



# PRESS RELEASE

## **International patient advocates partner to fund Spanish gene-therapy project to treat Friedreich's ataxia**

- Scientists at the Institute for Research in Biomedicine (IRB) and the "Centro de Biología Molecular Severo Ochoa" (CBMSO) launched a gene-therapy project to treat Friedreich's ataxia two months ago.
- The project is the result of an initiative of Spanish people affected by this rare disease who are grouped in GENEFA in collaboration with the Spanish Federation of Ataxias and the BabelFamily. The Friedreich's Ataxia Research Alliance (FARA), one of the main nonprofit organizations for FA research in the United States now joins the endeavor.
- The support provided by FARA will account for 50% of the project budget of 300,000 euros. Of note, FARA's funds come from the support of patient families and communities raising funds at a grassroots level and from other International advocates such as FARA – Ireland.

*Downingtown, PA January 28, 2014* The gene therapy-based research project to tackle Friedreich's ataxia launched in November in labs at the Institute for Research in Biomedicine (IRB), in Barcelona, and the "Centro de Biología Molecular Severo Ochoa" (CBMSO), in Madrid, has received 100,000 US\$ per year for two years from the Friedreich's Ataxia Research Alliance (FARA). FARA is an US based nonprofit organization, and since 1998 it has provided a rigorous and solid funding program for research projects all over the world that aim to tackle Friedreich's ataxia, a rare and serious hereditary neurodegenerative disease for which only palliative treatments are currently available.

The unique quality of the project is that the idea for it came from those affected by the disease, patients and relatives, who, in their endeavors to find a cure got in touch with basic research groups in order to start a long-term project. The GENEFA Platform, in close collaboration with FEDAES and the BabelFamily, started fund-raising efforts in May 2013, and in November signed an agreement with IRB and CBMSO, headed by Joan Guinovart, director of the IRB, and Margarita Salas, president of the "Fundación Severo Ochoa".

"The monthly subscriptions of members of the GENEFA Platform form the basis of the funding; however, the organization has also held a wide range of fund-raising events and activities and has been supported by donations from companies, associations, and relatives and friends of those affected by Friedreich's ataxia. We are all working hard towards finding a cure and now the collaboration of FARA guarantees the funding required

for this purpose," explains Juan Carlos Baiges, on behalf of the members of the Platform.

The project involves the research groups headed by Javier Díaz-Nido at the CBMSO, an international expert in gene therapy and Friedreich's ataxia, and Ernest Giralt at IRB, an international authority on the design of transporters, such as nanoparticles, that can carry drugs into the brain and thus overcome the blood-brain barrier. Patients with this ataxia inherit a mutated version of the frataxin gene, which causes neurodegeneration. The project seeks to rescue this defect in cells of the central nervous system.

Jennifer Farmer, Executive Director of FARA, explains "When you are working on a rare disease, critical resources, such as funding, are quite small so it is imperative that FARA and other global groups with an interest in supporting and advancing research in Friedreich's ataxia work together. We can't afford to duplicate effort or compete." She goes on to add that, "We are proud to give our support to the solid scientific project led by doctors Díaz-Nido and Giralt to identify new therapies and at the same time to strike up an alliance with patients in Spain."

To find out more about the gene therapy project read the news article: [Patients and scientists join forces to tackle Friedreich's ataxia](#) (14 November 2013)

Information about the organizations involved:

**The Friedreich's Ataxia Research Alliance (FARA)** is a public, non-profit organization based in the United States dedicated to curing Friedreich's Ataxia (FA) through research. FARA grants and programs provide support for basic and translational FA research, pharmaceutical/ biotech drug development, clinical trials, and scientific conferences. [www.CureFA.org](http://www.CureFA.org)

**GENEFA, Platform for a Friedreich's Ataxia cure** - Research on gene therapy, was set up in March 2013 by a group of people affected by the disease (patients, relatives and friends). It is a non-profit volunteer-based organization that seeks to support research efforts into finding a cure for Friedreich's ataxia. [www.genefa.org](http://www.genefa.org)

**The Spanish Federation of Ataxias (FEDAES)** is a non-profit organisation that encompasses various regional associations and forms part of other national and European federations. It seeks to promote research into ataxias. It includes and supports the GENEFA Platform, in one of its many endeavors to further efforts to improve the treatment of ataxia. [www.fedaes.org](http://www.fedaes.org)

**BabelFamily** is a non-profit organization based in Spain. It is formed by an international group of volunteers that unite skills to support the greater FA community of patients, doctors, researchers, scientists and associations in their mission to find treatments and a cure for Friedreich's ataxia. [www.babelfamily.org/en/](http://www.babelfamily.org/en/)

**INSTITUTE FOR RESEARCH IN BIOMEDICINE (IRB)** is one of eight centres in Spain to be first recognized as a "Severo Ochoa Centre of Excellence" by the Spanish government. The 23 groups hosted by the institute are devoted to basic and applied research with the common goal of conducting multidisciplinary projects that address important biomedical problems affecting our society. The institute is home to approximately 440 employees from 38 countries. [www.irbbarcelona.org](http://www.irbbarcelona.org) / @IRBBarcelona

/ [www.facebook.com/irbbarcelona](http://www.facebook.com/irbbarcelona)

**CENTRO DE BIOLOGÍA MOLECULAR SEVERO OCHOA (CBMSO)** was founded in 1975 as a mixed centre between the "Universidad Autónoma de Madrid" (UAM) and Spanish National Research Council (CSIC) with the mission to promote research of excellence in Molecular Biology. Its current facilities, a new building on the Cantoblanco campus of the UAM, were opened in January 2008. The CMBSO comprises over 70 groups devoted to various aspects of biomedical and organised into 5 departments. The Severo Ochoa Foundation (FSO) furthers research efforts in the field of Molecular Biology, especially with regard to CBMSO but also throughout Spain. [www.cbm.uam.es/](http://www.cbm.uam.es/) [www.fsochoa.es/](http://www.fsochoa.es/)

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