

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

PTC Therapeutics, Inc. (PTC) and PPMD are collaborating to discover new drugs to treat Duchenne Muscular Dystrophy (DMD). PTC is committed to identifying new treatments for DMD patients and already has a drug (PTC124) in clinical trials that will hopefully treat the estimated fifteen percent of DMD patients that have the disease because of a specific type of mutation called a nonsense mutation. The goal of this drug discovery collaboration is to identify new drugs that would be tested in the clinic to treat all DMD patients.

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. The GEMS technology has proven to be a very robust technology that can address difficult drug targets. PTC has a number of drug discovery programs that have already validated the applicability of GEMS technology across multiple therapeutic areas.

PTC's scientists have been investigating five different gene targets that could by used in the GEMS screening technology. These targets were chosen because altering the amount of the protein expressed would be anticipated to help treat DMD patients. Based on this initial work, PTC scientists have subsequently developed high-throughput screening assays for each proposed target that would be used in the GEMS technology.

The GEMS assays for the first four targets have already been screened against PTC's compound library. The PTC chemical library contains approximately 200,000 diverse compounds. PTC is pleased with the results of these screens. The screening of the remaining target will occur shortly. PTC is excited by the rapid progress of this project and anticipates completing the high-throughput screens of the five proposed targets in January 2005.

Once these screens are completed, the next step in the drug discovery process is to identify the compounds that reproducibly demonstrate activities observed in the initial screen. Following confirmation of the compounds activity, they will be directly tested directly against the gene expressing protein of interest. PTC's scientists are currently developing these assays so that the biological activity of the compounds selected in the high-throughput screens can be evaluated.

PTC is very pleased with the how this collaboration is progressing. Our plan is to continue to move these compounds through the drug discovery process as rapidly as possible. The initial steps of the process described above have gone exceptionally well, and our goal is to identify a small set of "lead compounds" that will then undergo an intensive period of "chemical lead optimization." Hopefully, the result of this optimization will be a drug candidate that can be tested in the clinic to determine its safety and efficacy for DMD patients.

For more information on PTC or the GEMS technology please visit <u>www.ptcbio.com</u>.