



## ***International Symposium: “Liver directed gene therapy for rare diseases”***

**Pamplona, 9<sup>th</sup> of October of 2014**



**uniQure**



**dkfz.** DEUTSCHES  
KREBSFORSCHUNGSZENTRUM  
IN DER HELMHOLTZ-GEMEINSCHAFT



## GENERAL INFORMATION

### VENUE

Centro de Investigación Médica Aplicada  
Avenida Pío XII, 55  
31008 Pamplona. Spain



### SYMPOSIUM COORDINATORS

**Dr. Gloria González Aseguinolaza**

*Centro de Investigación Médica Aplicada. Pamplona.*

**Dr. Jesus Prieto Valtueña**

*Clinica Universidad de Navarra. Pamplona*

### MANAGEMENT COMMITTEE

**Dr. Harald Petry**

*uniQure. Amsterdam*

**Dr. Nadina Grosios**

*uniQure. Amsterdam*

**Dr. Bruno Sangro**

*Clinica Universidad de Navarra. Pamplona*

**Dr. Delia D'Avola**

*Clinica Universidad de Navarra. Pamplona*

**Dr. Antonio Fontanellas**

*Centro de Investigación Médica Aplicada. Pamplona.*

**Dr. Manfred Schmidt**

*National Center for Tumor Diseases, Heidelberg*

**Dr. Rafael Enríquez**

*Hospital 12 de Octubre, Madrid*

**Dr. Pauline Harper**

*Karolinska Institute. Stockholm*

**Dr. Andrew Solomon**

*Centro de Investigación Médica Aplicada. Pamplona*

### CONTACT

Tlf: +34948194700

asolomon@unav.es

### REGISTRATION

[www.aipgene.org](http://www.aipgene.org)

<http://www.aipgene.org/international-symposium/inscription.html>

## SCIENTIFIC PROGRAMME

*9<sup>th</sup> OF OCTOBER*

**08:45 - 09:10 REGISTRATION AND CONGRESS DOCUMENTATION**

**09:10 Welcome**

**09:15 Developing AAV Vectors for Clinical Success**

**Jude Samulski, MD, PhD** *Keynote Speaker*  
*University of North Carolina, Chapel Hill, United States*

**10:00 Primary Hyperoxaluria - new treatment options wanted!**

**Bernd Hoppe, MD, PhD**  
*University of Bonn, Bonn, Germany*

**10:30 Gene therapy for cell-autonomous inborn errors of metabolism: primary hyperoxaluria**

**Eduardo Salido, MD, PhD**  
*Hospital Universitario de Canarias, Tenerife, Spain*

**11:00 Coffee break**

**11:20 Acute Intermittent Porphyria: Pathophysiology and genetics**

**Laurent Gouya, MD**  
*French Institute of Health and Medical Research, Paris, France*

**11:50 Porphyria disease: EU funded initiative AIPGENE Project**

**Jesus Prieto, MD, PhD**  
*Clínica Universidad de Navarra, Pamplona, Spain*

**12:20 Haemophilia and the challenge of gene therapy**

**Wolfgang Miessbach, MD**  
*Hemophilia Comprehensive Care Centre, Frankfurt, Germany.*

**12:50 AAV5-based Gene Therapy for Haemophilia B – the development of AMT-060**

**Nadina Grossios, PhD**  
*uniQure, Amsterdam, The Netherlands*

**13:20 Lunch**

**14:15 Lysosomal Storage Disease: Mucopolysaccharidosis**

**Mercedes Pineda, MD, PhD**  
*Hospital Sant Joan de Deu, Barcelona, Spain*

**14:45 Towards a Gene Therapy for Neurological and Somatic Mucopolysaccharidosis type III**

**Fatima Bosh, PhD**  
*Universidad Autónoma de Barcelona, Barcelona, Spain*

*International Symposium: "Liver directed gene therapy for rare diseases"*

**15:15 Gene therapy for treatment of Sanfilippo syndrome type B**

**Tim Miller, PhD**

*Abeona Therapeutics, EEUU*

**15:45 Current management of Crigler-Najjar Syndrome**

**Ulrich Baumann, MD**

*Hannover Medical Centre, Hannover, Germany*

**16:15 Gene therapy for Crigler-Najjar Syndrome, a journey towards the clinic**

**Federico Mingozzi, PhD**

*University Pierre and Marie Curie and Genethon, Paris, France*

**16:45 Coffee break**

**17:05 Wilson Disease**

**Peter Ferenci, MD**

*Vienna General Hospital, Vienna, Austria*

**17:35 Can Wilson disease be cured using gene therapy?**

**Gloria González-Aseguinolaza, PhD**

*Centro de Investigación Médica Aplicada, Pamplona, Spain*

**18:05 Glybera - reaching the market with 6 year follow-up data**

**Christian Meyer, MD, PhD**

*uniQure, Amsterdam, The Netherlands*

**18:35 Closing Symposium**