inpart Industry Call for Opportunities

Novel Therapeutic Interventions to Address the Root Genetic Cause of Neurodevelopmental Disorders

Our client, a global leader in therapies for rare genetic diseases, is seeking **novel approaches to address the root genetic cause of neurodevelopmental disorders**. Specifically, the team is looking for interventions which promote a disease modifying effect in developmental **epileptic encephalopathies and neurodevelopmental disorders - CDKL5 deficiency disorder; Fragile X Syndrome; Rett Syndrome, SYNGAP1 disorder and STXBP1 disorder**.

Approaches of Interest:

- **Disease modifying treatments**, for example through allele-specific knockdown, protein replacement/enhancement/stabilization, or modulation of endogenous gene regulation and expression.
- Therapeutic modalities of interest include **nonviral approaches**, mRNA, oligonucleotides (RNAi, siRNA, ASO, saRNA), small molecules, and protein replacement/substitution//stabilization. Innovative and unique approaches are encouraged.

Validation:

- Opportunities with **supporting data demonstrating disease modification** are of high interest.
- **Basic and preclinical research** that assesses treatment feasibility are also within scope (e.g. studies determining the necessary timing and therapeutic levels to modify disease in *in vivo as well as in vitro* models).

Out of Scope:

- Viral gene therapy approaches.
- Disease indications not listed above.

Submission Information & Opportunity for Collaboration

Submission of 200–300-word briefs is encouraged, along with any supplementary information e.g. relevant publications, patents or slide decks. The team encourages including the proposed next steps in developing the research towards commercialization. In submitting to this campaign, you confirm that your submission contains only non-confidential information.

Our client is open to a range of collaboration opportunities, with the most appropriate outcome being decided on a case-by-case basis. Example outcomes include funded research collaborations and agreements or licencing of assets. Our client is looking to establish long-standing partnerships in the field of neurodevelopmental disorders.

Opportunities sought

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

Submissions

Please submit relevant, non-confidential opportunities online here

Deadline: **27th May 2025 - 10:59 pm GMT**

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