



SYMPOSIUM ON AHC

Hospital Sant Joan de Déu

9 January 2019, 8:30 – 17:00



8:30 **Registration**

9:00 **Opening and Welcome**

Bridget Vranckx, *President of AESHA (Spanish Association of AHC)*

Dr. Jaume Campistol, *Paediatric Neurologist, Hospital Sant Joan de Déu*

Morning Session - Chair Dr. Jaume Campistol

9:10 **The importance of research in rare diseases**

Jordi Cruz, *FEDER (Spanish Federation of Rare Diseases)*

9:20 **AHC: Current situation and understanding of the disorder**

Dr. Carmen Fons, *Hospital Sant Joan de Déu (Barcelona, Spain)*

9:45 **Research on AHC and family associations' role**

Rosaria Vavassori, *IAHCRC (International Consortium for the Research on AHC)*

10:15 **OBSERV-AHC Prospective Observational Natural History and Therapy Study**

Dr. Mohamad Mikati, *Duke University School of Medicine (Durham, USA)*

11:00 Coffee-break

11:30 **Behavioural disorders and psychiatric comorbidity in AHC**

Dr. Eleni Panagiotakaki, *Centre Hospitalier Universitaire de Lyon (France)*

12:00 **Therapeutic alternatives in AHC**

Dr. Elisa De Grandis, *Università degli Studi di Genova (Italy)*

12:30 **Medium throughput screening of candidate compounds to the treatment of AHC**

Prof. Francesco Danilo Tiziano, *Institute of Genomic Medicine of Catholic University (Rome, Italy)*

13:00 **AAV9 gene therapy project**

Video presentation

13:15 **General Discussion**

13:45 Lunch

Afternoon Session – Chair Dr. Carmen Fons & Albert Vilamala

15:30 **Meet the Experts: Dr. Mohamad Mikati, Dr. Eleni Panagiotakaki, Dr. Elisa De Grandis**

17:00 **Closing Remarks**

Organizing Committee

Dr. Carmen Fons, *Head of the Paediatric Neurology Service, HSJD*

Dr. Jaume Campistol, *Paediatric Neurologist, HSJD*

Bridget Vranckx, *President of AESHA*

Marc Gambús, *Secretary and Treasurer of AESHA*

Albert Vilamala, *Member of AESHA Board*