Vertex Scientists Awarded the 2025 Lasker~DeBakey Clinical Medical Research Award for Pioneering Discoveries in Cystic Fibrosis

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BOSTON--(BUSINESS WIRE)--Sep. 11, 2025-- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) today announced that Senior Vice President Paul Negulescu has been named as one of the winners of this year's Lasker~DeBakey Clinical Medical Research Award for his role "in the development of a novel, life-saving treatment for cystic fibrosis (CF) — namely, a triple-drug combination therapy, TRIKAFTA®, that has helped countless people with this genetic disease." Dr. Negulescu is one of three awardees, alongside Jesús (Tito) González, a former Vertex scientist, and Michael Welsh, Professor of Internal Medicine-Pulmonary, Critical Care and Occupational Medicine, University of Iowa.

The prize honors the groundbreaking work of Dr. Negulescu, Dr. González and the entire Vertex team who discovered and developed the first and only approved medicines that address the underlying cause of CF. To date, Vertex has brought five transformational medicines to patients with CF which have fundamentally changed the way this disease is treated.

"I'm honored to represent my co-leaders on the CF program, Fred Van Goor and Sabine Hadida, and the more than a thousand people at Vertex who have worked over two decades to make the impossible possible. Together, we've brought five medicines including TRIKAFTA and now ALYFTREK ® to tens of thousands of people with CF around the world — medicines that have fundamentally changed the course of this disease," saidDr. Negulescu. "This award recognizes that collective effort and serves as a reminder of the work ahead in reaching all people with CF."

This year's recipients represent several major scientific inflection points in the modern history of CF — from research into the cellular mechanisms that cause CF all the way through to the first FDA-approved medicines that treat the underlying cause of the disease.

Established in 1945 by Mary and Albert Lasker, pioneering biomedical research advocates, the Lasker Awards are now widely regarded as America's preeminent biomedical research prize. Since 1945, the Lasker Foundation has awarded more than 400 prizes through the Lasker Awards, which recognize the contributions of leaders who have made major advances in the understanding, diagnosis, treatment, cure and prevention of human disease. Over the years, 101 Lasker Laureates have also received the Nobel Prize, including 13 since 2018. More details on the Lasker Award recipients, the full citations for each award category, video interviews and photos of the awardees, and additional information on the Foundation are available at laskerfoundation.org.

About Cystic Fibrosis

Cystic fibrosis (CF) is a rare, life-shortening genetic disease affecting more than 109,000 people, including 94,000 people in North America, Europe and Australia. 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 75,000 people with CF in more than 60 countries on six continents. This represents approximately 2/3 of the diagnosed people with CF eligible for CFTR modulator therapy.

ABOUT ALYFTREK AND TRIKAFTA IN THE U.S.

ALYFTREK is indicated for the treatment of cystic fibrosis (CF) in patients aged 6 years and older who have at least one *F508del* mutation or another responsive mutation in the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene. *

TRIKAFTA is indicated for the treatment of CF in patients aged 2 years and older who have at least one *F508del* mutation in the *CFTR* gene or a mutation in the *CFTR* gene that is responsive based on clinical and/or *in vitro* data. *

*If the patient's genotype is unknown, an FDA-cleared CF mutation test should be used to confirm the presence of the indicated mutation(s) for the respective product.

IMPORTANT SAFETY INFORMATION

WARNING: DRUG-INDUCED LIVER INJURY AND LIVER FAILURE

Elevated transaminases have been observed in patients treated with ALYFTREK.

TRIKAFTA can cause serious and potentially fatal drug-induced liver injury. Cases of liver failure leading to transplantation and death have been reported in both clinical trials and the postmarketing setting in patients with and without a history of liver disease taking TRIKAFTA, a fixed-dose combination drug containing elexacaftor (ELX), tezacaftor (TEZ), and ivacaftor (IVA), the same or similar active ingredients as ALYFTREK. Liver injury has been reported within the first month of therapy and up to 15 months following initiation of TRIKAFTA.

Assess liver function tests (ALT, AST, alkaline phosphatase, and bilirubin) in all patients prior to initiating ALYFTREK or TRIKAFTA, then

every month during the first 6 months of treatment, every 3 months for the next 12 months, and at least annually thereafter. Consider more frequent monitoring for patients with a history of liver disease or liver function test (LFT) elevations at baseline.

Interrupt ALYFTREK or TRIKAFTA for significant elevations in LFTs or in the event of signs or symptoms of liver injury. Consider referral to a hepatologist. Follow patients closely with clinical and laboratory monitoring until abnormalities resolve. If resolved, resume treatment only if benefit is expected to outweigh risk. Closer monitoring is advised after resuming treatment.

ALYFTREK or TRIKAFTA should not be used in patients with severe hepatic impairment (Child-Pugh Class C). ALYFTREK or TRIKAFTA is not recommended in patients with moderate hepatic impairment (Child-Pugh Class B). ALYFTREK should only be considered when there is a clear medical need and benefit outweighs risk. If ALYFTREK is used, monitor patients closely. If TRIKAFTA is used, use with caution at a reduced dosage and monitor patients closely.

