



HTG Highlights the Advantages of Its Drug Discovery Engine

May 16, 2023

TUCSON, Ariz., May 16, 2023 (GLOBE NEWSWIRE) -- [HTG Molecular Diagnostics](#), Inc. (Nasdaq: HTGM) (HTG), a platform-based life science tools and drug discovery company, today reported achievement of another technical milestone in its drug discovery business.

HTG is pioneering a proprietary platform-based approach that is designed to help improve drug discovery, referred to as transcriptome-informed drug discovery and design. HTG's objective is to develop best-in-class molecules for the treatment of diseases, with the ability to apply its platform agnostically across therapy areas. At the center of this approach is HTG's proprietary RNA profiling technologies, functionally married with an advanced medicinal chemistry using a novel artificial intelligence (AI)-driven platform, allowing for the improved selection and design of molecules. Currently, the company's most advanced discovery programs are in oncology with an emphasis on the treatment of acute myeloid leukemia (AML).

Using its proprietary platforms, HTG has successfully designed a first generation chemical library. The proportion of compounds with meaningful activity (hit rate) for this first library was approximately 25% in cell-based test system models. The company's lead compounds target AML and have demonstrated *in vitro* efficacy both as standalone agents and in combination with current standards of care, with increased efficacy versus standard of care alone. Transcriptomic analysis of these cells revealed meaningful information that contributed to the understanding of the efficacy of the candidate molecules relative to the other compounds used as reference materials for this particular pharmacologic target. Most revealing and impressive about this outcome was that select HTG candidate molecules were found to be associated with key desirable biological differences in gene expression based on known biological pathways. These results included downregulating the expression of FLT3, a gene that is well-recognized to play a role in AML proliferation and that is also mutated in the majority of AML cases, and upregulating the expression of TET2, a known tumor suppressor gene where loss of signaling is linked to progression of this cancer. This reflects a desirable biological difference, especially when compared to the reference non-HTG comparator compound, where the expression of these particular genes was regulated in the opposite direction to the HTG compounds.

"Traditional drug discovery efforts are primarily limited to modality-target assays *in vitro*," said Dr. Robert Spitale, Founding Associate Dean of Research and Professor, School of Pharmacy and Pharmaceutical Sciences at the University of California Irvine. "These assays, which have been valuable for the discovery of new drugs, do not represent the complexity of the cell. The hard part is understanding how drugs work on their targets and affect cell function and behavior – the ultimate goal would be to do drug discovery using assays that can tell developers what is happening to the entire cell system. To do so, we need robust and rapid assays, that also generate a lot of data to look at the entire cell response. This is the exciting part of HTG's platform – a whole cell response that allows its users to dig into the details. What's more, connecting changes in RNA expression to expected or desired outcomes of a drug screen function is a big leap forward and strongly supports the premise of HTG's new direction in drug discovery."

The findings and other data generated from the first library have been fed back into HTG's AI-driven discovery engine and a second generation of candidate molecules has been rendered, with a hit rate for this subsequent library of approximately 35%. Subsequent *in vitro* efficacy studies on this second generation of molecules have also demonstrated further improvement in efficacy in cell-based test system models.

The lead molecules for this second library are currently progressing through the remaining portion of lead optimization and HTG expects to have sufficient data to support entry of these lead candidate molecules into development in the third quarter of 2023. The company has estimated market opportunity for the initial oncology indication for its first drug candidate at approximately \$600M globally. Additionally, the company believes that the target potentially has additional value in at least six or more solid tumor indications based upon initial efficacy data generated by HTG in these other cancers.

"I see the data that our team has generated in these studies as a tangible demonstration of the power of the drug discovery engine we have built," said Dr. Stephen A. Barat, SVP of Therapeutics at HTG. "We have demonstrated that we can progress from target to drug candidate in approximately 12 months with this platform approach, with the added benefit of having much deeper knowledge about the biological responses in diseased cells at these earlier development stages. We believe this ability to enrich our understanding of the mechanism of action for the lead compounds will allow us to make more informed selection choices far earlier in the process, which in turn will translate into greater chances for success in development."

"We continue to advance the capabilities of our discovery platform, including an exciting capability which is allowing us to use transcriptomic signatures representative of cellular perturbation from pharmacologic target modulation as a starting point where the AI system can design molecules, thus creating a capability for two way 'conversations' between chemistry and biology," Dr. Barat continued. "This further increases the utility of this discovery platform for not only selecting and designing new molecules, but also as a key tool in potentially repurposing other drugs."

About HTG:

HTG is accelerating precision medicine from diagnosis to treatment by harnessing the power of transcriptome-wide profiling to drive translational research, novel therapeutics and clinical diagnostics across a variety of disease areas.

Building on more than a decade of pioneering innovation and partnerships with biopharma leaders and major academic institutes, HTG's proprietary RNA platform technologies are designed to make the development of life science tools and diagnostics more effective and efficient and to unlock a differentiated and disruptive approach to transformative drug discovery. For more information visit www.htgmolecular.com.

Forward-Looking Statements:

Statements contained in this press release regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding HTG’s objectives and ability to develop best-in-class molecules; the ability of HTG’s platform to enable improved selection and design of molecules; HTG’s expectations to have sufficient data to support entry of its lead candidate molecules into development in the third quarter of 2023; the estimated market opportunity for the initial oncology indication for HTG’s first drug candidate and the potential for other solid tumor indications for the same target; the potential of HTG’s AI-driven drug discovery engine; HTG’s ability to progress from target to drug candidate using its platform approach in approximately 12 months; the ability of HTG’s platform to potentially repurpose other drugs; HTG’s expected pipeline advancement; and the capabilities of HTG’s technology. Words such as “designed to,” “believe,” “anticipate,” “expect,” “potential,” “will” and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements necessarily contain these identifying words. These forward-looking statements are based upon management’s current expectations, are subject to known and unknown risks, and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, including risks associated with drug discovery and development; the risk that our technologies may not provide the benefits that we expect; risks associated with our ability to develop and commercialize our products; observations in cell-based test system models and in vitro efficacy results may not be replicated in trials in humans; risks associated with our ability to enter into licensing, partnering or other transactions for any candidates we discover or develop; the risk that our products and services may not be adopted by biopharmaceutical companies or other customers as anticipated, or at all; and risks related to our need for additional capital. These and other factors are described in greater detail in our filings with the Securities and Exchange Commission (SEC), including under the “Risk Factors” heading of our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, as filed with the Securities and Exchange Commission on May 10, 2023. All forward-looking statements contained in this press release speak only as of the date on which they were made, and we undertake no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

HTG Investor Contact:

Ashley Robinson
LifeSci Advisors
Phone: (617) 430-7577
Email: arr@lifesciadvisors.com



Source: HTG Molecular Diagnostics, Inc.