



Vertex and Ribometrix Establish Strategic Collaboration to Discover and Develop RNA-Targeted Small Molecule Therapeutics

September 30, 2019

-Partnership will leverage Ribometrix's discovery platform for up to three therapeutic programs-

-Ribometrix to receive \$20 million in upfront payment and Vertex equity investment; potential for additional milestones and royalty payments-

BOSTON & DURHAM, N.C.--(BUSINESS WIRE)--Sep. 30, 2019-- Vertex Pharmaceuticals Incorporated (NASDAQ:VRTX) and RNA therapeutics developer [Ribometrix](#), Inc. announced today that the companies have entered into a strategic collaboration to discover and develop novel RNA-targeted small molecule therapeutic candidates for serious diseases. The collaboration will combine Ribometrix's proprietary discovery platform for identifying and optimizing small molecule therapeutics that modulate RNA function by targeting three-dimensional (3D) RNA structures with Vertex's scientific, clinical and regulatory capabilities for up to three therapeutic programs, including one ongoing discovery program from Ribometrix.

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"Targeting RNA is a potentially powerful new approach for developing small molecule therapeutics, and Ribometrix has developed an innovative technology for the accelerated discovery of selective candidates in this new space," said Mark Bunnage, Senior Vice President and Site Head, Boston Research at Vertex. "This partnership adds a novel mechanism to our internal toolkit which has the potential to enhance our ongoing research capabilities and support the expansion of our pipeline of transformative medicines in serious diseases."

"As a company founded on groundbreaking research and expertise in RNA structural biology, we have seen firsthand how new technologies are dramatically expanding the druggability of RNA and the potential for transforming the treatment of previously intractable diseases," said Michael Solomon, Ph.D., Chief Executive Officer of Ribometrix. "Vertex is a leader in creating transformative medicines through serial innovation, and we are proud to partner with them to rapidly and effectively develop a core discovery platform of small molecule therapeutics targeting RNA."

About the Collaboration

Under the terms of the agreement, Vertex will pay Ribometrix approximately \$20 million upfront, which includes an equity investment in the company. Initially, Ribometrix will undertake two discovery programs with Vertex, and Vertex has an option to add a third program. Following discovery and optimization of novel therapeutic molecules, Vertex has the option to take an exclusive global license for the development and commercialization of molecules for that program. Ribometrix is eligible to receive more than \$700 million in total potential payments based upon the successful achievement of specified research, development, regulatory and commercial milestones as part of the collaboration. In addition, Vertex will pay tiered royalties on future net global sales on any products that result from the collaboration.

About the Ribometrix RNA-Targeted Therapeutics Platform

Ribometrix's platform uses multiple specialized technologies to first identify 3D RNA motifs in therapeutically compelling RNAs and then to discover small molecules that bind to these 3D motifs in therapeutic ways. Ribometrix has shown that many RNAs implicated in disease contain structural pockets that may be targeted with small molecule ligands. Ribometrix scientists use state-of-the-art proprietary assays to identify ligands that bind specifically in these pockets and therapeutically modulate these RNAs. Potential benefits over current protein and RNA-based approaches include potency, selectivity, oral bioavailability, tissue distribution and central nervous system penetration.

About Ribometrix

Ribometrix is a biotechnology company pioneering small molecule therapeutics that directly target and modulate the function of RNA to create a completely new class of medicines to treat human diseases. Ribometrix leverages its world-leading expertise in three-dimensional structural analysis of RNA to drive its discovery platform for the identification of novel small molecules that modulate the function of RNA and proteins associated with human disease. Ribometrix completed a \$30 million Series A financing in late 2018 and is headquartered in Durham, North Carolina. For more information, visit www.ribometrix.com.

About Vertex

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases. The company has three 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 medicines in other serious diseases where it has deep insight into causal human biology, such as sickle cell disease, beta thalassemia, pain, alpha-1 antitrypsin deficiency, Duchenne muscular dystrophy and APOL1-mediated kidney diseases.

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, UK. 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 nine consecutive years on *Science* magazine's Top Employers list and top five on the 2019 Best Employers for Diversity list by Forbes. 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.

Special Note Regarding Forward-Looking Statements

This press release contains forward-looking statements as defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, Dr. Bunnage's statements in the second paragraph of the press release, Dr. Solomon's statements in the third paragraph of the press release, and statements regarding future activities of the parties pursuant to the collaboration. While Vertex believes the forward-looking statements contained in this press release are accurate, these forward-looking statements represent Vertex's beliefs only as of the date of this press release and there are a number of factors that could cause actual events or results to differ materially from those indicated by such forward-looking statements. Those risks and uncertainties include, among other things, Vertex may not realize the anticipated benefits of the collaboration, and the other risks listed under Risk Factors in Vertex's annual report and quarterly reports filed with the Securities and Exchange Commission and available through the company's website at www.vrtx.com. Vertex disclaims any obligation to update the information contained in this press release as new information becomes available.

(VRTX-GEN)



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Source: Ribometrix, Inc.

Vertex Contacts

Investors:

Michael Partridge, 617-341-6108

or

Zach Barber, 617-341-6470

or

Leah Gibson, 617-961-1507

Media:

617-341-6992

mediainfo@vrtx.com

Ribometrix contact:

Tom Donovan, Ten Bridge Communications

tom@tenbridgecommunications.com

+1 857-559-3397