WARNINGS AND PRECAUTIONS

DRUG-INDUCED LIVER INJURY AND LIVER FAILURE

- Elevated transaminases have been observed in patients treated with ALYFTREK. TRIKAFTA, which contains the same or similar active ingredients as ALYFTREK, can cause serious and potentially fatal drug-induced liver injury. Liver failure leading to transplantation and death has been reported in patients with and without a history of liver disease taking TRIKAFTA. Liver injury has been reported within the first month of therapy and up to 15 months following initiation of TRIKAFTA
- Assess LFTs (ALT, AST, alkaline phosphatase, and bilirubin) in all patients prior to initiating ALYFTREK or TRIKAFTA, then every month during the first 6 months of treatment, every 3 months for the next 12 months, and at least annually thereafter. Consider more frequent monitoring for patients with a history of liver disease or LFT elevations at baseline, or a history of elevated LFTs with drugs containing ELX, TEZ, and/or IVA
- Interrupt ALYFTREK or TRIKAFTA in the event of signs or symptoms of liver injury, which may include:
    - Significant elevations in LFTs (e.g., ALT or AST >5x the upper limit of normal (ULN) or ALT or AST >3x ULN with bilirubin >2x ULN)
    - Clinical symptoms suggestive of liver injury (e.g., jaundice, right upper quadrant pain, nausea, vomiting, altered mental status, ascites)
- Consider referral to a hepatologist and follow patients closely with clinical and laboratory monitoring until abnormalities resolve. If resolved, and if benefit is expected to outweigh risk, resume treatment with close monitoring
- ALYFTREK and TRIKAFTA should not be used in patients with severe hepatic impairment. ALYFTREK and TRIKAFTA are not recommended in patients with moderate hepatic impairment and should only be considered when there is a clear medical need and benefit outweighs risk. If ALYFTREK is used, monitor patients closely. If TRIKAFTA is used, use with caution at a reduced dosage and monitor patients closely

HYPERSENSITIVITY REACTIONS, INCLUDING ANAPHYLAXIS

- Hypersensitivity reactions, including cases of angioedema and anaphylaxis, have been reported in the post marketing setting for TRIKAFTA. If signs or symptoms of serious hypersensitivity reactions develop during treatment, discontinue ALYFTREK or TRIKAFTA and institute appropriate therapy. Consider benefits and risks for the individual patient to determine whether to resume treatment

PATIENTS WHO DISCONTINUED OR INTERRUPTED ELX-, TEZ-, OR IVA-CONTAINING DRUGS DUE TO ADVERSE REACTIONS

ALYFTREK

- There are no available safety data for ALYFTREK in patients who previously discontinued or interrupted treatment with drugs containing ELX, TEZ, or IVA due to adverse reactions. Consider the benefits and risks before using ALYFTREK in these patients. If ALYFTREK is used in these patients, closely monitor for adverse reactions as clinically appropriate

DRUG INTERACTIONS

Use With CYP3A Inhibitors

- Exposure to vanzacaftor, tezacaftor, and deutivacaftor, or elexacaftor, tezacaftor, and ivacaftor are increased when used concomitantly with strong or moderate CYP3A inhibitors. The dose of ALYFTREK or TRIKAFTA should be reduced when used concomitantly with moderate or strong CYP3A inhibitors

Use With CYP3A Inducers

- Following concomitant use of strong or moderate CYP3A inducers with ALYFTREK, exposures of vanzacaftor, tezacaftor, and deutivacaftor were decreased, which may reduce ALYFTREK effectiveness. Concomitant use with strong or moderate CYP3A inducers is not recommended
- Exposure to ivacaftor is significantly decreased and exposure to elexacaftor and tezacaftor are expected to decrease by concomitant use with CYP3A inducers, which may reduce therapeutic effectiveness of TRIKAFTA. Concomitant use with

strong CYP3A inducers, such as rifampin, rifabutin, phenobarbital, carbamazepine, phenytoin, and St. John’s wort, is not recommended

CATARACTS

- Non-congenital lens opacities/cataracts have been reported in pediatric patients treated with TRIKAFTA, which contain ivacaftor (similar to an active ingredient in ALYFTREK). Baseline and follow-up ophthalmological examinations are recommended in pediatric patients initiating treatment

ADVERSE REACTIONS

ALYFTREK

- **Serious adverse reactions** that occurred more frequently with ALYFTREK than with ELX/TEZ/IVA in 2 or more patients (≥0.4%) were influenza (1.5%), increased AST (0.4%), increased GGT (0.4%), depression (0.4%), and syncope (0.4%)
- **The most common adverse reactions** occurring in ≥5% of patients and at a frequency higher than ELX/TEZ/IVA by ≥1% were cough, nasopharyngitis, upper respiratory tract infection, headache, oropharyngeal pain, influenza, fatigue, increased ALT, rash, increased AST, and sinus congestion

TRIKAFTA

- **Serious adverse reactions** that occurred more frequently in patients treated with TRIKAFTA compared to placebo included rash (1% vs <1%) and influenza (1% vs 0%)
- **The most common adverse reactions** occurring in ≥5% of patients treated with TRIKAFTA and at a rate higher than placebo by ≥1% were headache, upper respiratory tract infection, abdominal pain, diarrhea, rash, alanine aminotransferase increased, nasal congestion, blood creatine phosphokinase increased, aspartate aminotransferase increased, rhinorrhea, rhinitis, influenza, sinusitis, blood bilirubin increased, and constipation

USE IN SPECIFIC POPULATIONS

PEDIATRIC USE

- Safety and effectiveness have not been established for ALYFTREK in patients younger than 6 years of age, nor for TRIKAFTA in patients younger than 2 years of age. The use in children under these ages is not recommended

Please see full Prescribing Information, including **Boxed Warning**, for Alyftrek and Trikafta.

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. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on LinkedIn, Facebook, Instagram, YouTube and X.

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