Clinical Trial and Commercialisation Workshop

Programme Tuesday 17 October 2017

ORGANISERS AND CHAIRS: Robin Ali and Alessandro Aiuti

In collaboration with:

AMERICAN SOCIETY of GENE & CELL THERAPY

09:30 - 10:45 — Planning a clinical trial

Room B05 - B07

INV14 **Good clinical practice specific to clinical trials with cellular therapies**
Kim Champion [UNIVERSITY COLLEGE LONDON]

INV15 **Translation of advanced therapy medicinal products (ATMPs) towards clinical trials – critical issues and implications of regulation (EU) no 536/2014**
Martina Schüssler-Lenz [PAUL EHRLICH INSTITUTE, LANGEN]

INV16 **Small populations clinical trials**
Simon Day [CLINICAL TRIALS CONSULTING & TRAINING, NORTH MARSTON]

Round table discussion

10:45 - 12:45 — Manufacturing of gene and cell products

Room B05 - B07

INV17 **Development and GMP production of large scale LV/RV viral vectors and genetically modified haematopoietic and T cells**
Giuliana Vallanti [MOLMED, MILAN]

INV18 **Building a GMP facility**
James Christie [MEIRAGTX LTD, LONDON]

INV19 **Leveraging a decade of iPSC expertise to advance cell therapies for heart failure, Parkinson's disease, immuno-oncology and ocular disease**
Steven Kattman [CELLULAR DYNAMICS INTERNATIONAL, MADISON, WI]

INV20 **AAV-based gene therapy: the vector manufacturing challenge**
Fulvio Mavilio [AUDENTES, SAN FRANCISCO, CA]

INV21 **Delivering *ex-vivo* gene therapy**
Bobby Gaspar [UNIVERSITY COLLEGE LONDON]

Round table discussion

12:45 - 13:45 LUNCH

13:45 - 15:00

Gene therapy and commercialisation challenges

INV22 **Adaptive development of advanced gene therapy products**
Anne-Virginie Eggimann [BLUEBIRDBIO, CAMBRIDGE, MA]

INV23 **Commercialisation challenges for cell and gene therapies**
Sven Kili [GSK, BRENTFORD]

INV24 **Overcoming unique challenges of clinical development for emerging gene therapies**
Tim Miller [ABEONA, CLEVELAND, OH]

Round table discussion

Room B05 - B07

15:00 - 16:30

Regulatory challenges in gene and cell therapy development

INV25 **ATMP: Differences between regulatory frameworks (EU vs US vs Japan); what post Brexit**
Sol Ruiz [SPANISH MEDICINE AGENCY, MADRID]

INV26 **Key regulatory challenges for developing gene and cell therapies in the EU**
Jackie Barry [CATAPULT, LONDON]

INV27 **Submission of an IND to the FDA**
Helen Heslop [BAYLOR COLLEGE OF MEDICINE, HOUSTON, TX]

INV28 **Price and reimbursement of ATMPs: The experience of the Italian Drug Agency**
Giovanni Tafuri [AIFA, ROME]

Round table discussion

Room B05 - B07