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 high-throughput screens performed against five target genes. These targets were chosen because altering the amount of the protein expressed would be anticipated to help treat DMD patients. The completion of these screens marks a major milestone 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’s scientists investigated five different gene targets believed to be medically relevant in DMD. Based on this initial work, PTC scientists developed high-throughput screening assays for each proposed target and screened each target against PTC’s compound library, which contains approximately 200,000 diverse compounds. The results for each screen have been analyzed and PTC is pleased to report that target-specific hit compounds have been identified. Following confirmation of the compound's activity, the compounds will be tested directly against the gene expressing the protein of interest.

PTC is very pleased with the completion of this major program milestone. The compounds demonstrating specificity will advance through the drug discovery process as rapidly as possible. The initial steps have progressed quickly, and the next goal is to identify a small set of “lead compounds” that will undergo an intensive period of “lead optimization.” The lead optimization process may last up to 24 months. The goal of the lead optimization process is to identify a “Development Candidate” that will be tested in a series of studies to enable submission of an IND – Investigational New Drug application (the application required for testing a drug in humans) to the FDA. These studies typically require twelve months and are referred to as the IND-Enabling Studies. Pending FDA concurrence, a compound would enter clinical studies typically a month after IND submission.

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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