



Inserm announces research agreement with Pfizer Inc. to study rare neuromuscular disease

Wednesday, February 28, 2018 - 08:00am

Paris, Feb. 28, 2018 - The French National Institute of Health and Medical Research (Inserm) announces a research collaboration with Pfizer Inc. Inserm Transfert, Inserm's Technology Transfer Office, signed the agreement on behalf of several French research organizations: Inserm (Institut National de la Santé et de la Recherche Médicale), Sorbonne Université (SU previously named UPMCParis6), the University of Evry (UEVE), the Centre d'Etude de Cellules Souches (CECS) and the Institute of Myology Association (AIM).

The collaboration aims to better understand the molecular and cellular mechanisms of myotonic dystrophy, also called Steinert disease, an inherited genetic disease that mainly affects the muscles. The originality of the work is based on the use of a human patient derived cell model developed by Inserm researchers. This cell model will be used as a research tool since it recapitulates the molecular abnormalities found in the disease.

Steinert's disease is one of the most common neuromuscular diseases in adults, characterized by progressive weakness and muscle atrophy, myotonia of rhythm and cardiac conduction defects as well as endocrine and cognitive impairments. The symptoms and their evolution vary from one individual to another; available treatment options focus on symptom management, and there is no cure available for patients with myotonic dystrophy patients.

The work will be jointly carried out by Pfizer research teams and by two Inserm research laboratories: the first laboratory is led by Dr. Denis Furling, research director in unit 974 "Center for Research in Myology" and head of the "Myotonic Dystrophy, Pathology and Biotherapy" team at the Institute of Myology. This Center is under the joint supervision of Inserm, SU and AIM. The second laboratory is headed by Dr. Cécile Martinat, research

director at Unit 861, who is in charge of the "Neuromuscular Diseases" team at the Institute of Stem Cell Therapy and Exploration of Monogenic Diseases. This Unit is under the joint supervision of Inserm, UEVE and CECS.

This collaborative research project aims to coordinate the expertise and know-how of Pfizer and French public researchers to work toward the common goal of potentially identifying new therapeutic targets and to speed up translational research on this rare disease.

About Inserm Founded in 1964, the French National Institute of Health and Medical Research (Inserm) is a public science and technology institute, jointly supervised by the French Ministry of National Education, Higher Education and Research and the Ministry of Social Affairs, Health and Women's Rights. Inserm is the only French public research institute to focus entirely on human health and positions itself on the pathway from bench to bedside. The mission of its scientists is to study all diseases, from the most common to the rarest. With a budget of 989 million euros in 2014, Inserm supports more than 300 laboratories across France. In total, the teams include nearly 15,000 researchers, engineers, technicians, post-doctoral students, etc. Inserm is a member of the National Alliance for Life and Health Sciences, founded in April 2009 with CNRS, Inserm, the CEA, INRA, INRIA, the IRD, the Pasteur Institute, the Conference of University Presidents (CPU) and the Conference of Chairmen of The Regional and University Hospital Centres. This alliance forms part of the policy to reform the research system by better coordinating the parts played by those involved and by strengthening the position of French research in this field through a concerted plan. www.inserm.fr

About Inserm Transfert Founded in 2000, InsermTransfert SA is the private subsidiary of the French National Institute of the Health and Medical Research (Inserm), dedicated to technology transfer (from invention disclosure to industrial partnership). Inserm Transfert also manages European and International research projects, supports large scale projects in epidemiology and public health. Inserm Transfert runs a 2M€/year proof of concept fund. The company also supports entrepreneurs in the biotech sector, in partnership with Inserm Transfert Initiative, a € 39.7m life sciences seed investment company. www.inserm-transfert.fr