Long term benefit of erythrocyte-delivered dexamethasone in Ataxia Telangiectasia

Urbino, Italy – May 19, 2015 - EryDel SpA (www.erydel.com), a Company specialized in the development of drugs and diagnostics delivered through autologous red blood cells, today announced the publication in Neurology Neuroimmunology & Neuroinflammation (Leuzzi et al. 2015) of the results of a 2-year extension study with EryDex System (EDS) in patients with ataxia telangiectasia (AT).

In a previous phase II study (Chessa et al. 2014), we showed that 6 monthly infusions of EDS were effective in improving neurologic impairment in patients with AT. The paper by Leuzzi et al., reports the results of the extension of this study for an additional 24-month period.

After the end of the first trial, 4 patients continued to be treated with monthly EDS infusions for an additional 24 months, and their clinical outcome was compared with that of 7 age-matched patients who stopped the treatment after the first 6 infusions. The protocol included serial assessment of ataxia (by International Cooperative Ataxia Rating Scale) and adaptive behavior (by Vineland Adaptive Behavior Scales) and clinical and laboratory tests revealing treatment- and steroid-dependent adverse effects, if present.

Patients in the extended study experienced a continuous neurologic improvement with respect to their pretreatment status, whereas controls showed a progressive neurologic deterioration (according to the natural history of the disease) after the discontinuation of the treatment. The EDS proved to be safe and well tolerated, and none of the side effects usually associated with the chronic administration of corticosteroids were observed during the entire trial.

About Ataxia Telangiectasia

Ataxia Telangiectasia (AT) is a rare genetic disease caused by biallelic mutations in the ataxia telangiectasia mutated (ATM) gene, for which no established therapy is currently available. ATM encodes a PI3Kinase protein shown to play a pivotal role in response to DNA damage and cell cycle control. Homozygosity or compound heterozygosity for ATM mutations result in a multi-systemic disorder, mainly involving the nervous and immune system. The major clinical feature of AT is severe progressive neurodegeneration with onset in infancy. These features include ataxia of the trunk and limbs, progressive supranuclear ophthalmoplegia, dysarthria, swallowing incoordination, facial hypomimia and delayed peripheral neuropathy. Other clinical features of patients with the classical phenotype include oculocutaneous telangiectasia, immunodeficiency with recurrent respiratory tract infections, radiosensitivity and an increased incidence of cancer.

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. EryDex received Orphan Drug designation for the treatment of AT both from the FDA and the EMA. A recently completed pilot Phase II trial in AT patients demonstrated statistically significant efficacy of EryDex on both the primary and secondary efficacy measures. EryDel has a pipeline of preclinical programs that use its proprietary RBC's delivery technology for the treatment of other rare diseases.

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