ADVANCES OF GENE THERAPY IN MONOGENIC DISEASES AFECTING THE HEMATOPOIETIC SYSTEM

Tuesday 9th February, 2016

Salón de actos Ernest Lluch. Ministerio de Sanidad, Servicios Sociales e Igualdad. Paseo del Prado 18-20. 28014 Madrid

INTRODUCTION

Gene therapy of patients suffering from a variety of monogenic diseases has experienced a remarkable progress both in terms of safety and efficacy. Such an improvement is particularly evident in approaches based on the ex vivo correction of hematopoietic stem cells (HSCs) with novel retroviral and lentiviral vectors. This workshop aims to share current protocols of HSC gene therapy with specialists treating patients suffering from monogenic diseases affecting the hematopoietic system. Specific emphasis will be made on primary immunodeficiencies, hemoglobinopathies and bone marrow failure syndromes.

SPECIFIC AIMS

- To facilitate the inclusion of Spanish patients affected by monogenic diseases in international HSC gene therapy trials
- To promote the development in Spain of HSC gene therapy trials already in progress in other countries
- To promote the development of new HSC gene therapy trials in Spain

Organizers: Spanish Agency of Medicines and Medical Devices (AEMPS) and Biomedical Research Centre on Rare Diseases (CIBERER).

SCIENTIFIC PROGRAM

- 9:00-9:15h: Opening**: Belén Crespo**, Director of the Spanish Agency of Medicines and Medical Devices and **Francisco Palau**, Director of the Biomedical Research Centre on Rare Diseases (CIBERER).

SESSION 1

Ongoing HSC gene therapy trials in Europe

CHAIR: **Nathalie Cartier** (President of the European Society for Gene and Cell Therapy) and **Juan Bueren** (Spanish Society for Gene and Cell Therapy)

- 9:15-9:45h: ***Evolving gene therapy in primary immunodeficiencies***. **Adrian Thrasher**. UCL Institute of Child Health. London.
- 9:45-10:00h: Discussion
- 10:00-10:30h: ***Hematopoietic stem cell gene therapy trials at the Necker Hospital***. **Marina Cavazzana**. Necker Hospital. Paris
- 10:30-10:45h: Discussion
- 10:45-11:15h: ***HSC gene therapy for inherited disorders: the experience of San Raffaele Telethon Institute for Gene Therapy***. **Alessandro Aiuti**. San Raffaele Hospital. Milano
- 11:15-11:30h: Discussion

11:30-12:00h: COFFEE

- 12:00-12:20h: ***Gene therapy of Fanconi anemia***. **Juan Bueren**. CIEMAT/CIBERER/IIS Fundación Jiménez Díaz. Madrid
- 12:20-12:30h: Discussion
- 12:30-13:00h: ***International, multicenter gene therapy trials for monogenic blood diseases***. **Fulvio Mavilio**. Genethon. Evry. France
- 13:00-13:15h: Discussion
- 13:15-13:45h: ***SCIDNET: A European collaborative initiative***. **Bobby Gaspar**. UCL Institute of Child Health. London.
- 13:45-14:00h: Discussion

14:00-15:00: BREAK

SESSION 2

National Registries and Transplantation of Patients with Monogenic Hematopoietic Diseases in Spain

CHAIR: **José María Moraleda** (Director of the Spanish Network on Cell Therapy, TERCEL and President of the Spanish Society of Hematology and Hemotherapy) and **José R. Regueiro** (President of the Spanish Society for Immunology).

- 15:00-15:15h: ***Registry of primary immunodeficiencies in Spain***. **Carlos Rodríguez-Gallego**. Spanish Registry of Primary Immunodeficiencies. H. Son Espases. Palma de Mallorca
- 15:15-15:35h: ***Newborn screening for primary immunodeficiencies***. **Olaf Neth**. H. Virgen del Rocío. Sevilla and **Pere Soler-Palacín**. H Vall d'Hebron. Barcelona
- 15:35-15:50h: ***Registry of patients with hemoglobinopathies in Spain***. **Elena Cela**. H Gregorio Marañon. Madrid
- 15:50-16:10h: General Discussion
- 16:10h-16:25h: ***Transplantation of patients with primary Immunodeficiencies***. **Luisa Sisinni/Isabel Badell**. H. Sant Pau. Barcelona
- 16:25-16:40h: ***Transplantation of patients with hemoglobinopathies***. **Cristina Díaz de Heredia**. H. Vall d'Hebron. Barcelona
- 16:40-16:55h: ***Transplantation of patients with bone marrow failure syndromes***. **Julián Sevilla**. H. Niño Jesús. Madrid
- 16:55-17:15h: General Discussion

SESSION 3

Regulatory aspects of hematopoietic gene therapy

- 17:15-17:45h: ***Regulatory aspects of Intra-European gene therapy trials: Transit of patients; transit of vectors; transit of transduced cells***

Maria Antonia Serrano. Head of the Clinical Trials Unit. Spanish Agency of Medicines and Medical Devices (AEMPS)

Sol Ruiz. Spanish Agency of Medicines and Medical Devices (AEMPS) and Chair of the Biotechnology Working Party, European Medicines Agency (EMA).

- 17:45-18:00h: Discussion
- **18:00h: CLOSE**

REGISTRATION: The Registration has no costs and will be open until the completion of the Conference Room. Attendees must register at this link:
http://goo.gl/1JzWa9

Organizers:

MINISTERIO DE SANIDAD, SERVICIOS SOCIALES E IGUALDAD

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