patients living with Aromatic L-Amino Acid Decarboxylase deficiency (AADCd). My steadfast commitment and long-standing global collaborations with healthcare professionals have brought these treatments along with hope to countless patients living with rare diseases around the world.

Having gained such breadth and depth of experience in drug discovery, development and commercialization, I then pivoted to the consulting side of the industry. In November 2023, I co-founded Precision Science Consulting LLC and in May 2024, I joined Rapid Commercialization Partners - a consulting firm - as Senior Vice President of Medical Affairs and Scientific Communications. I am focused on providing strategies for optimal commercial readiness and strategic planning for clinical-stage biotech companies. Leveraging my unique experience launching five rare disease drugs - two of which I led from bench to patient - as well as my broad and deep expertise in Research & Development, Alliance Management, and Global Medical Affairs, I offer strategic and insightful advice for the successful commercialization of rare disease drugs.

As a Fulbright scholar from Cyprus, I earned my BA in Biology and Chemistry from Wellesley College in Massachusetts, followed by a PhD in Molecular Biology and Human Genetics from the University of Pennsylvania. I am the author of numerous scientific publications including ones in the prestigious journals of *Nature* and *Science*. I am a patent holder, and a globally accomplished speaker. I also hold a Certificate of Achievement in Alliance Management (CAAM) from the Association of Strategic Alliance Professionals. My deep scientific expertise, relationship-building skills, positivity, and resilience help me in my quest to improve the lives of patients with rare genetic diseases and their families.

As part of my volunteer and philanthropic work, I serve as Vice President and Secretary of the Association of Greek American Professional Women (AGAPW), a charitable and educational New York–based nonprofit organization that seeks to expand career opportunities and promote community and leadership among Greek American professional women and also offers scholarships to Greek American college students. I also serve on the Board of Directors of the Cyprus Children's Fund, supporting children of enclaved and refugee families, and children facing medical or financial hardship, as well as nonprofit organizations supporting children with medical and special needs in Cyprus. I am also the General Secretary of Lampousa, a Cypriot-American organization. Lastly, I am a mentor and member of ALLILONnet, an organization which fosters networking and mutual support among Greeks and philhellenes worldwide.

My impactful contributions to the scientific and medical fields have been recognized in both the USA and Cyprus. In May 2019, I appeared on the cover of «Η ΓΥΝΑΙΚΑ (*The Woman)*», a magazine by *The National Herald*, in an article titled "Pannie Trifillis: Woman Scientist of the Greek American Community." In October 2020, I was a guest on the Cyprus Broadcasting Corporation's "Cypriots of the World" TV program, where I discussed my life and career. In June 2023, I was named as an Honorary Member of the Cyprus Biological Society at the Excellence in Biology 2023 ceremony held at the University of Cyprus Medical School. In April 2024, as a Greek Cypriot with dual citizenship - from Cyprus and the USA - I appeared on the cover of the *GOLD* business magazine, in a feature titled "Living the American Dream: How 9 Diaspora Cypriots Succeeded in the USA." That same year, in October 2024, I was included in the 2024 *Marquis Who's Who in the United States of America*, the official biographical registry recognizing outstanding professionals with notable achievements. Finally, in October 2025, I was presented with the Madame Figaro Cyprus, Woman of the Year award, in the category Scientist/Academic.

I was born in Famagusta, Cyprus and lived through the 1974 Turkish invasion and occupation. In 2024, on the somber 50th anniversary of that event, I published a book titled «**1974-2024 50 Years Is Way Too Long to Wait!**» with historical and photographic material from my life before and after the invasion - as my legacy for future generations. I am married, I have two adult children, and I reside in New Jersey, USA.