ACTUATE THERAPEUTICS RECEIVES RARE PEDIATRIC DISEASE DESIGNATION FOR 9-ING-41 FOR TREATMENT OF NEUROBLASTOMA

CHICAGO, IL and FORT WORTH, TX, October 26, 2017 – Actuate Therapeutics, Inc., a clinical stage biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to 9-ING-41, Actuate's lead clinical development candidate, for the treatment of neuroblastoma.

The Rare Pediatric Disease Designation of 9-ING for the treatment of pediatric neuroblastoma comes in addition to Orphan Drug Designations previously granted by the FDA for neuroblastoma, and separately, for treatment of glioblastoma. Upon receiving market approval by the FDA for 9-ING-41 for treatment of neuroblastoma, Actuate will be eligible to receive a Priority Review Voucher.

The FDA grants Rare Pediatric Disease designation to therapeutics intended to treat serious or life-threatening rare diseases that primarily affect individuals under the age of 18. Neuroblastoma is a very rare type of malignant tumor that develops from neural crest cells and is almost exclusively a disease of children. It is a devastating pediatric cancer and most patients older than 18 months present with multi-organ metastatic disease. High grade or recurrent disease is refractory to treatment with chemotherapy and almost uniformly fatal. Each year, less than 1000 children are diagnosed with neuroblastoma in the United States, and yet it accounts for approximately 15 percent of all pediatric cancer fatalities. Thus, neuroblastoma represents a significant unmet medical need and the development of new therapeutic agents is urgently needed.

In research presented at the 2015 AACR Annual Meeting, Actuate scientists and collaborators validated Glycogen Synthase Kinase-3 (GSK-3) as a potential therapeutic target in human neuroblastoma. GSK-3 is highly expressed in the majority of human neuroblastoma tumors. Pharmacological inhibition of GSK-3 by 9-ING-41 significantly decreased the viability of neuroblastoma cells. In vivo xenograft studies showed that the combination of 9-ING-41 with cytotoxic chemotherapy led to significant regression of neuroblastoma tumors whereas chemotherapy or 9-ING-41 alone had modest effects on tumor growth.

"The Rare Pediatric Disease Designation strongly encourages innovation focused on addressing serious unmet medical needs for children with rare life-threatening diseases – neuroblastoma epitomizes such a challenging condition. Focusing on this malignancy by modulating a novel target, GSK-3, with 9-ING-41 is very exciting. We are very encouraged by the positive responses we continue to receive in our interactions with the FDA, our Scientific Advisors, and the research community. Immediately after the upcoming submission of our IND, we plan to initiate our initial Phase 1/2 clinical trial of 9-ING-41 in patients with a wide range of cancers in early 2018. We then intend to promptly conduct a Phase 2 clinical trial of 9-ING-41 focused on children with neuroblastoma. " said Dr. Frank Giles, Chief Medical Officer of Actuate.

"GSK-3 is emerging as a potentially very important target in human malignancies and inflammatory diseases. Actuate's internal research and multiple basic science and clinical collaborations are rapidly increasing our knowledge on this target and how best to modulate it to patient's benefit. Advancing 9-ING-41 to the clinic in order to benefit patients with refractory diseases such as neuroblastoma is a very promising and meaningful endeavor."

About Actuate Therapeutics, Inc.

Actuate is a clinical stage pharmaceutical company focused on the development and commercialization of novel therapeutics for high impact cancers and inflammatory diseases. For additional information, please visit the Company's website at http://www.actuatetherapeutics.com