Ataluren Aniridia Study

PTC Therapeutics has announced plans to initiate a Phase 2 proof-of-concept study for ataluren in patients with Aniridia caused by a nonsense mutation by the end of 2015.

Ataluren, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as PAX 6 in Aniridia. Ataluren (with the brand name Translarna) is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Translarna is an investigational new drug in the United States, with Phase 3 clinical trials underway in nonsense mutation Duchenne muscular dystrophy and cystic fibrosis.

Preclinical data demonstrates that treatment for 10 days with Ataluren in a mouse model of Aniridia allowed full-length PAX6 protein to be synthesized in the nonsense mutation PAX6 gene, but not in a PAX6 gene harboring a splice-site mutation. In this model, ataluren inhibited disease progression, reversing the congenital ocular malformations in the cornea, lens and retina, and restoring electrical and behavioral responses of the retina. This data demonstrates the potential for ataluren to address Aniridia in those patients who have the disorder due to a nonsense mutation. The clinical study of ataluren for Aniridia patients will be initiated by the end of the year with details on study to follow.

ABOUT PTC THERAPEUTICS, INC.

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA is copied from DNA through the transcription process. PTC’s internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.