



Vertex and Molecular Templates Establish Collaboration to Discover and Develop Novel Targeted Conditioning Regimens to Enhance Hematopoietic Stem Cell Transplants

November 18, 2019

-Molecular Templates to receive \$38 million upfront payment, including equity investment, with potential for additional milestone and royalty payments on future sales-

BOSTON and AUSTIN, Texas, Nov. 18, 2019 -- Vertex Pharmaceuticals Incorporated (Nasdaq: VRTX) and Molecular Templates, Inc. (Nasdaq: MTEM; "Molecular Templates" or "MTEM") today announced that the two companies have entered into a strategic research collaboration to discover and develop novel targeted conditioning regimens that may enhance the hematopoietic stem cell transplant process, including transplants conducted as part of treatment with ex vivo CRISPR/Cas9 gene editing therapies such as CTX001. CTX001 is currently being evaluated in two ongoing Phase 1/2 studies in patients with transfusion-dependent beta thalassemia and severe sickle cell disease, where a hematopoietic stem cell transplant is required as part of treatment with CTX001. The collaboration will seek to discover a new conditioning regimen utilizing MTEM's engineered toxin body (ETB) platform, which is designed to specifically target and remove specific cells to enable successful engraftment of new cells.

"We believe that gene editing holds significant promise in the treatment of severe hemoglobinopathies such as sickle cell disease and beta thalassemia, and Molecular Templates' unique technology platform could play an important role in creating a targeted conditioning regimen that could replace chemotherapy currently required in conditioning regimens and thus enhance the overall future treatment experience for patients," said David Altshuler M.D., Ph.D., Executive Vice President and Chief Scientific Officer at Vertex.

"Vertex has a proven history of employing novel technologies to develop innovative medicines for diseases with high unmet medical needs, making them an ideal partner to expand the use of MTEM's ETB platform for therapeutics outside of oncology," said Eric Poma, Ph.D., Molecular Templates' Chief Executive and Scientific Officer. "MTEM is excited to be working with Vertex to focus on discovering and developing the next generation of targeted conditioning agents. This collaboration further validates the potential of our ETB platform and provides meaningful capital to support continued advancement of our own product pipeline."

Under the collaboration, MTEM will conduct research activities for the use of ETBs for up to two targets selected by Vertex. The initial research will be focused on discovering a novel conditioning regimen using MTEM's ETB technology platform. In addition, Vertex has an option to select a second target as part of the collaboration. Upon designation of a clinical development candidate that emerges from the research efforts, Vertex has the option to exclusively license molecules against the designated target. Vertex will have exclusive rights to develop molecules that emerge from the research program for any indication.

Vertex will make an up-front payment of \$38 million to MTEM, including an equity investment. MTEM is also eligible to receive future development, regulatory and sales milestones and option payments of up to \$522 million (across two targets) and tiered royalty payments on future sales.

About Molecular Templates

Molecular Templates is a clinical-stage oncology company focused on the discovery and development of differentiated, targeted, biologic therapeutics for cancer. We believe our proprietary biologic drug platform technology, referred to as engineered toxin bodies, or ETBs, provides a differentiated mechanism of action that may address some of the limitations associated with currently available cancer therapeutics. ETBs utilize a genetically engineered form of Shiga-like Toxin A subunit, or SLTA, a ribosome inactivating bacterial protein, that can be targeted to specifically destroy cancer cells. Additional information about Molecular Templates can be obtained at www.mtem.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 four 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 10 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.

Vertex 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, the statements by Dr. Altshuler in the second paragraph and Dr. Poma in the third paragraph, and statements regarding our expectations for (i) the ability of conditioning regimens to enhance the hematopoietic stem cell transplant process, (ii) the discovery of new conditioning regimens, (iii) future research and development activities, (iv) our rights under the agreement, and (v) future payments to MTEM under the agreement. 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 Vertex may not realize the anticipated benefits of the collaboration, and other risks listed under Risk Factors in Vertex's annual report and subsequent 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.

MTEM Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. The words “may,” “will,” “could,” “would,” “should,” “expect,” “plan,” “anticipate,” “intend,” “believe,” “estimate,” “predict,” “project,” “potential,” “continue,” “target” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Examples of such statements include, but are not limited to, statements relating to the outcome of the collaboration between Molecular Templates and Vertex as it relates to Molecular Templates’ ETB platform; the role of Molecular Templates’ ETB platform with respect to the treatment of severe hemoglobinopathies; whether Vertex will exercise its options; and Molecular Templates’ receipt of future development, regulatory and sales milestones and royalty payments.

Forward-looking statements are not guarantees of future performance and involve risks and uncertainties. Actual events or results may differ materially from those discussed in the forward-looking statements as a result of various factors including, but not limited to, the uncertainties inherent in the preclinical and clinical development process; whether Molecular Templates’ cash resources will be sufficient to fund its continuing operations for the periods and/or trials anticipated; Vertex’s future operating plans with respect to the collaboration and its decision whether to exercise its options; the ability of Molecular Templates to protect its intellectual property rights; and legislative, regulatory, political and economic developments. These forward-looking statements are made as of the date of this press release, and Molecular Templates assume no obligation to update the forward-looking statements, or to update the reasons why actual results could differ from those projected in the forward-looking statements, except as required by law. Investors should consult all of the information set forth herein and should also refer to the risk factor disclosure set forth in the reports and other documents Molecular Templates files with the SEC available at www.sec.gov, including, without limitation, Molecular Templates’ Annual Report on Form 10-K for the fiscal year ended December 31, 2018 and Molecular Templates’ Quarterly Reports on Form 10-Q.

(VRTX-GEN)

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