

October 14, 2009 - ZYSTOR CLEARED TO BEGIN CLINICAL TRIAL

ZyStor Therapeutics, Inc., a biotechnology company developing a new class of targeted protein therapeutics for the treatment of Lysosomal Storage Diseases using the Company's proprietary Glycosylation Independent Lysosomal Targeting technology, today announced that it has received clearance from the U. S. Food and Drug Administration to proceed into a clinical trial for its first drug candidate, ZC-701, an enzyme replacement therapy for the treatment of Pompe disease.

Jonathan LeBowitz, Chief Scientific Officer of ZyStor Therapeutics, Inc., said, "We are pleased and very proud to announce that ZC-701 has been cleared by the FDA for a Phase I human safety trial. A small and dedicated team has brought ZC-701 from concept to the clinic and now ZyStor is poised to bring this promising treatment to Pompe patients. We hope that these patients will benefit from the improved targeting of ZC-701 to the tissues in the body that are most affected by Pompe disease."

Pompe disease is one of a group of about 50 rare genetic disorders called Lysosomal Storage Diseases (LSDs) that interfere with the body's ability to degrade complex molecules within the lysosome. Pompe disease affects 5,000-10,000 people worldwide, making it one of the most prevalent LSDs. It is caused by an inherited deficiency in a lysosomal enzyme, acid-alpha-glucosidase (GAA). Deficiency of GAA results in lysosomal glycogen accumulation in multiple tissues, with cardiac and skeletal muscle tissues most seriously affected. The most severe form of Pompe disease is the infantile-onset form in which patients have critical cardiomyopathy and heart failure, severe muscle hypotonia and die prior to 2 years of age.

ZyStor's Glycosylation Independent Lysosomal Targeting (GILT) technology is the first peptide-based targeting technology that enables efficient targeting of enzyme replacement therapeutics to the lysosomal compartment of cells in a variety of tissues. ZC-701 is a recombinant protein containing ZyStor's proprietary GILT tag fused to GAA, thereby allowing more efficient delivery of GAA to the lysosome of muscle cells. In preclinical research, ZC-701 was found to be both safe and highly efficacious in well-studied animal models. In animal models, preclinical efficacy was seen at doses much lower than those reported for the currently approved drug for the treatment of Pompe disease. It is anticipated that the first-in-human trial of ZC-701 will be conducted in late-onset Pompe patients.

Dr. Barry Byrne, Professor of Pediatrics and Molecular Genetics & Microbiology at the University of Florida College of Medicine, said, "The FDA approval of the ZyStor IND represents an important step in developing an improved treatment for Pompe patients. Clinical studies will show whether this new therapeutic approach provides the same benefit in humans as in preclinical testing."

About ZyStor Therapeutics, Inc.

ZyStor Therapeutics, Inc. is a privately held biotechnology company based in Milwaukee, Wisconsin developing a novel class of targeted protein therapeutics incorporating the Company's proprietary GILT technology that allows more efficient delivery of therapeutic enzymes to the lysosome and is thus applicable to the treatment of lysosomal storage diseases. This proprietary technology is termed Glycosylation Independent Lysosomal Targeting (GILT) because the peptide tag replaces mannose-6-phosphate (M6P) as the moiety normally targeting the lysosome. Use of a peptide targeting strategy not only improves lysosomal delivery of the therapeutic but also offers manufacturing advantages over conventional protein therapeutics for the treatment of LSDs.