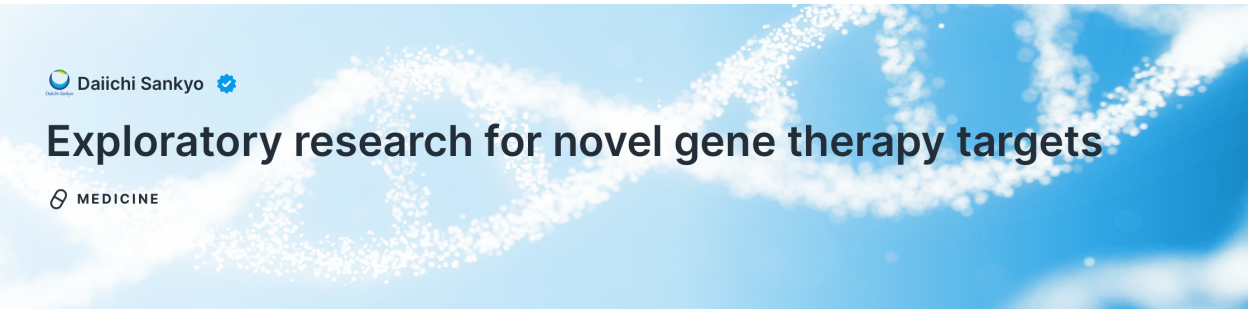

Background
Gene therapy has emerged as a promising medical approach, offering a potential treatment path for diseases that have long been considered incurable. This therapy involves the manipulation of genetic material within a patient's cells to alleviate or cure diseases.

Despite the significant progress made in this field, there remain numerous disorders for which effective gene therapies have yet to be developed. Many challenges arise either from the complexity of the diseases, as some involve multiple genes, or from delivery issues, as getting therapeutic genes into the right cells safely and efficiently remains a major hurdle. For instance, certain tissues, like the brain or heart, are difficult to reach, and some gene delivery systems, such as viral vectors, can trigger immune responses or have limited targeting capabilities. Furthermore, the identification and validation of novel targets for gene therapy pose significant challenges.

Discovering suitable targets, especially for common diseases, requires a deep understanding of how specific genes contribute to disease, which is often complicated by incomplete knowledge of genetic functions. Even when promising targets are identified, laboratory models do not always predict unintended side effects or how these therapies will behave in humans, making it difficult to ensure both safety and efficacy. Therefore, there is an urgent need to explore and develop new gene therapies to address these unmet medical needs.

What we're looking for
We are looking for innovative research projects that can identify and explore novel targets for gene therapy, particularly in CNS (central nervous system) and CVM (cardiovascular and metabolism) related diseases. We are interested in proposals that explore therapeutic effects through gene supplementation, knockdown, or a combination of both. The targets should be delivered by Adeno-Associated Virus (AAV) to the relevant tissues and cell types and should be distinguishable from existing therapies.

Solutions of interest include:
- Investigation of novel disease mechanisms and identification of gene targets that are not adequately addressed by existing therapies, focusing on approaches to

target protein overexpression or harmful mutations using AAV-delivered RNA interference (RNAi) or CRISPR-based gene knockdown.
- In vitro models using human tissues or patient-derived cells for omics and genomic analysis that can evaluate pharmacological effects and validate selectivity of the knockdown.
- Exploratory research focusing on novel gene therapy targets with high clinical translatability, leveraging omics or genomic analysis from patient-derived tissues and cells to correlate target gene modulation with disease phenotypes.
- Use of novel CRISPR variants or alternative gene-editing technologies that limit off-target activity while delivering therapeutic effects via AAV.
- Research that can evaluate the pharmacological effects mediated by novel targets in in vitro/in vivo systems.
- AAV-delivered gene therapies that produce proteins, enzymes, or other therapeutic agents secreted into systemic circulation, addressing diseases that require widespread effects (e.g., lysosomal storage disorders), along with in vitro and in vivo models to monitor secretion levels and assess efficacy and safety.

Our must-have requirements are:
- The target must exhibit therapeutic effects in tissues and cell types that are accessible through AAV delivery, such as liver, muscle, or the central nervous system. This includes cases where the therapeutic effect is achieved by the drug being secreted into systemic circulation.
- For a novel target of single gene knockdown, the target should be limited to those that can be differentiated by gene therapy from existing therapies against the same target.

Our nice-to-have's are:
- An exploratory study of novel targets with clinical translatability using patient cells and tissues that allow for omics data analysis or genomic analysis.
- Genome editing technologies that offer higher selectivity for target sequences, ensuring high efficacy and safety.

What's out of scope:
- Diseases with a prevalence of less than 1 in 100,000 individuals in Japan and the US.
- Therapies aimed at treating cancer.

Acceptable technology readiness levels (TRL): Levels 1-5
1. Basic principles observed
2. Concept development
3. Experimental proof of concept
4. Validated in lab conditions
5. Validated in relevant environment
6. Demonstrated in relevant environment

7. Regulatory approval
8. Product in production
9. Product in market

What we can offer you

Eligible partnership models:

Sponsored research

Benefits:

Sponsored Research

Funding is proposal dependent, with up to $ 100K for 12-month project with potential follow-on funding for 1 year.

Who we are

At Daiichi Sankyo, we attach significant importance to working with academic institutions, startups and bioventure companies to discover new therapeutics in the place where hypotheses are brought and tested in order to expand possibilities for scientific innovation breakthrough. We build sustainable relationships with partner institutions and companies through open and fair alliance management and trust based on mutual respect as the foundation for effective collaborations. Our goal is to jointly create new value for patients by maximizing each other's expertise and strengths.

Reviewers

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