Pan-UCL AAV Gene Therapy Symposium

Friday 28th June 2024 | 09:30-17:10
Kennedy Lecture Theatre, UCL Great Ormond Street Institute of Child Health, 30 Guilford Street London WC1N 1EH

09:30-09:55	Registration and set up of posters.
09:55-10:00	Welcome on behalf of the organising committee, Director, Genetic Therapy Accelerator Centre, QS IoN, Prof. Francesco Muntoni.
10:00-10:05	Opening Introduction from the Director, QS IoN, Prof. Michael Hanna.
10:05-10:10	Consolidating our expertise, Investing in the future of AAVs at UCL, Pro-Vice Provost, (Scientific and Technology Platforms), Prof. Stephanie Schorge.
10:10-13:00	First Session: Preclinical Developments and Model Systems. Chairs: Prof. Simon Waddington and Prof. Manju Kurian.
10:10-10:40	AAV Synthesis, Prof. Ahad Rahim.
10:40-11:10	Engineering and Manufacturing of Viral Vectors, Prof. Paul Dalby.
11:10-11:40	AAV Capsid Design, Dr Killian Hanlon.
11:40-12:00	AAV Cassette Design, Dr Jenny McIntosh.
12:00-12:30	Advanced in vitro modelling of neuromuscular diseases and gene therapies, Prof. Francesco Saverio Tedesco.
12:30-13:00	3 flash poster presentations from submitted abstracts (7 minutes + 3 for discussion).
12:30-12:40	Dual-targeting CRISPR-CasRx AAV gene therapy reduces C9orf72 ALS/FTD sense and antisense repeat RNAs in vitro and in vivo, Alexander J. Cammack.
12:40-12:50	AAV9-Mediated Gene Therapy In A Knock-In Mouse Model Of Infantile Neuroaxonal Dystrophy, Amy Geard.
12:50-13:00	Brain-directed AAV gene therapy corrects lethal neurodegeneration and improves locomotor behaviour in a mouse model of CLN5 Batten disease, Wenfei Liu.
13:00-14:00	Lunch, poster viewing and networking.
14:00-17:00	Second Session, Clinical Application of AAVs. Chairs: Prof. Claire Booth and Prof. Paul Batty.
14:00-14:30	AAV developments for epilepsies, Prof. Gabriele Lignani.
14:30-15:00	AAV delivery to the CNS, Prof. Robert Brownstone.
15:00-15:30	AAV gene therapy for haemophilia, Prof. Pratima Chowdary.

15:30-16:00 Tea and coffee break.

16:00-16:30 High AAV doses safety in neuromuscular disorders, Prof. Francesco Muntoni.
16:30-17:00 3 flash poster presentations from submitted abstracts (7 minutes + 3 for discussion).
16:30-16:40 AAV9-mediated gene targeting of natural antisense transcript as a novel treatment for Dravet Syndrome, Juan Antinao Diaz.
16:40-16:50 Development of an efficient and safe preclinical gene editing platform for the treatment of Wiskott Aldrich Syndrome, Asma Naseem.
16:50-17:00 AAV9 gene therapy efficacy and safety in transgenic mice models of developmental epileptic encephalopathy, Hester Chu.

17:00: 17:05 Prize draw for the submitted posters. Prof. Paul Gissen and Dr Jo Ng.
17:05-17:10 Closing remarks, Prof. Simon Waddington.
17:10-19:00 Reception and networking.