Background
Genetic disorders are classified in three main categories: monogenic disorders, chromosomal abnormalities, and multifactorial conditions. Over the past two decades, several gene therapy products have been approved for monogenic disorders, however, there have been no cures for chromosomal abnormalities so far.

Chromosomal abnormalities are generally caused by numerical or structural changes in chromosomes, such as translocations, deletions, and duplications. These alterations affect multiple genes at once, making it difficult to develop treatments that target single molecules or specific genes, as is possible for monogenic disorders.

Recently, researchers have identified certain genes that play a significant role in the manifestations of chromosomal abnormalities, even though these disorders typically involve many genes. These 'relatively causal' genes offer a promising starting point for developing more focused therapies. The development of cell and animal models based on these findings has provided valuable tools for studying the underlying mechanisms of chromosomal abnormalities. These discoveries will pave the way for innovative drug discovery in this area.

What we're looking for
We are looking for innovative research projects that can evaluate the efficacy of gene therapies targeting specific genes in chromosomal abnormalities. Our primary interest is in research focused on Down syndrome (trisomy 21), but other conditions such as trisomy 13, trisomy 18, as well as 5p- and 4p- syndromes are also within the scope. We are also interested in research focused on identifying key target genes involved in these abnormalities.

Solutions of interest include:

- Utilization of organoid models or patient-derived induced pluripotent stem cells (iPSCs) with engineered chromosomal abnormalities to map out gene expression and study the impact of target genes on disease phenotypes.
- Validation of known target genes through in vitro or in vivo studies to assess their effect on disease phenotypes and potential therapeutic benefits.
- Identification of novel 'causal' genes contributing to chromosomal abnormalities using transcriptomic, proteomic, and genomic data from patient-derived tissues and cells.

- Investigation of the therapeutic potential of causal genes in animal models with a focus on broad-spectrum or life-threatening manifestations of chromosomal abnormalities.

Our must-have requirements are:

- Proposals must include a unique idea of target genes supported by any scientific data (clinical or nonclinical) or animal/organ/cell models related to a certain chromosomal abnormality.
- The target gene is expected to improve a wide range of clinical manifestations related to the chromosomal abnormality and/or significantly address a single life-threatening symptom.
- The target gene is expected to show pharmacological effects in tissues and cell types where the gene can be delivered by current gene therapy (liver, muscle, CNS, eyes, etc.).

Our nice-to-have's are:

- Research proposals that present both unique target gene ideas and disease models.

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 or infectious diseases.

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

Ryusuke Sugita
Senior Researcher

Masatoshi Nagamochi
Associate Director

Mikio Kato
senior director

Kousei Shimada
Director

Please contact the University of South Florida Technology Transfer office representative for submission – Roisin McNally at rmcnally@usf.edu