

Innovative Therapies for Genetic Mitochondrial Disorders and Leukodystrophy

A **genetic disease-focused** pharmaceutical company is seeking **novel therapeutic targets for genetic mitochondrial diseases and leukodystrophies**. The team is also interested in innovative **drug discovery and development platforms** tailored to these disease areas.



Approaches of Interest:

- Submission for **mitochondrial diseases** should focus on: **(i) Target molecules expressed in brain cells** (neurons, microglia, astrocytes, oligodendrocytes) **(ii)** Mechanisms that improve oxygen consumption, NAD^+/NADH ratio, or overall mitochondrial function (other MoAs will be considered) **(iii)** Demonstrated improvement in survival or behavior in *in vivo* disease models is preferred **(iv)** Priority diseases include Leigh syndrome, MELAS, MERRF, NARP, and KSS (other diseases will be considered)
- Submissions for **leukodystrophies** should focus on: **(i) Target molecules in oligodendrocytes or oligodendrocyte progenitor cells (OPCs)** (targets in other brain cell types will be considered) **(ii)** Mechanisms promoting myelination, OPC differentiation, or inhibiting demyelination (other MoAs will be considered) **(iii)** Priority diseases include Pelizaeus-Merzbacher disease, Metachromatic leukodystrophy, and Krabbe disease (other diseases will be considered)
- Modalities of interest include **small molecules, peptides, antibodies and gene therapy**
- A highly **novel and original drug discovery and development platform**, with demonstrated validation and use in real-world drug discovery

Out of Scope:

- Opportunities relating to cell therapy or medical devices
- Discovery or development platforms that are unvalidated or widely adopted, established platforms

Developmental Stages of Interest:

- Opportunities from basic research phase to preclinical stage, with proof-of-concept
- Opportunities with validation in human disease lines are preferred; validation in animal disease models will also be considered

Submission Information

Submission of one-page, 200–300-word briefs is encouraged, along with any optional supplementary information e.g. relevant publications. Our client is also open to novel research proposals which can be outlined using this [submission form](#). In submitting to this campaign, you confirm that your submission contains only non-confidential information.

Opportunity for Collaboration

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 licensing assets, project/PhD funding, and research collaborations.

Please check your organization's external collaboration policy and/or guidelines before submitting to this anonymous campaign, which may result in introduction and subsequent partnering discussions with the client company. Please get in touch with campaigns@inpart.io if you have any queries.

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: **9th June 2025 - 10:59 pm GMT**

Have any questions?

Contact our team at discover@in-part.co.uk

