



Skyhawk and Vertex Establish a Strategic Collaboration to Discover and Develop Novel Small Molecules that Modulate RNA Splicing for Serious Diseases

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Vertex receives exclusive options to license a number of Skyhawk's product candidates directed at serious diseases

Skyhawk receives \$40 million upfront as well as potential milestones and royalty payments on future sales

WALTHAM, Mass. & BOSTON--(BUSINESS WIRE)--Dec. 22, 2020-- Skyhawk Therapeutics, Inc. and [Vertex Pharmaceuticals Incorporated](#) (Nasdaq: VRTX) announced today a strategic research collaboration and licensing agreement aimed at the discovery and development of novel small molecules that modulate RNA splicing for the treatment of serious diseases.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20201222005032/en/>

"We believe that splice site modulation holds significant promise for the treatment of diseases which today have limited or no therapeutic options," said Mark Bunnage, D.Phil., Senior Vice President and Site Head, Boston Research at Vertex. "This collaboration brings Skyhawk's innovative technology together with Vertex's research and development experience; and fits perfectly with our strategy of investing in new technologies that will help us transform multiple serious diseases."

"We are excited to enter this collaboration with Vertex, a global leader in creating transformative medicines through serial innovation," said Bill Haney, Chief Executive Officer of Skyhawk. "In collaboration with the Vertex team, we look forward to using our SkySTAR™ platform to discover and develop novel small molecule therapeutics that modulate RNA splicing which have the potential to transform the lives of patients with serious diseases."

Under the collaboration agreement, Vertex will pay Skyhawk \$40 million upfront. Skyhawk will grant Vertex options to exclusively license worldwide intellectual property rights to candidates discovered and developed under the collaboration that are directed to program targets. Following Vertex's exercise of its options, Vertex will be responsible for further development and commercialization. Skyhawk is also eligible to receive up to \$2.2 billion in potential milestone payments, as well as potential royalties on future sales.

About Skyhawk Therapeutics

Skyhawk develops and commercializes therapies using its novel SkySTAR™ platform, building small molecules that provide breakthrough treatments for patients. Skyhawk has productive collaborations across a broad range of disease areas ranging from neurodegenerative disease to oncology.

For more information visit:

www.skyhawktx.com

<https://www.linkedin.com/company/skyhawk-therapeutics/>

https://twitter.com/Skyhawk_Tx

About Vertex Pharmaceuticals

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has multiple approved medicines that treat the underlying cause of cystic fibrosis (CF) — a rare, life-threatening genetic disease — and has several ongoing clinical and research programs in CF. Beyond CF, Vertex has a robust pipeline of investigational small molecule medicines in other serious diseases where it has deep insight into causal human biology, including pain, alpha-1 antitrypsin deficiency and APOL1-mediated kidney diseases. In addition, Vertex has a rapidly expanding pipeline of genetic and cell therapies for diseases such as sickle cell disease, beta thalassemia, Duchenne muscular dystrophy and type 1 diabetes mellitus.

Founded in 1989 in Cambridge, Mass., Vertex's global headquarters is now located in Boston's Innovation District and its international headquarters is in London. Additionally, the company has research and development sites and commercial offices in North America, Europe, Australia and Latin America. Vertex is consistently recognized as one of the industry's top places to work, including 11 consecutive years on Science magazine's Top Employers list and a best place to work for LGBTQ equality by the Human Rights Campaign. For company updates and to learn more about Vertex's history of innovation, visit www.vrtx.com or follow us on Facebook, Twitter, LinkedIn, YouTube and Instagram.

Vertex/Skyhawk Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, statements by Mark Bunnage, D.Phil. and Bill Haney in this press release, statements about the potential benefits and results that may be achieved through Vertex's collaboration with Skyhawk, and statements regarding upfront and milestone payments and potential royalties on future sales. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent the company's beliefs only as of the date of this press release and there are a number of risks and uncertainties that could cause actual events or results to differ materially from those expressed or implied by such forward-looking statements. Those risks and uncertainties include, among other things, that the anticipated benefits and potential of Vertex's collaboration with Skyhawk may not be achieved, that data may not support further development of the therapies subject to the collaboration due to safety, efficacy or other reasons, and other risks listed under the heading "Risk Factors" in Vertex's annual report and subsequent quarterly reports filed with the Securities and Exchange Commission (SEC) and available through Vertex's website at www.vrtx.com and on the SEC's website at www.sec.gov. You should not place undue reliance on these statements. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

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