

Private Company

## Translational approaches for mitochondrial function driven diseases

8 MEDICINE

### Background

Mitochondria are dynamic organelles critical in cellular bioenergetics, metabolism, modulating stress responses and maintenance of homeostasis. Mitochondria have their own DNA, and maintenance of mitochondrial dynamics is important for energy production and normal functioning of cellular processes like differentiation, cell cycle, senescence and apoptosis. Mitochondrial defects can be classified as either primary that are mitochondrial-autonomous defects or secondary due to non-mitochondrial pathways that triggers loss of mitochondrial functioning (fission, fusion, mitophagy, DNA mutations). Despite this, there is an insufficient understanding of the mechanism causing mitochondrial dysfunction as well as limited progress in target-based approaches. Due to the lack of established biomarkers, there have been few studies on pharmacological strategies for targeting mitochondrial function. Another critical aspect in advancing mitochondrial drug development is the need to establish optimal cellular models that can serve to validate potential treatments.

Since modulation of mitochondrial dynamics can cause lethal consequences, establishing well-defined biomarkers and optimal cellular models would enable researchers to identify the specific mechanisms associated with diseases and to validate effectiveness of mitochondrial targeting therapies.

### What we're looking for

We are looking for mechanism- and target-based approaches to explore mitochondrial biology in immune related diseases. Our focus is on integrating these approaches in foundational research models, to explore the fundamental biological and pathological processes of a disease, and in translational settings, to develop or test a new treatment. We are interested in studies using preclinical disease models, such as cellular and animal systems, as well as research involving human samples from both healthy individuals and those affected by diseases.

#### Solutions of interest include:

- Target-based mechanisms
- Tool molecules that can manipulate mitochondrial function
- Omics and bioinformatic-based disease validation
- Pharmacological or genetic screening methods

- High throughput in vitro test models
- Animal models with clear translational value
- Experts with relevant experience in mitochondrial biology and modulation

**Our must-have requirements are:**

- In vitro data illustrating mitochondrial mechanism and pathways in immune related diseases (e.g. autoimmune, neurodegenerative, neuromuscular, epilepsy etc.)
- Evidence of mitochondrial mechanisms i.e. mitophagy, mitochondrial dynamics, metabolism, senescence leading to disease pathology or effecting immune cell function
- Research activities should have legal clearance for intellectual property rights (freedom to operate)

**Our nice-to-have requirements are:**

- Although it is not required, in vivo translational animal models are highly desired
- Although it is not required, in vitro cell assays for senescent cells are highly desired
- Mechanism or pathways involved in mitochondrial function in diseases

**What's out of scope:**

- Approaches with poorly defined selectivity for mitochondrial function and specific targets
- Solid tumor focused cancers

**Acceptable technology readiness levels (TRL): Levels 3-6**

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**
- **Licensing**
- **Co-development**
- **Material transfer**

**Benefits:****Sponsored Research**

Open to a range of potential funding options and models, including e.g. PhD studentships. Financial contribution to be determined based on individual projects and collaboration structure, with range of \$50 - \$200K, including a minimum of 50% indirect costs (financial support negotiable based on specific opportunities and partnerships).

**Tools and Technologies**

A range of support activities available, including generating molecules (focus on antibody and small molecule modalities), bioinformatics analysis and support, in vivo models and target validation activities.

**Expertise**

Established pharmaceutical company with proven success in drug development and approvals in the immunology and neurology therapeutic area. Partners will benefit from proven drug development expertise, with an assigned primary contact/sponsor to offer guidance, technical input and supporting the direction and translational needs of specific projects where relevant.

Please contact the University of South Florida Technology Transfer office representative for submission – Karla Schramm at [kschramm@usf.edu](mailto:kschramm@usf.edu)