EryDel Provides Regulatory Update on EryDex for the Treatment of Ataxia Telangiectasia

- **European filing in 2H 2023**
- **US regulatory path defined: Special Protocol Assessment (SPA) Agreement with the FDA**

Bresso (Milano); Italy – February 22, 2023 - **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 that the company is preparing a European Marketing Authorization Application (MAA) for submission in H2 of 2023 for EryDex for the treatment of Ataxia Telangiectasia (AT). AT is a rare pediatric neurodegenerative disorder with no approved therapy available. Following a positive consultation with the European Medicines Agency (EMA), subsequent correspondence concluded that the data package available for submission is comprehensive and meets the requirements for the regulatory authority to complete its review of EryDex.

In addition, EryDel has reached an agreement with the U.S. Food and Drug Administration (FDA) under the special protocol assessment (SPA) process on the design, endpoints and statistical analysis of a new Phase 3 clinical trial (the NEAT Trial). The SPA provides that the design and planned analysis of the trial, as set forth in the protocol submitted to the FDA, adequately addresses the objectives necessary to support the submission of new drug application for EryDex.

"We are pleased to have completed significant steps, which clarify the regulatory path for EryDex for the treatment of AT," said Luca Benatti, Chief Executive Officer of EryDel. "In Europe we are on track to file the European MAA and in the U.S., we have moved ahead with the FDA on the SPA. These are major milestones as we progress our work to bring this valuable therapy to the AT community."

EryDel will continue to work with both the FDA and EMA to progress EryDex towards filing. The EU Marketing Authorization Filing is expected in 2H2023.

About Ataxia Telangiectasia

Ataxia Telangiectasia (AT) is a rare, inherited, autosomal recessive, devastating multisystem disorder. AT has a worldwide prevalence estimated to be between 1 in 40,000 to 1 in 100,000 live births (Rothblum-Oviatt et al., 2016). AT is a condition with high morbidity and mortality. The disease is characterized by progressive cerebellar degeneration, leading to increasing impairment of motor function during the early school years and wheelchair dependency in the second decade of life. Other features include telangiectasia, immunodeficiency, recurrent pulmonary infections, radiation sensitivity and a

predisposition to the development of malignancies. There is currently no approved treatment option for this devastating disease.

About EryDel SpA

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 (AT), a rare autosomal recessive neurological 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, and its long-term extension have been successfully completed and EU regulatory filing is under preparation. In addition to EryDex, EryDel's technology platform in enzyme replacement therapy supports a wide range of therapeutic opportunities led by Ery-PAL for the treatment of PKU.

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