Deep Genomics Nominates Industry's First AI-Discovered Therapeutic Candidate

- *Artificial intelligence enables target and drug candidate to be identified in less than 18 months*
- *Initial target of Wilson disease represents significant clinical need, new era of drug discovery*

Toronto, Ontario – September 25, 2019. Deep Genomics, the leading AI therapeutics company, announced today that its proprietary artificial intelligence-based drug discovery platform has identified a novel treatment target and corresponding drug candidate for Wilson disease, a rare, serious, and potentially life-threatening genetic disorder.

"This is an important milestone for patients affected by Wilson disease and it represents a significant advance in the drug discovery community more broadly," said Brendan Frey, founder and CEO of Deep Genomics. "Within 18 months of initiating our target discovery effort, we identified a genetic mutation that causes the disease, the chemical properties needed in a molecule to target the mutation, and a compound that warrants further investigation. We are delighted to nominate the first ever AI-discovered therapeutic candidate and are eager to move it rapidly into the clinic for the potential benefit of patients."

Deep Genomics will develop the candidate, DG12P1, for the treatment of patients with Wilson disease who harbor a genetic mutation that impairs the body's ability to remove copper. Wilson disease affects approximately one in every 30,000 people worldwide and, if left untreated, can cause life-threatening organ damage.

"Researchers have struggled for two decades, without success, to understand the mechanism of this genetic mutation that causes Wilson disease," said Frederick K. Askari, M.D., Ph.D., associate professor and director of the Wilson disease program at the University of Michigan. "The clarity that this artificial intelligence platform has brought to the scientific community is astounding and the potential of a therapy that could operate at the genomic level to correct the disease process is exciting. Patients can now have hope that a therapy may be developed that will recapitulate normal gene function and make their copper problems go away."

Path to Discovery

Deep Genomics' AI system scanned over 2,400 diseases and over 100,000 pathogenic mutations while searching for good drug development opportunities. By analyzing hundreds of millions of data points, Deep Genomics' AI platform was able to predict and confirm the precise disease-causing mechanism of the mutation Met645Arg, one of several genetic mutations that leads to loss of function of the ATP7B copper-binding protein, and thereby identify a clear therapeutic target. The AI system was then used to identify 12 lead candidates out of thousands of potential compounds, taking into account *in vitro* efficacy and toxicity.

DG12P1 was designed to correct the exon skipping mechanism of Met645Arg and, after tolerability experiments, Deep Genomics declared it the ideal candidate to advance toward IND. Further details on the disease-causing mechanism are available at https://www.biorxiv.org/content/10.1101/693572v3.

In a subsequent analysis, Deep Genomics' AI platform was able to rapidly identify two additional mutations that can cause Wilson disease. These potential therapeutic targets are currently being experimentally confirmed.

"Our expectation is that, going forward, Deep Genomics' platform will enable them to go from known target to first patient dosed in less than half the time of the industry standard, and they may be able to do this even faster with subsequent programs," said Arthur A. Levin, Ph.D., a member of the company's Strategic Advisory Board. "This is truly unprecedented and opens the door to a smarter, faster, and vastly more efficient means of identifying viable drug candidates for a host of diseases. Developing new therapeutics is full of unknowns, but I am certain that we are witnessing a new era of drug discovery."

About Wilson Disease

Wilson disease can be caused by several different mutations that lead to loss of a protein required for copper transport (ATP7B). Without the necessary protein, copper is improperly regulated in the body and accumulates at toxic levels in the liver and central nervous system, leading to hepatic, neurological and psychiatric symptoms. The standard of care for patients presents serious adverse effects and adherence issues.

About Deep Genomics

Deep Genomics is developing a universe of individualized genetic medicines by creating AI systems that are used to accelerate all steps of drug discovery and development, including target discovery, lead optimization, toxicity assessment and innovative trial design. Since its inception in 2015, Deep Genomics has used high throughput assays and advanced robotics systems to generate billions of data points and has built dozens of carefully engineered and validated machine learning systems that support drug development. Deep Genomics is located in the heart of Toronto, the fastest growing tech hub in North America. For more information, visit www.deepgenomics.com and follow us on Twitter at @deepgenomics.

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