Technical Project Summary- SDI

Project title
iPsomics
Project leader + organisation
iPsomics – Peter Ketelaar
File number:
10920012310003
Key words
iPSCs, organ-on-a-chip, single cell sequencing, CRISPR editing, drug discovery

Domain 4D Map (link)

☒ A. Basic Science & Target Identification
☐ B. Target Pharmacology
☐ C. Lead Identification
☐ D. Lead Optimization
☐ E. Clinical Research & Development
☐ F. Regulatory review
☐ G. Post-marketing
☐ H. Medical Landscape
☐ I. Commercial Manufacturing & Distribution

Aims and objectives
Our two main aims are:

1) The development of single-cell phenotype iPSC-derived disease models for drug discovery
2) The development of single cell phenotyping tools to improve molecular disease diagnosis to improve treatment stratification.

Towards these aims, we will transform two academic facilities that provide single cell sequencing and iPSC/CRISPR services into a research infrastructure for academic and commercial customers. In addition, we will further develop our services to become a self-sustainable company at the end of the project.

User group and application
Our research infrastructure serves academic as well as commercial customers. Our services are made available via iPsomics, a startup company, which will offer the services of two pre-existing academic facilities: the UMCG iPSC/CRISPR facility and the UMNCG research sequencing facility. Interested customers can reach out to iPsomics (www.ipsomics.com; info@ipsomics.com) to inquire about our services.

Our targeted users are academics who require deeply phenotyped (CRISPR-engineered) iPSC-derived disease models, including organ-on-a-chip models for their research purposes as well as commercial customers who need these models for assay development and/or drug discovery. In addition, our current targeted users include academic and commercial customers who require single sequencing services, as well as bulk sequencing services.

Our unique selling points include:

- High throughput generation and single cell phenotyping of iPSC-derived models.
- A pipeline starting from somatic cells, all the way to deeply phenotyped disease models including organ-on-a-chip models.
- A unique amplification-free single cell whole genome sequencing pipeline to quantify karyotype heterogeneity, an important predictor of disease outcome, e.g. for cancer.

Impact
Our project will create impact via:

- Drug discovery via iPSC-derived disease models. Our technology development will yield an innovative pipeline for the generation of collections of such iPSC-derived models, which will improve coverage of the variability within the human population and decrease in the demand for animal models.

- A pipeline to improve diagnosis and stratification of treatment for (cancer) patients.

As a startup company, we can function as service provider, as a collaborator or consortium member developing joint IP, and as a partner for production and R&D.

Technical description of the shared infrastructure
In this project, we will establish an accessible research infrastructure that combines iPSC, Organ-on-a-chip, CRISPR and single cell sequencing technology to support drug discovery, and to improve diagnosis to prevent deleterious therapy/overtreatment.

Our platforms include:

- A fully automated high throughput pipeline to derive induced pluripotent stem cells from somatic cells provided by customers.
- A high throughput CRISPR genome editing platform to generate isogenic iPSC-derived disease models.
- Over time,
- An FDA-approved Organ-on-a-chip model systems for drug discovery
- Various single cell sequencing pipelines, including shallow whole genome sequencing to quantify copy number changes in primary tissue samples or cell cultures as a predictor for (cancer) cell genome evolution.
- Other competitive sequencing services (single cell RNA, bulk RNA, etc.), taking advantages of in house developed library preparation protocols.

As a research infrastructure, we will devote up to 50% of our efforts towards novel technology development that builds upon our existing technology platforms. In the coming years we expect to develop:

- Many new single cell sequencing applications that can be combined as multi-omic approaches to deeply phenotype disease models and disease/patient samples to improve diagnosis and stratify treatment.
- A biobank of deeply phenotyped iPSC lines and differentiated progeny for which use rights can be provided to pharma industry for drug discovery and drug validation purposes.

Importantly, we will make all of our pipelines available to interested customers/collaborators as soon as they are validated the maximize impact throughout the project.

Project group and collaborating partners

This project is a consortium of the following participants.

- **iPsomics**: project leader and newly established company. 100% daughter from the UMCG. iPsomics will commercialize and further develop the UMCG technology to improve drug discovery and availability in the Netherlands.
- **UMCG** with the departments **ERIBA** and **Genetics**. ERIBA will give access to background IP on iPSCs, CRISPR and various sequencing technologies. Genetics will give access to background IP on organ-on-a-chip models. Both departments furthermore pitch in substantial in-kind personnel and equipment.
- **Pivot Park Screening Center** will help validate iPSC-derived disease models by providing access to their high throughput drug discovery infrastructure.
- **Life Cooperative** will help with business development for iPsomics.
- **GenomeScan** will help with quality assurance for iPsomics.

Innovation and Distinctiveness

Our project is unique as we are:

- The first startup company that combines single cell phenotyping with iPSC-derived disease models.
- We are one of the few companies in the Netherlands that have a fully automated iPSC-derivation pipeline.
- Our single cell genomics pipeline is unique worldwide and a standard in the field to quantify single cell karyotype heterogeneity.
- Our strong link with academia and the clinic provides us unique access to patient samples.

Alignment PharmaNL programme

The aim of PharmaNL is to increase the availability of drugs in the Netherlands. We contribute to this by

- Developing advanced iPSC-derived disease models for drug discovery screens, thus **facilitating the discovery of new drugs** in the Netherlands
- Developing a pipeline to improve disease diagnostics to help stratify treatment thus **improving drug availability** in the Netherlands.