**EryDel and Orphan Technologies collaborate to develop therapy for mitochondrial neurogastrointestinal encephalomyopathy (MNGIE)**

Bresso (Milano), Italy – June 4th, 2018 - EryDel SpA, a biotech company specialized in the development of drugs and diagnostics delivered through autologous red blood cells and Orphan Technologies Limited (OT), a biotech company dedicated to developing first-in-class therapeutics and life changing therapies for patients with rare disorders, announced the signing of a licensing and supply agreement to develop a novel treatment for mitochondrial neurogastrointestinal encephalomyopathy (MNGIE), a progressive and devastating metabolic disease.

The primary goal of this collaborative effort is to enhance the development of OT's Erythrocyte Encapsulated Thymidine Phosphorylase (EE-TP) product for the treatment of MNGIE by using EryDel's automated autologous red blood cell technology.

MNGIE is a rare autosomal recessive inherited metabolic disease that affects the digestive and nervous systems and leads to death before the age of 40. Patients are unable to break down thymidine and deoxyuridine due to defects in the gene responsible for the production of TP causing it to build up in the cells and impair mitochondrial function. The current standard of care treatment is palliative.
EE-TP which is exclusively licensed to OT, is being developed in collaboration with St George's University of London. The technology introduces TP into a patient's own red blood cells.

Patients with urgent medical needs have already been given EE-TP through compassionate use treatment. Administration of EE-TP was reported to be effective in reducing the elevated plasma and urine concentrations of thymidine and deoxyuridine, toxic substances that accumulate in tissues of MNGIE patients.

EryDel has developed a proprietary technology that allows reproducible and fully automated encapsulation of TP enzyme into patient erythrocytes. A similar technology, applied to the slow and constant release of dexamethasone is currently in a Phase 3 registration trial conducted by EryDel for the treatment of ataxia telangiectasia (AT), a rare neurological disease.

Luca Benatti, EryDel's Chief Executive Officer, commented: "We are very pleased to announce the signing of this collaboration with Orphan Technology. This partnership represents an important validation of EryDel's technology for the encapsulation of therapeutic enzymes and opens opportunities to expand our platform beyond our lead product for the treatment of ataxia telangiectasia."

Frank Glavin, CEO of Orphan Technologies said: "This collaborative effort, with contributions from Erydel, St. Georges' and Orphan Technologies, represents a true step forward in the evaluation of this promising opportunity to treat patients suffering from MNGIE."

**About mitochondrial neurogastrointestinal encephalomyopathy**
*Mitochondrial Neurogastrointestinal Encephalomyopathy (MNGIE) is a rare autosomal recessive inherited disease caused by defects in a gene responsible for the production of thymidine phosphorylase (TP). Patients are unable to break down thymidine and deoxyuridine, causing it to build up in the cells, where they destroy mitochondrial DNA. The disease affects mainly the digestive and nervous systems. Symptoms can appear at any time from birth but usually start during the second decade of life, and worsen with time. MNGIE is a debilitating disease that is long lasting and life threatening due to its effects on gut movement and on the nervous system including the brain. Abnormalities of the digestive system are among the most common and severe features leading to extreme weight loss, reduced muscle mass, progressive gastrointestinal dysmotility (early satiety, nausea, dysphagia, reflux, abdominal pain diarreha), cachexia (loss of weight, fatigue, muscle atrophy), ophthalmoplegia and demyelinating peripheral neuropathy (paresthesias, tingling, numbness, pain) Leukoencephalopathy (deterioration of brain white matter) is the hallmark of the disease although usually do not cause symptoms.*

**About EryDel**

*EryDel SpA is a biotechnology company specialized in the development of drugs delivered through red blood cells (RBCs) by using a proprietary medical device technology. Its most advanced product, EryDex System (EDS) is under late stage development 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 completed pilot Phase II trial in AT patients demonstrated statistically significant efficacy of EDS on both the primary and secondary efficacy measures. An international multi-center, Phase III pivotal study, ATTeST, is being conducted. EryDel has a pipeline of preclinical programs that use its proprietary RBC's delivery technology for the treatment of other rare diseases.*

*The ATTeST project* has received funding from the *European Union's Horizon 2020 research and innovation programme* under grant agreement No 667946".

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**About Orphan Technologies**

*Orphan Technologies Limited is dedicated to developing novel therapies to dramatically improve the lives of patients suffering from the rare disorder, homocystinuria and related diseases. OT-58, our lead drug development candidate, has been optimized as an enzyme replacement therapy for classical homocystinuria, a genetic disease characterized by debilitating cardiovascular, skeletal, neurologic, and ophthalmologic complications. OT-58 is designed to reduce homocysteine levels via a targeted mechanism of action and may have therapeutic applications in other diseases. For more information, please visit www.orphantechnologies.com*