EryDel announces start of ATTEST, a Pivotal Phase III Trial of EDS in Ataxia Telangiectasia (A-T)

Urbino, Italy – March 6, 2017 - EryDel SpA (www.erydel.com), a biopharmaceutical company specialized in the development of drugs and diagnostics delivered through autologous red blood cells, today announced the initiation of the **A**taxia **T**elangiectasia **T**rial with the **E**ryDex **S**yst**e**m (ATTEST) study (http://attest-trial.com), a pivotal Phase 3 trial to evaluate the efficacy, safety and tolerability of EDS in patients with A-T.

EDS is an innovative product that delivers dexamethasone sodium phosphate by encapsulating the drug into red cells taken from the patient's own blood, which are then re-infused into the patient. EDS has received Orphan Drug designation for the treatment of A-T both from the FDA and EMA. EryDel recently completed a pilot Phase II trial in AT patients that demonstrated the statistically significant efficacy of EDS on both primary and secondary outcome measures.

The randomized, double-blind, placebo controlled ATTEST Study is expected to enroll 180 patients (three group of 60) at centers of excellence in the United States, Europe, India, Australia, Tunisia and Israel. The Study protocol was designed following extensive discussions with regulatory authorities in The United States and Europe.

Luca Benatti, EryDel's Chief Executive Officer, commented: "We are very pleased to announce the initiation of our planned pivotal Phase 3 trial today. A-T is a relentlessly progressive disease for which no therapy is currently available. EryDel, with the support of Patient Associations, Clinicians and A-T experts worldwide is committed to complete regulatory development and bring this innovative therapy to market".

Howard Lederman, M.D., Ph.D. Professor, and Director of the A-T Clinical Center at the Johns Hopkins Hospital (Baltimore) said: "AT is a devastating disease that causes progressive loss of neurologic function and makes most children wheelchair dependent by the age of 10 or 12 years. This is the first large-scale clinical trial of any drug to treat the neurologic manifestations of AT. Patients and families are excited to participate in the EryDel trial with the hope that it will slow the loss of neurologic function. Physicians and neuroscientists have the same hopes, and also expect that the results of the trial will shed light on the causes of progressive brain damage in this and other diseases".

About Ataxia Telangiectasia

Ataxia Telangiectasia (A-T) is a rare genetic disease caused by bi-allelic mutations in the ataxia telangiectasia mutated (ATM) gene, for which no established therapy is currently available. ATM encodes a PI3Kinase protein shown to play a pivotal role in response to DNA damage and cell cycle control. Homozygosity for ATM mutations results in a multi-systemic disorder, mainly involving the nervous and immune systems, but also predisposing patients to develop cancer. The major clinical feature of A-T is severe progressive neurodegeneration from early infancy. Specific features include progressive ataxia of the trunk and limbs, involuntary movements, oculomotor apraxia, difficulties with speech and swallowing, and peripheral neuropathy. Other clinical features of patients with the classical phenotype include oculocutaneous telangiectasia, immunodeficiency with recurrent respiratory tract infections, radiosensitivity and an increased incidence of cancer.

About EryDel

EryDel SpA is a biopharmaceutical company specialized in the development of drugs and diagnostics delivered through red blood cells (RBCs). The most advanced product, EDS (Dexamethasone Sodium

Phosphate delivered through autologous RBCs), has potential for the treatment of Ataxia-Telangiectasia, a rare autosomal recessive disorder for which no established therapy is currently available. EDS has received Orphan Drug designation for the treatment of A-T both from the FDA and EMA. A recently completed pilot Phase II trial in A-T patients demonstrated statistically significant efficacy of EDS on both the primary and secondary efficacy measures. EryDel has a pipeline of preclinical programs that use its proprietary RBC's delivery technology for the treatment of other rare diseases.

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