ESTEVE and UAB expand their research to two new gene therapies for Sanfilippo B syndrome and Hunter syndrome

ESTEVE and the Universitat Autònoma de Barcelona (UAB) create a promising platform of gene therapy projects with the addition of two new therapies for Sanfilippo B (EGT-201) and Hunter syndromes (EGT-301).

The EMA and the FDA recently granted orphan drug designation to EGT-301.

These two new therapies join the leading program EGT-101 for the treatment of Sanfilippo A, whose clinical trial will start in late 2016.

25 February, 2016.- ESTEVE strengthens its gene therapy platform with the addition of two new investigational gene therapies, EGT-201 for the treatment of Sanfilippo B syndrome and EGT-301 for the treatment of Hunter syndrome, both developed in collaboration with the group of Professor Fàtima Bosch at the Universitat Autònoma de Barcelona (UAB). EGT-201 and EGT-301 join EGT-101, designed to treat Sanfilippo A syndrome, to create a promising gene therapy platform.

ESTEVE announced that the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) have granted Orphan Drug Designation to its novel gene therapy program EGT-301 for the treatment of Hunter syndrome or mucopolysaccharidosis II (MPS II). EGT-301 consists of an adeno-associated viral vector of serotype 9 (AAV9) containing the human Iduronate-2-sulfatase (I2S) transgene designed to restore I2S functional deficiency in patients with Hunter syndrome. ESTEVE is currently initiating regulatory preclinical development of EGT-301.

EGT-101, the lead project in ESTEVE's gene therapy platform, consists of an AAV9 vector containing the human sulfamidase (SGSH) transgene and it aims to restore SGSH functional deficiency in patients with Sanfilippo A syndrome. ESTEVE plans to initiate phase I/II clinical trials for EGT-101 by the end of 2016. EGT-101 received orphan drug designation by the FDA and EMA in 2011.

EGT-201 consists of an AAV9 vector containing the alpha-N-acetyglucosaminidase (NAGLU) transgene and it aims to restore NAGLU functional deficiency in patients with Sanfilippo B syndrome. ESTEVE is currently initiating regulatory preclinical development of EGT-201. EGT-201 received orphan drug designation by the FDA and EMA in 2013.

"*With the addition of EGT-201 and EGT-301 to create a strong pipeline of projects, ESTEVE reaffirms its commitment to developing gene therapies to treat severe and debilitating rare diseases. ESTEVE plans to expand its platform to other currently untreated disease conditions with the aim of bringing cures to underserved patients and becoming a reference in the field of gene therapy for rare diseases*," said **Dr. Carlos Plata**, Chief Scientific Officer at ESTEVE.

"*The Orphan Drug Designations obtained for all three projects underscore the excellence of the preclinical discovery capabilities at the UAB, while reinforcing the public-private partnership between the University and ESTEVE*", said **Fàtima Bosch**, PI of the project and Director at the Center for Animal Biotechnology and Gene Therapy (CBATEG) and Full Professor at the UAB. *"We are excited to continue our collaboration with ESTEVE in the quest to develop and bring novel gene therapy cures for rare diseases to patients in need, and to that end we have recently renewed and strengthened the collaboration between CBATEG/UAB and ESTEVE".*

These projects have received financial support from the Spanish Ministry of Health, Social Policy and Equality, and from the Spanish Ministry of Economy and Competitiveness.

About Sanfilippo syndromes Type A and B, and Hunter syndrome (MPS III A, MPS III B and MPS II)

Sanfilippo Type A and B and Hunter syndromes are lysosomal storage disease in which a given enzyme has lost its functional activity, leading to the accumulation of glycosaminoglycan substrates. This leads to a disruption in cellular function in multiple tissues and organs, amongst them the brain.

Sanfilippo A and B syndromes are devastating diseases that lead to progressive and significant deterioration in the mental status of children who rarely live beyond their adolescence. Sanfilippo A is caused by the loss of the activity of the enzyme Sulfamidase (SGSH), while in Sanfilippo B there is a deficiency in alpha-N-acetyglucosaminidase (NAGLU). Sanfilippo A affects approximately 1 in 100,000 births, while in Sanfilippo B the incidence is 0.5 in 100,000 births. Both are often diagnosed once the symptoms and signs have begun to appear.

Hunter syndrome is caused by the X-linked deficiency in iduronate-2-sulfatase. It affects 0.4 in 100,000 births and patients may present symptoms ranging from mild to severe. In all cases, quality of life may be significantly affected and in the severe cases, life expectancy is heavily reduced like in the case of Sanfilippo A and B.

About Orphan Drug Designation

Orphan Drug Designation is a regulatory status assigned to medicines intended for use in rare diseases, those that affect fewer than 200,000 individuals in the US or with a prevalence of no more than 5 in 10,000 people in the EU, or for drugs that are not expected to recover the development and marketing costs. Orphan status provides sponsors with development and commercial incentives for designated drug products. Medicines with Orphan Drug Designation follow the standard regulatory processes required in the path for obtaining marketing approval. Sponsors must establish safety and efficacy of a compound in the treatment of a disease through adequate and well-controlled studies.

Public-private partnership ESTEVE-UAB

The gene therapy platform was initiated by the research team of Professor Fàtima Bosch at the CBATEG of UAB and since 2009 is being developed within the framework of a public-private partnership between ESTEVE and the University. In this partnership, ESTEVE leads all activities associated with the management and protection of intellectual property, regulatory activities, the coordination and supervision of GMP manufacturing, the preclinical toxicology studies as well as all clinical development. The CBATEG research team at the UAB brings to the partnership their scientific know-how and expertise in gene therapy including viral vector design and the development of preclinical disease models. CBATEG/UAB and ESTEVE have recently strengthened their collaboration by signing a long-term contract to pursue the discovery and development of gene therapies for other rare diseases.

About CBATEG of the Universitat Autónoma de Barcelona

The research team led by Dr. Fátima Bosch at the Center for Biotechnology and Gene Therapy (CBATEG) of UAB, investigates the pathophysiological causes of diabetes mellitus using transgenic animal models and develops gene therapy approaches for this disease based on genetic engineering of tissues with viral and non-viral vectors. In recent years, the group has applied its expertise to the development of gene therapies for inherited metabolic diseases such as Mucopolysaccharidosis.

About ESTEVE

ESTEVE (www.esteve.com) is a leading chemical-pharmaceutical group in Spain with a strong international presence. Founded in 1929 and chaired over by Joan Esteve, it currently employs 2,279 people. It has facilities and subsidiaries in Europe, USA, Mexico and China, and had a sales revenue of 870 million Euros in 2015. Firmly committed to excellence, the Company uses its best efforts to promote health and improve people's quality of life. Research being ESTEVE's hallmark, a portfolio of highly innovative projects ultimately aims to provide responses to unmet medical needs. ESTEVE being a socially responsible Company, it ensures that all projects entered by it align with its CSR vision. You may follow ESTEVE at Twitter's link: @ESTEVE_news

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