

X-Chem and Vertex Enter into Multi-Target Genetic Disease Collaboration

- *Agreement will enable discovery of small-molecule leads against validated targets for severe, genetic diseases*
- *X-Chem to receive upfront payment and potential research, development and regulatory milestones and licensing fees, plus additional royalties on future sales*

WALTHAM, Mass. – May 15, 2017 – X-Chem, Inc. (X-Chem), a privately held biotechnology company applying its innovative drug discovery platform to the generation of novel small molecule therapeutics, today announced a collaboration with Vertex Pharmaceuticals Incorporated (NASDAQ: VRTX). Under the terms of the agreement, X-Chem will apply its proprietary DEX™ libraries, which contain more than 120 billion small molecules, toward the discovery of new drug leads against targets involved in multiple specialty diseases. Vertex will have the option to license lead-like hits discovered under the collaboration, and will be responsible for further development and commercialization of the resulting programs.

“We look forward to working closely with Vertex, a recognized innovator in discovery of first-in-class treatments for severe, life-threatening diseases,” said Rick Wagner, Ph.D., Chief Executive Officer of X-Chem. “The combination of X-Chem’s DEX™ platform and Vertex’s expertise has great potential to generate promising leads that could lead to medicines for the treatment of serious, specialty diseases with high unmet needs.”

“As Vertex continues to grow and diversify its R&D pipeline, we look to complement our productive internal efforts with other organizations who share our passion to treat diseases for which there is no existing treatment,” said Mark Bunnage, Senior Vice President and Boston Research Site Head at Vertex. “With a cutting-edge small molecule lead discovery platform, X-Chem is ideally suited to help Vertex tackle challenging rare-disease targets.”

Under the terms of the agreement, X-Chem will receive an upfront payment and potential research, development and regulatory milestones and licensing fees. X-Chem is also eligible for royalties on revenues from medicines originating from the collaboration. The agreement includes the option for an expansion to include additional targets.

About the DNA-Encoded X-Chem (DEX™) Library and Platform

Due to the size and diversity of the DEX™ library, X-Chem can discover multiple series of novel, potent and selective lead compounds at an unprecedented rate of success against a wide range of targets, including some that previously failed using conventional screening methods. A number of proprietary innovations in library design, screening methodology and bioinformatics underlie the exceptional performance of the DEX™ platform. In particular, X-Chem’s approach to library construction allows for additional chemical reactions to become useable in DNA-encoded library synthesis. Together,

these developments result in a much greater repertoire of diversity for small molecules, which cover a range of categories including fragment molecules, small molecular weight heterocyclic compounds, and macrocyclic structures. This diverse library, combined with a heightened ability to detect active molecules, has yielded a robust process that has been highly successful against targets categorized as difficult or intractable.

About DNA-Encoding

The X-Chem drug discovery engine is based on a library, currently in excess of 120 billion compounds and growing, generated by iterative combinatorial synthesis of small molecules tethered to DNA tags that record the synthetic history of the small molecule. Every small molecule in the library has a unique DNA barcode attached to it. The library is screened as a mixture using affinity-based binding to a target of interest. Certain rare molecules in the library that bind to the target can be “fished out,” while the rest of the molecules are washed away. DNA sequencing methods are then used to detect molecules that are enriched when bound to the target. The diverse nature of the library produces multiple families or clusters of related molecules that bind to the target, forming a basis for emergent structure-activity relationships. Structure-activity relationships are typically used by medicinal chemists to guide iterative chemical maturation of a molecule into a drug. Based on the synthetic history encoded in the DNA sequence information, molecules are then made without the DNA tag attached, and tested for activity in conventional assays.

About X-Chem

X-Chem, Inc. is a privately-owned biotechnology company based in Waltham, Mass. The company’s mission is to apply its powerful product engine to the discovery of small molecule compounds against high-value therapeutic targets. X-Chem has established partnerships with Roche, AstraZeneca, Bayer, Pfizer, Alexion, MD Anderson Cancer Center, Sanofi, Janssen, and several other leading pharmaceutical companies, biotechnology organizations, and academic centers. For further information on X-Chem, please visit: <http://www.x-chemrx.com/>.

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