

Amgen to work with Italian scientists on new gene therapy

Friday 16 September 2016

Amgen Inc has signed a research agreement with two Milan-based institutions to develop an *ex-vivo* gene therapy for cancer. The research will be based on some of the same principles that guided the development of Strimvelis, an *ex vivo* stem cell gene therapy that was approved by the European Commission in May of this year.

The research collaboration is with the San Raffaele Hospital and Scientific Institute, and a two year old biotech company called Genenta Science srl. The financial terms of the agreement were not disclosed.

In an interview with *MedNous*, Pierluigi Paracchi, a co-founder and the chief executive of Genenta, explained that the prospective therapy draws on work done at the San Raffaele Hospital on gene transfer therapy to treat rare diseases. The researchers' most advanced product to date is Strimvelis, which was co-developed and is being commercialised by GlaxoSmithKline Plc.

Strimvelis is a therapy for children who have an ultra-rare immune disorder called adenosine-deaminase-deficient severe combined immunodeficiency (ADA-SCID). The therapy consists of CD34+ cells taken from the patient and transduced to express the adenosine deaminase enzyme. Once the cells have been injected back into the patient, they are able to develop into blood and immune cells giving the patient an ability to produce lymphocytes and fight infection.

Genenta's prospective cancer therapy involves taking hematopoietic stem cells from a patient and transducing them with a lentiviral vector carrying the gene for interferon alpha. The modified stem cells are then reinjected into the patient where they migrate to specific monocytes and macrophages. The monocytes and macrophages then release interferon alpha into the tumour microenvironment where they can kill the tumour.

"The point about our technology is that we have the capability to express interferon alpha inside the tumour microenvironment. We change the balance inside the tumour microenvironment by supporting the immune system to fight cancer thanks to the local expression of interferon alpha," Mr Paracchi said.

Luigi Naldini, director of the division of regenerative medicine, stem cells and gene therapy at the San Raffaele Scientific Institute, is also a co-founder of Genenta. Dr Naldini is an expert in lentiviral vector based gene therapy.

Genenta was founded in July 2014 and in March 2015 it raised €10 million in a Series A financing round supported by Banca Esperia, the Private Bank of Mediobanca and Mediolanum.

The research collaboration with Amgen is the company's first pharma collaboration.

Genenta announced the agreement on 16 September 2016.

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