

# GENE THERAPY

Searching for a cure for a rare genetic disease:  
Wolfram Syndrome

A small group of families and the UAB come together in a pioneering project, which is currently the only one the world.



**AFASW**  
Alianza de Familias Afectadas  
por el Síndrome de Wolfram

# GREETINGS FROM AFASW!

We are a small association of **families** affected by a serious neurodegenerative genetic disease: Wolfram Syndrome (also called DIDMOAD).

We are promoting a huge project, with our own resources and **with the help of many people like you.**

A state-of-art gene therapy research project unique in the world with the aim of getting treatment for this illness.



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# THE RESEARCH

This project, based on gene therapy, will be carried out in Spain by the UAB (a Spanish University based on Barcelona) and consists of three phases:

- Preclinical proof of concept in in vitro cells.
- Preclinical proof of concept in animal model
- Request for the authorization of designation of Orphan Medication to the EMA & FDA.

*"This project, driven by private initiative, is currently the most advanced research in the fight against this disease"*



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# RESEARCH TEAM

The project will be carried out by two high-level Spanish researchers with more than 25 years of experience in gene therapy.

This same research team currently has similar projects underway for other diseases in very advanced stages of research.

## Research Team

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**Assumpcio Bosch**  
UAB Professor

- Instituto de Neurociencias; Dept Bioquímica y Biología Molecular Universitat Autònoma Barcelona
- Instituto de Investigación del Hospital Vall d'Hebrón; Barcelona



**Miguel Chillon**  
ICREA Researcher

- Instituto de Investigación del Hospital Vall d'Hebrón; Barcelona
- Instituto de Neurociencias; Dept Bioquímica y Biología Molecular; Universitat Autònoma Barcelona

## **WHO WILL BENEFIT FROM THIS INITIATIVE?**

Medical experts estimate between 15,000 and 30,000 patients worldwide have this disease. Among them the promoters of the project. We hope that the investigation will advance to find a treatment.

## **WHAT WILL I BE SUPPORTING WITH MY COLLABORATION?**

With your contribution, you will help the advanced research in gene therapy for Wolfram Syndrome and achieve a cure for the disease.

## **HOW CAN I COLLABORATE?**

### **Wire transfer**

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Swift CCRIES2AXXX

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### **Online Donation**

