EryDel Announces Top-line Results from Phase 3 ATTeST Trial Demonstrating Significant Clinical Benefit of EryDex in Ataxia Telangiectasia

Bresso (Milano); Italy – July 12, 2021 - 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 today topline results from its Phase 3 ATTeST clinical trial in patients with Ataxia Telangiectasia (AT), a devastating neurological disease for which there is no approved therapy.

The ATTeST trial was a pivotal multicenter, double-blind and placebo-controlled study conducted in the United States, Europe, Asia, Australia and Africa. 175 patients age 6 and above with AT were enrolled and randomized to either high or low dose of EryDex or placebo treatment in a 1:1:1 ratio. The treatment duration was 6 months for the efficacy and extended to 12 months for long term safety assessment.

The primary endpoint was the change of the modified International Cooperative Ataxia Rating Scale (mICARS), for the ex-US and the Rescored mICARS (RmICARS) for the US, from baseline to month 6 compared to placebo. The pre-specified ITT analysis of patients age 6 to 9 (N= 88) showed statistically significant differences vs. placebo for the primary endpoint (mICARS p=0.020 and RmICARS p=0.025). These results were also supported by analysis of the key secondary end point, the clinical global impression of change (CGI-C, p=0.04), and a patient self-assessment of quality of life as measured on the visual analogue scale of the EuroQol Instrument further supported the meaningfulness of this Improvement.

The 6-month safety analysis (which is also including safety information of patients who have, at time of database lock, completed up to months 12) showed that EryDex is well tolerated and did not identify any alarming safety signals that might be expected with chronic steroid use as confirmed by an Independent Data Safety Monitoring Board.

“The findings of the ATTeST study for the younger patients open, for the first time ever, the door to an effective and safe treatment for our AT patients. This is a very promising outcome for the whole AT community, which had been without any viable treatment option so far” commented **Howard Lederman**, MD, PhD, Professor of Pediatrics, Medicine and Pathology at the Johns Hopkins University School of Medicine and Principal Investigator of the ATTeST study at the Johns Hopkins Hospital in Baltimore.

Dr. Stefan Zielen, Professor of Allergology, Pneumology and Mucoviscidosis and Principal Investigator at the Department for Children and Adolescents, Goethe University of Frankfurt, Germany added “Ataxia Telangiectasia is a devastating disease starting in early childhood that is affecting patients around the world with no perspective for meaningful therapeutic intervention. Natural history data shows that it is progressing rapidly particularly in children under age 10. These results suggest a remarkable slowing done of this progression and mean new hope for improving the lives of our patients and their families”.

Guenter R Janhofer, MD, PhD, Chief Medical Officer of EryDel, concluded from these findings that "the statistically significant and clinically meaningful efficacy results for EryDex in Ataxia Telangiectasia offer, for the first time, hope to patients and caregivers affected by AT. After completion of analysis of the 12-month safety data EryDel will engage with the FDA to agree on the next steps for preparing a New Drug Application (NDA), followed by regulatory submissions in Europe and selected other countries".

About Ataxia Telangiectasia (AT)

Ataxia Telangiectasia is a rare genetic disease caused by biallelic mutations in the ataxia telangiectasia mutated (ATM) gene, for which no established therapy is currently available. 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, immunodeficiency with recurrent respiratory tract infections, radiosensitivity and an increased incidence of cancer.

About EryDel

EryDel SpA is 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. 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. EryDex is an automated outpatient bedside technology to ex-vivo encapsulate dexamethasone sodium phosphate (DSP; an inactive pro-drug) into patient's red blood cells, which are then re-infused, allowing the slow release in circulation of low doses of dexamethasone (active drug) over a month. EryDex has received Orphan Drug designation for the treatment of AT both from the FDA and the EMA. An international multicenter, Phase 3 pivotal study, ATTeST, has been successfully completed and regulatory filing is under preparation.

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