EryDel Announces Completion of Patient Enrollment in Phase 3 ATTeST Trial the Largest Clinical Study Ever Conducted in Ataxia Telangiectasia (AT)

Bresso (Milano); Italy, - April 14, 2020 - EryDel SpA, a global late-stage biotech company aimed at developing and commercializing therapies for the treatment of rare diseases delivered by its proprietary red blood cell technology, announced the completion of patient enrollment of ATTeST, the Phase 3 pivotal trial in Ataxia Telangiectasia (AT). Topline data from the study are expected at the end of 2020.

"This is a significant milestone for the AT community who have collaborated with us in this endeavor" stated Luca Benatti, Ph.D., Chief Executive Officer. "Positive data would enable EryDel to file our first NDA in the second half of 2021 and would represent an important step toward becoming a fully integrated company that can bring innovative therapies to patients."

"We have completed enrollment of ATTeST, the largest clinical study ever conducted in AT", said Guenter R. Janhofer, M.D., Ph.D., EryDel's Chief Medical Officer. "We thank all of the patients in this trial and their families, who have sacrificed time and energy to participate in this trial. We are also deeply grateful for the support we have received from investigators and the clinical site staff, as well as for the extensive participation of the patient associations." Janhofer continued, "following the positive Phase 2 study we hope that the ATTeST trial will confirm the effect of EryDex on improving neurological symptoms and potentially delaying disease progression."

"Treatment of the neurologic problems associated with AT is paramount to improve the lives of our patients and their families, as there are no treatments known today to slow or stop the neurological decline. Therefore, we look forward to seeing the data from ATTeST, when it becomes available later this year" said Howard Lederman, M.D., Ph.D., Professor of Pediatrics at the Johns Hopkins University School of Medicine, and director of the Ataxia Telangiectasia Clinical Center. Dr. Lederman is an investigator in the ATTeST trial along with 21 other investigators in 11 countries on 5 continents.

About ATTeST

ATTeST (**A**taxia **T**elangiectasia **T**rial with the **E**ryDex **S**ys**T**em), is a pivotal Phase 3 trial to evaluate the efficacy, safety and tolerability of EryDex in patients with Ataxia Telangiectasia (AT). EryDex is an innovative product that delivers dexamethasone by encapsulating its pro-drug into red blood cells taken from the patient's own blood, which are then re-infused into the patient. EryDex has received Orphan Drug designation for the treatment of AT both from FDA and EMA.

About EryDel

EryDel SpA, a global, late-stage biotech company aimed at developing and commercializing therapies for the treatment of rare diseases delivered by its proprietary red blood cell (RBC) technology. Its most advanced product, EryDex is under late stage development for the treatment of Ataxia Telangiectasia, a rare autosomal recessive disorder for which no established therapy is currently available. A completed Phase II trial in AT patients demonstrated statistically significant efficacy of EryDex on both the primary and secondary efficacy

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measures. An international multi-center, Phase III pivotal study, ATTeST, is currently being conducted. EryDel has a pipeline of preclinical programs that use its proprietary RBC delivery technology for the treatment of other rare diseases including enzyme replacement therapies.

About Ataxia Telangiectasia

AT is a rare, hereditary, life-threatening, progressive and highly disabling disorder affecting predominantly pediatric patients. Presently no cure or effective therapy for modifying and/or to significantly impact disease progression is available. The major clinical feature of AT is severe progressive neurodegeneration from early infancy. Specific features include progressive ataxia, involuntary movements, oculomotor apraxia, difficulties with speech and swallowing. Other clinical features include immunodeficiency with recurrent respiratory tract infections. The therapeutic landscape for AT management is limited to palliative, multidisciplinary supportive care in order to treat individually the non-neurological symptoms associated with AT, the major contributors to the severe outcome of the disease.

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