EryDel Consortium Gains €6 million of EU Funding for Development of EDS for the treatment of Ataxia Telangiectasia

Urbino, Italy – January 07, 2016 - EryDel SpA (www.erydel.com), a Company specialized in the development of drugs and diagnostics delivered through autologous red blood cells, today announced the award of a grant of €6 millions funding from the European Union's Horizon 2020 research and innovation programme, under grant agreement No 667946, call PHC14: "New therapies for rare diseases". EryDel will coordinate the 40-month project named IEDAT (Intra Erythrocyte Dexamethasone in the treatment of Ataxia Telangiectasia).

The IEDAT consortium also includes the Universita' degli Studi di Urbino Carlo Bo (Italy), the AT Center at the Johns Hopkins University (USA), the Sheba Medical Center (Israel), the Johann Wolfgang Goethe Universität (Germany), the Ataxia Telangiectasia Society (UK) and Absiskey (France).

The funding will support a Phase III clinical trial for EryDel's experimental combination product EryDex System (EDS), for the treatment of Ataxia Telangiectasia (AT), a rare progressively disabling and life-shortening genetic disease for which no therapy is currently available.

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 reinfused into the patient. EDS has received Orphan Drug designation for the treatment of AT both from the FDA and the 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 phase III trial will be an international, multi-centre, randomized, prospective, double-blind, placebo-controlled study, designed to assess the effect of 2 dose ranges of EDS, on neurological symptoms of AT patients.

An international patient registry will also be set up, with the aim of establishing and maintaining a comprehensive clinical database of patients with AT and closely related conditions, enabling the monitoring of AT epidemiology, the development of an evidence-based natural history of the condition, identification of biomarkers as well as development of clinical guidelines.

Additional activities will involve testing of the AT-NEST scale and of a possible biomarker predictive of treatment efficacy.

William Davis, CEO of the AT Society commented: "This is terrific news for families living with AT. This is the first ever large-scale clinical trial of a treatment that could have a significant impact on the course of AT. The patient registry, too, will help us better understand and treat this devastating condition. This shows what can be achieved when companies, universities, clinicians and patient organisations work together. It will give thousands of people new hope for the future."

Luca Benatti, EryDel's CEO, commented: "We are excited to have the support of the European Union Horizon 2020 program for the development of the EDS. AT is a relentlessly progressive disease for which no therapy is currently available. EryDel, with the support of Patients Associations, clinicians and AT experts worldwide is committed to complete regulatory development and bring this innovative therapy to market".

About Ataxia Telangiectasia

Ataxia Telangiectasia (AT) is a rare genetic disease caused by biallelic 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 result in a multi-systemic disorder, mainly involving the nervous and immune systems. The major clinical feature of AT 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 delayed 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 Drug Delivery Company specialized in the development of drugs and diagnostics delivered through red blood cells (RBCs) by using a proprietary medical-device technology. The most advanced product, EryDex (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. EryDex has received Orphan Drug designation for the treatment of AT both from the FDA and the EMA. A recently completed pilot Phase II trial in AT patients demonstrated statistically significant efficacy of EryDex 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.

For further information, please contact: Emanuela Germi at +39 02 36504470 or *emanuela.germi@erydel.com*