Phase II results in Ataxia Telangiectasia published in Orphanet Journal of Rare Diseases

Urbino, Italy – February 20, 2014 - EryDel SpA (www.erydel.com), a Drug Delivery Company specialized in the development of drugs and diagnostics delivered through autologous red blood cells, today announced the publication in Orphanet Journal of Rare Disease (Chessa et al. 2014) of the results of a Phase II six-month study in Ataxia Telangiectasia patients showing that treatment with EryDex led to a significant improvement in neurological symptoms without association with the typical steroid side effects.

An improvement in the primary end-point, as measured by the ICARS scale (reduction of the score) was detected in the intention-to-treat (ITT) population ($n = 22$; $p = 0.02$) as well as in patients completing the study (per protocol PP) ($n = 18$; $p = 0.01$), with a mean reduction of 4 points (ITT) or 5.2 points (PP). When compared to baseline, a significant improvement was also observed in VABS ($p < 0.0001$, ITT, RMANOVA), with statistically significant increases at 3 and 6 months ($p < 0.0001$). EryDex was well tolerated; the most frequent side effects were common AT pathologies.

The complete electronic version of this article can be found at http://www.ojrd.com/content/9/1/5.

About Ataxia Telangiectasia

Ataxia Telangiectasia (AT) is a rare autosomal recessive disorder with onset in the first years of life. AT is characterized by progressive cerebellar ataxia and oculomotor apraxia, oculocutaneous telangiectasias (90% of patients), recurrent sinopulmonary infections (70%), predisposition to cancer, increased alpha-fetoprotein levels (95%), and reduced or absent IgA levels (70%) and ATM protein (98%) (data from the Italian Registry for AT). The causative defective gene, ATM, cloned in 1995, encodes a PIK3 protein shown to play a pivotal role in the response to DNA damage and in the cell cycle control. As a consequence of the DNA repair deficiency, infections are the most common cause of death, and cancer the second most common. Most patients with AT die in the second decade of life, although some individuals survive longer. Neurological degeneration is the major contributor to the severe outcome of the disease. No established therapy is currently available; treatments are symptomatic and supportive only.

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. EryDel has recently completed a €15m series B financing led by Genextra SpA. The proceeds will be used to progress the planned development, discussed with regulatory agencies, of EryDex in the treatment of neurological symptoms of Ataxia Telangiectasia. EryDel is planning a single, comprehensive, confirmatory, placebo-controlled trial that will determine the efficacy and safety of EryDex in AT. A recently completed pilot Phase II trial demonstrated statistically significant efficacy of EryDex on both the primary and secondary efficacy measures. EryDel has obtained Orphan Drug designation for EryDex in the treatment of AT both from the FDA and the EMA.