

## Post Doc Position

**Title:** Identification of modifiers of the dystrophin gene expression to restore functional proteins using HTS technology on cell progenies derived from human pluripotent stem cells

**Location :** I-stem, Evry, France

### Istem

I-stem, the Institute for Stem cell Therapy and Exploration of Monogenic diseases, established on 1st January 2005, is a research and development centre dedicated to the treatments based on the potential offered by pluripotent stem cells applicable to rare diseases of genetic origin. I-Stem is composed of two separate administrative entities of roughly equal size, one associated with public institutions (Inserm / UEVE 861) and one directly related to the AFM Telethon, a charity association (CECS, the Centre for the Study of Stem Cells). Defined by three keywords, “therapeutics”, “monogenic diseases” and “pluripotent stem cells”, the field of activity of I-Stem extends from basic research to the pathological mechanisms and transfer of new therapies to clinical research. Programs of each of the research teams are devoted either to a set of genetic diseases or the development of new technologies. Currently, the major pathological indications studied concern diseases of muscle, neurons, skin, retina, neurodegenerative diseases and those associated with developmental abnormalities of the cerebral cortex or neural crest and diseases characterized by accelerated aging. Research teams explore technological tools, especially cells production in bulk and screening of chemical compounds. Research teams permanently interact with each other around common projects around the technological platforms that are shared.

### **Duration:**

2 Years

### **Description:**

The objectives of the muscle disease team is to open new therapeutic avenues for muscle genetic disease such as Duchenne Muscular Dystrophy caused by lesion in dystrophin gene using cell derived from human pluripotent stem cells (hES and IPS). Recently, the Muscular disease team, led by Christian Pinset, has developed a cell system for expression of human dystrophin. This cell system based on cell progenies derived from human pluripotent stem cells (hES and hIPS) permit to reach a high level of dystrophin in only 72h. The simplicity and robustness of this cell system allow the development of high throughput screening approaches (HTS). With hips cells derived from DMD patients with different genetic lesions, the objective of the project will be the identification of modifiers of the dystrophin gene expression to restore the protein functionality using HTS technology.

**Keywords:** hES, IPS, HTS, Dystrophin, Developmental and Cell biology, Duchenne Myopathy

### **Application**

The candidate should be creative, have a background in developmental biology, he/she must have an interest in translational medicine and automated approaches. Please send us a detailed CV, including a complete bibliography and recommendation letters.

### **Contact**

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