Vertex Researcher, Paul Negulescu Ph.D., Receives the 2025 Canada Gairdner International Award for Pioneering Research and Discovery of Medicines for Cystic Fibrosis

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BOSTON--(BUSINESS WIRE)--Apr. 11, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that Paul Negulescu, Ph.D. Senior Vice President, Vertex has been awarded the 2025 Canada Gairdner International Award “for pioneering research into the cellular and molecular mechanisms underlying the genetic disease cystic fibrosis, leading to the development of transformative drug therapies based on these mechanisms, thereby improving and saving countless lives.” Negulescu shares the award with Michael J. Welsh, M.D., University of Iowa.

“For more than 20 years, Paul and the team of dedicated Vertex researchers have focused on discovering and developing breakthrough therapies for people living with cystic fibrosis (CF). Paul’s outstanding vision, leadership, determination and collaborative nature has resulted in what was once thought impossible — the discovery of the first-ever protein folding corrector medicines that treat the underlying cause of CF and have forever transformed the course of this disease,” said David Altshuler, M.D., Ph.D., Executive Vice President, Global Research, and Chief Scientific Officer at Vertex.

“I am honored and humbled by this award that recognizes not only me, but also the dedication, creativity and scientific excellence of the thousands of people across Vertex, and in the CF community, who have been steadfast in their commitment to bringing these medicines to people with cystic fibrosis around the world,” said Negulescu.

The gene responsible for CF was discovered in 1989, but until the work of Vertex scientists led by Negulescu, treatments for CF addressed only its symptoms rather than the underlying cause of disease. Today, Vertex’s five approved medicines treat CF by restoring function of the defective CFTR protein. The most recently approved medicine is a triple combination therapy that has the potential to treat more than 90% of people with CF with any of more than 300 different mutations, with once-daily dosing. The research team continues its relentless efforts to discover and develop even more effective therapies as well as novel approaches for the approximately 10% of people who are not expected to respond to existing therapies, such as a messenger ribonucleic acid (mRNA) approach currently in clinical development.

Through the Canada Gairdner International Award, the Gairdner Foundation, celebrates the world’s most creative and accomplished researchers whose unique scientific contributions have increased the understanding of human biology and disease, and improved the health and wellbeing of people around the world. The Canada Gairdner International Award is one of the most preeminent science awards in Canada and is widely recognized as one of the top scientific prizes in the world.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 94,000 people globally. CF is a progressive, multi-organ disease that affects the lungs, liver, pancreas, GI tract, sinuses, sweat glands and reproductive tract. CF is caused by a defective and/or missing CFTR protein resulting from certain mutations in the *CFTR* gene. Children must inherit two defective *CFTR* genes — one from each parent — to have CF, and these mutations can be identified by a genetic test. While there are many different types of *CFTR* mutations that can cause the disease, the vast majority of people with CF have at least one *F508del* mutation. *CFTR* mutations lead to CF by causing CFTR protein to be defective or by leading to a shortage or absence of CFTR protein at the cell surface. The defective function and/or absence of CFTR protein results in poor flow of salt and water into and out of the cells in a number of organs. In the lungs, this leads to the buildup of abnormally thick, sticky mucus, chronic lung infections and progressive lung damage that eventually leads to death for many patients. The median age of death is in the 30s, but with treatment, projected survival is improving.

Today Vertex CF medicines are treating over 68,000 people with CF across 60 countries on six continents. This represents 2/3 of the diagnosed people with CF eligible for CFTR modulator therapy.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases and conditions. The company has approved therapies for cystic fibrosis, sickle cell disease, transfusion-dependent beta thalassemia and acute pain, and it continues to advance clinical and research programs in these areas. Vertex also has a robust clinical pipeline of investigational therapies across a range of modalities in other serious diseases where it has deep insight into causal human biology, including neuropathic pain, APOL1-mediated kidney disease, IgA nephropathy, primary

membranous nephropathy, autosomal dominant polycystic kidney disease, type 1 diabetes and myotonic dystrophy type 1.

Vertex was founded in 1989 and has its global headquarters in Boston, with international headquarters in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia, Latin America and the Middle East. Vertex is consistently recognized as one of the industry's top places to work, including 15 consecutive years on Science magazine's Top Employers list and one of Fortune’s 100 Best Companies to Work For.

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