MT1621

MT1621, Thymidine/deoxycytidine a deoxynucleoside combination therapy, is in development for an inherited mitochondrial disorder, thymidine kinase 2 deficiency (TK2d). Pre-clinical and initial clinical data from studies with deoxynucleoside combination therapy suggest that it may meaningfully alter the course of disease in patients with TK2 deficiency. Modis has exclusively licensed worldwide rights to data and intellectual property related to a broad range of mtDNA depletion disorders from its academic collaborators. Modis is advancing discussions with the FDA and EMA on the regulatory path to approval for MT1621. The company is planning to conduct additional clinical studies with the goal of obtaining regulatory approval to make MT1621 available to patients globally.

MT1621 is a therapy that targets the underlying pathophysiology of TK2d by restoring mitochondrial DNA replication fidelity. The drug candidate consists of a combination of deoxynucleosides (the building blocks of mtDNA) given orally as a dissolved solution. Deoxynucleoside combination therapy improves nucleotide balance, increases mtDNA copy number, improves cell function, and prolongs life in preclinical models of TK2d.

MT1621 has been granted orphan drug designation by both the FDA and EMA, and Modis is advancing discussions with health authorities on the regulatory path to approval. During initial clinical studies, there were no unexpected safety issues with deoxynucleoside combination therapy, and Modis anticipates upcoming studies to confirm a safety profile that is generally well tolerated by patients with TK2 deficiency. Data from initial clinical studies suggest that this therapy may meaningfully alter the course of disease in patients with TK2 deficiency. The company is planning to conduct additional clinical studies with the goal of obtaining regulatory approval to make MT1621 available to patients globally. If approved, MT1621 would represent the first commercial therapy for TK2d or any MDD.