

BIOLOGICAL RESOURCES FOR RESEARCH IN MONOGENIC DISEASES

iPS Workshop (Atelier)

In the period of just a few months, iPS, pluripotent stem cells induced by genetic reprogramming, have become major research tools for stem cells, such that all the research groups working in the field have added them to their arsenal. Moreover, the iPS cell lines, like ES cells, when they are derived from appropriate donors, may become exceptional biological resources for all those who work on monogenic diseases. Pluripotent and capable of unlimited self-regeneration, they provide a means of access to *in vitro* studies on any cell phenotype in any cellular volume. If they are donated by carriers of a genetic defect responsible for a disease, they can considerably facilitate access to an understanding of molecular and cellular mechanisms of disease, thereby opening a pathway which has previously been closed to large-scale approaches, and permitting utilization of all the technological resources for research on treatments.

**In its continuing search for all the means which lead to the
promotion of research on monogenic diseases,
the Association Francaise contre les Myopathies
proposes to provide free access to iPS cell lines
to all research groups who wish to use them to explore the
mechanisms of those diseases and their treatment.**

With this goal, it is opening an iPS Atelier in the Center for the Study of Stem Cells (CECS, **Centre d'Etude des Cellules Souches**), a laboratory financed by AFM which is integrated in the I-STEM Institute in Evry.

Research teams that will succeed in their application will be welcome to send one member to CECS together with relevant cells of interest for their program for the time needed for both developing the requested iPS cell lines and for training in undifferentiated stem cell maintenance as well as differentiation protocols of use. The iPS Atelier, a dedicated training structure on the site of the I-STEM Institute, will welcome and train that team member in genetic reprogramming, maintenance of cell lines and the differentiation of stem cells into lines of interest, while offering direct guidance for the development of iPS cell lines of interest. The iPS Atelier of CECS will provide with biological and technological resources, as well as the *savoir-faire* of teams from the Institute, free of charge. The end goal of the program is for the applicant team to obtain within a few months for its own use both the biological resources and the required specific skill.

This is a permanent grant program, and applications will be evaluated rapidly in order to avoid delaying the planned projects. The teams who wish to benefit from the iPS Atelier should submit a research project justifying their needs in the framework of studies on monogenic diseases. Once their application is accepted, they will be guided by the iPS Atelier team which will give them the technical information necessary and the time-frame for realization. The production of the cell lines and training of the methods of culture and differentiation will require the presence of a person competent in cell culture (research scientist, post-doc, thesis student, engineer or technician) full-time at the I-STEM site in Evry for a period estimated to be about three months.