

# 143 - Fondazione Telethon meets the Patients' Associations needs through the development of a "seed grant"

ALESSANDRA CAMERINI\*, ALESSANDRA ZATTI, ERMANNO RIZZI, ELENA BRUNO, MANUELA BATTAGLIA- FONDAZIONE TELETHON, MILANO (ITALY)

## BACKGROUND

How to understand whether a scientific research project is of value or not? How to choose one project over another? Which scientists to trust in? These are key questions of rare disease patient organisations. Fondazione Telethon decided to address these needs through the development of the new initiative, "Seed Grant" Project: a call for grant proposals dedicated to Patients' Association able to invest a full budget in a research project. Fondazione Telethon supports the association in all the phases of the research project selection focusing on the disease of interest.



## THE PILOT PROJECT

**The Pilot “Seed Grant” combined the expertise of Fondazione Telethon with the work of the Italian Glut1 Association, to give new perspectives in research on the glut1 deficiency syndrome.**

The patients’ Association and Telethon defined a call focused on the Glut1 deficiency syndrome to support a 1-year scientific project with 50.000 €. An “ad hoc” panel of experts was formed, made up of 4 international researchers. The call opened on Sept 2019: 13 proposals were received; the panel selected the 3 most deserving projects. On January 2020, the Association, with the support of Telethon, decided the best project to fund.

### THE RESEARCH VIEW

The winning project is coordinated by prof. Federico Zara, of Medical Genetics of the Gaslini Institute and University of Genoa. The project aims to develop new molecules capable of increasing the permeability of the blood-brain barrier to glucose and of increasing the expression of the deficient transporter Glut1 and therefore of testing its effectiveness in an innovative experimental platform based on the use of patient cells. The project therefore responds to the growing demand for personalized medicine in the field of rare diseases.

### THE PATIENTS’ ORGANIZATION VIEW

“This initiative is fundamental for our association. As a small association of patients and caregivers, fundraising is never easy, and every hard-earned cent needs to be spent rationally. And while one of our major goals is to support research, we realize that we can’t conduct an independent assessment the true scientific value of research proposals on our own – said the President of the Association- With this in mind, we reached out to Telethon for support in launching the call for proposals, evaluating those that arrived. With the help of Telethon we can therefore guarantee our families and our donors that our funds, collected with great effort, will be spent in a manner consistent with our objectives. We are grateful for this opportunity and excited for the start of the research project”.

## NEXT STEP

AFTER THE PILOT PROJECT AND GIVEN THE SUCCESS AND INTEREST RAISED, TELETHON DECIDED TO EMBED THE SEED GRANT CALL IN ITS YEARLY FUNDING INITIATIVES.



1

TO DATE, 10 ASSOCIATIONS EXPRESSED THEIR INTEREST IN SOWING THEIR SEED (GRANT), WITH THE NECESSARY BUDGET AVAILABLE. COLLABORATING WITH TELETHON TO DEVELOP THE BEST POSSIBLE RESEARCH.



2

ONLY WITH HIGH STANDARDS OF QUALITY AND EXCELLENCE IT WILL BE POSSIBLE TO BUILD THE BEST RESEARCH AND TO COLLECT MATURE RESULTS IN FUTURE.



3