January 19th, 2021

Kind attention of:
Paola Bona, President
AISEA – Associazione Italiana Sindrome Emiplegia Alternante
bona@aiseaonlus.org

Bridget Vranckx, President
AESHA - Asociación Española del Síndrome de la Hemiplejia Alternante
bridget.vranckx@aesha.org

Dominique Poncelin, President
AFHA - Association Française de l'Hémiplégie Alternante
d.poncelin@gmail.com

Tony Pena, President
CureAHC Foundation
ajp1896@aol.com

Simon Frost, President
Hope for Annabel Foundation
simon@tibercapital.com

Joshua Marszalek, President
AHC Foundation
joshua@ahckids.org

Dear All,

Following the official communication by La Marató de TV3, we are very happy and proud to inform you that "**TREAT-AHC. Identification of compounds for the treatment of Alternating Hemiplegia of Childhood by drug repositioning. Molecular and pre-clinical evaluation and clinical outcome measure validation**", is one of the 41 projects selected for granting by the Foundation, out of 226 applications. To our knowledge, this is among the first projects on AHC that have been granted by third Institutions, not directly involved with patient associations.

The project involves the groups of the persons signing the present letter and has received a grant of 395,500 € in 3 years. This is a very ambitious project aimed at leading into clinical trials, candidate compounds to the treatment of AHC, identified by *in vitro* experiments and validated in animal models. The moving from basic to translational research will require a high degree of readiness and awareness of the condition, that will be provided by the long-lasting experience of our units.

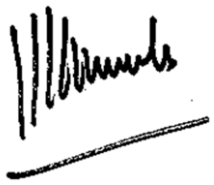
As you might be aware, this project has been started some years ago with the development of a cell model of AHC, conceived for both getting clues on the disease mechanisms and identifying molecular therapeutic targets. Since its origins, this project has been so far almost entirely sustained, with enormous efforts, by AISEA also with the support of AEHSA: after failing a number grant applications, the relevance of this project, dedicated to a very rare disorder, has been finally recognized with this success we love to celebrate with you.

As you might imagine, the proper development of the study will require dedicated collaborations of persons working in the different centres, thus the amount of money assigned by La Marató de TV3 will not be sufficient to cover the costs of the whole project. Thus, we hope that this first success will prompt yours and other patient associations to continue (or even increasing) supporting the development of this and other therapeutic projects for AHC. Indeed, as we have learned from the experience of other rare disorders, focusing on a single therapeutic strategy is rarely successful: even if an approach may work in a given condition, disease mechanisms are deeply different from one disease to another, thus the replication of the success is not obvious.

We feel today's success as a corner stone, a starting point to provide enough preliminary data to apply for further grant applications and also to search for industrial partners that will be of crucial support for the forthcoming phases. As in the case of any research project, we cannot be sure that we will be successful in bringing candidate compounds to the treatment of AHC into the clinic: in any case our project will provide extremely important data for the future of AHC therapeutics. We are confident that, also using this grant as a lever, together we will make great strides in the field of AHC.

Warmly,

Jaume Campistol Plana
Catedràtic de Pediatria
Universitat de Barcelona
Servei de Neurologia
Barcelona



Alexis Arzimanoglou
Coordinator of the ERN EpiCARE
Co-coordinator of the IAHCRC
Paediatric Clinical Epileptology and Neurophysiology Dpt,
University Hospitals of Lyon
Lyon
San Joan de Deu Hospital,
Barcelona

Elisa De Grandis
Coordinator of the Italian IAHCRC Node
Child Neuropsychiatry Unit
G. Gaslini Institute
Genova

Maria del Carmen Fons
Coordinator of the Spanish IAHCRC Node
Pediatric Neurology Department
San Joan De Deu Hospital
Barcelona

Fiorella Gurrieri
Medical Genetics
Campus Bio-Medico University
Rome

Mohamad Mikati
Division of Pediatric Neurology and Developmental Medicine
Duke University Medical Center Durham
Coordinator of IAHCRC

Eleni Panagiotakaki
Coordinator of the French IAHCRC Node
Paediatric Clinical Epileptology and Neurophysiology Dpt
University Hospitals of Lyon
Lyon

F. Danilo Tiziano
Section of Genomic Medicine
Catholic University
Roma