Principal Investigator

Pioneering Research Institute, RIKEN
**Shintaro Iwasaki**

Adopted Theme

Mitochondrial Translation-Enhancing Drug Discovery

## Subject of Research

**Mitochondrial Translation-Enhancing Drug Discovery**

## Overview


This project aims to refine an integrated drug discovery platform that enables highly sensitive and quantitative assessment of mitochondrial translation activity by combining proprietary technologies such as Mito-FUNCAT, HT-Thor-Ribo-Seq, and MitoIP-Thor-Ribo-Seq. Through this platform, we will identify small molecules that enhance mitochondrial translation as well as novel genetic targets, thereby generating promising lead compounds. The platform allows a stepwise workflow from compound screening to target identification and mechanistic elucidation, ensuring efficient and reproducible lead discovery. By doing so, we seek to provide new therapeutic options for rare mitochondrial diseases such as MELAS and Leigh syndrome, for which no fundamental treatments currently exist, and further expand applications to age-related disorders such as sarcopenia and age-related macular degeneration. Ultimately, this project aims to open the path toward innovative therapeutics that address both rare and common diseases driven by mitochondrial dysfunction.

## Business Models (when applying)

The business model of this project is a hybrid approach that combines in-house drug development pipelines with collaborative research and licensing opportunities based on our proprietary drug discovery platform. This structure enables both the monetization of core technologies and the advancement of therapeutic pipelines, ensuring sustainable growth and multiple exit strategies.

## Activity Planning (when applying)

**Advancement of the Drug Discovery Platform and Lead Identification**
Integrate proprietary technologies such as Mito-FUNCAT, HT-Thor-Ribo-Seq, and MitoIP-Thor-Ribo-Seq, and conduct high-throughput screening using large compound libraries. Complement this with CRISPR-based screening to identify target genes and generate lead compounds.

**Establishment of POC and Mechanistic Elucidation Using Disease Models**
Establish in vitro POC in patient-derived cells from MELAS and Leigh syndrome, and in vivo POM/POP in mouse models. Further leverage additional disease models such as MERRF to identify target molecules and elucidate mechanisms of action.

**Intellectual Property and Business Preparation**
File patents for the first pipeline based on obtained data and secure rights for future out-licensing. In parallel, conduct market research to select exit indications, refine business and fundraising strategies, and bring in experienced management talent, aiming for the launch of a startup in 2028.