AAV-Based In Vivo Gene Therapy for Rare to Common Disease, and Innovative Gene Regulation Technologies

This campaign has been commissioned by an international Japanese pharmaceutical company who combat diseases using a combination of ground-breaking technology and biology, with an extensive track record of academic collaboration. They are interested in AAV-based in vivo gene therapy through research programs for rare to common disease, and innovative gene regulation technologies.

1) AAV-based in vivo gene therapy program for rare to common disease

Approaches of Interest:

- Therapeutic target: Mechanism of action is proven or well-understood
- Target disease: Rare to common disease, prevalence is above 1/50,000
- Preclinical proof of concept has been evaluated in clinically translatable study (e.g. in vivo animal model study, in vitro patients derived cells/tissues assay)
- Neuromuscular disorders, Central Nervous System Disorders and Cardiovascular Disorders are slightly prioritised, but there are no limitations as to other therapeutic areas, with the exception of ophthalmology and oncology

Out of Scope:

- Ultra-rare diseases (prevalence of below 1/50,000)
- Ophthalmology and cancer
- Ex vivo gene therapy, cell therapy, non-viral vector and other viral vector based research, unless they can be substituted by AAV-based gene therapy

2) Next generation gene regulation technology applicable for AAV-based in vivo gene therapy

Approaches of Interest:

- Innovative gene regulation technology for AAV-based in vivo gene therapy
- Tangible research target should be nominated with the technology

Out of Scope:

- Gene editing by making double-strand break
- Well-known gene regulation technology unless there is obvious inventiveness, e.g. gene supplement (<3.5kb, by single AAV), shRNA/miRNA, CRISPRa/CRISPRi
- Tissue specific promoter screening

Opportunities sought

- Technologies
- Academics and expertise
- Centres of excellence
- Research projects
- Spinout companies

Submissions

Please submit relevant, non-confidential opportunities online via: discover.in-part.com

Deadline: 11th January 2021 - 11:59 pm GMT

Have any questions? Contact our team at discover@in-part.co.uk

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