

*Gene and Cell therapy*

## Stem Cell & Organoid Medicine

### Brief Description of Technology

Organoids provide an unprecedented opportunity to study human organ development and physiology, and investigate the cause of disease.

#### TECHNOLOGY ID

2018-0401

#### TECHNOLOGY TYPE

Biologic Therapy

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### Technology Overview

A convergence of breakthroughs in stem cell technology and developmental biology has enabled investigators to make induced pluripotent stem cells (iPSCs) and organoids, miniature versions of organs, from a patient's own cells. iPSCs derived from fully differentiated cells can be grown indefinitely in a petri dish and have the potential to form any cell or tissue type in the body. We are using iPSCs as a tool to study the normal processes that drive organ development and to uncover the molecular basis of human birth defects. Leveraging information from organogenesis in animal models we are establishing new methods to direct the differentiation of iPSCs into 3-dimensional organ tissues, called organoids. While miniature in form, organoids have a tissue structure and physiology similar to normal organs. Organoids provide an unprecedented opportunity to study human organ development and physiology, and investigate the cause of disease. In particular, liver organoids can be used to examine drugs in development for liver toxicity effects.

### Applications

- Organoids derived from iPSCs for GI indications (esophagus, intestines, stomach, and liver)
- A novel platform for drug screening
- Gene and cell therapies
- Personalized regenerative medicine

### Advantages

Autologous stem cells can be used for gene therapy or regeneration. Immunologic responses from allogenic cells are bypassed.

Organoids recapitulate the processes of organ formation in a petri dish. Once formed, organoids can provide an unlimited supply of cells and tissue for gene and cell therapies.

### Investigator Overview

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