Progress Update for Drug Discovery Research Program to Discover Novel Agents to Treat Duchenne Muscular Dystrophy (DMD)

PTC Therapeutics, Inc. (PTC) and Parent Project Muscular Dystrophy (PPMD) are collaborating to discover new drugs to treat Duchenne muscular dystrophy (DMD). In an ongoing effort to identify new treatments for DMD patients, PTC is excited to report the completion of the compound selection process. Following the high-throughput screens performed against five target genes, hits were identified and selected from the PTC compound library. These compounds were re-evaluated to confirm activity. A subset of molecules demonstrated activity when retested and were evaluated for their drug-like properties. Selected molecules are undergoing pharmacological profiling and focused chemistry efforts towards the goal of identifying lead molecules. The completion of the compound selection process and the initiation of a focused chemistry effort mark two major milestones in the progress of the program.

PTC is using a proprietary drug discovery platform technology called GEMS (Gene Expression Modulation by Small-molecules) to search for new drugs for DMD patients. The GEMS technology allows PTC to identify small molecules that up- or down-regulate the production of proteins. GEMS has proven to be a very robust technology that can address difficult drug targets. PTC has a number of drug discovery programs that have validated the applicability of GEMS across multiple therapeutic areas.

PTC is very pleased with the completion of these major program milestones. Target specific hits have been identified and these compounds will advance through the drug discovery process as rapidly as possible. The next major goals are to demonstrate activity on the endogenous target proteins, initiate pharmacological characterization and ultimately identify a small set of “lead compounds” that will undergo an intensive period of “lead optimization.” Initiation of the lead optimization process marks a major milestone in the drug discovery process.

For more information on PTC or the GEMS technology please visit www.ptcbio.com.

For more information on the drug discovery and development process, please visit www.fda.gov.

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