DefiniGEN

DISEASE MODEL INNOVATION

Key Facts

- **10 years** of iPSC innovation
- Exemplar disease models
- Access to stem cell library with over **300** donors
- **26** staff, **7** PhD scientists
- ISO 9001:2015 certification
- Located at the Babraham Research Campus

DefiniGEN offer a suite of assay services including:

- Low or high throughput ELISAs
- Immunocytochemistry (ICC)
- qPCR
- Western blotting
- Mass spectrometry
- Flow cytometry
- Confocal imaging

About

DefiniGEN's purpose is to improve and save lives by accelerating the development of treatments for life-threatening conditions. We do this by providing highly predictive cell-based *in vitro* models of liver and metabolic related diseases to the drug development industry.

We provide a unique and proprietary induced Pluripotent Stem Cells (iPSC) derived disease modeling platform technology that allows us to create phenotypically relevant disease models and bioassays that both accelerate and de-risk the development of next-generation therapies, offering IND enabling efficacy and toxicology studies.

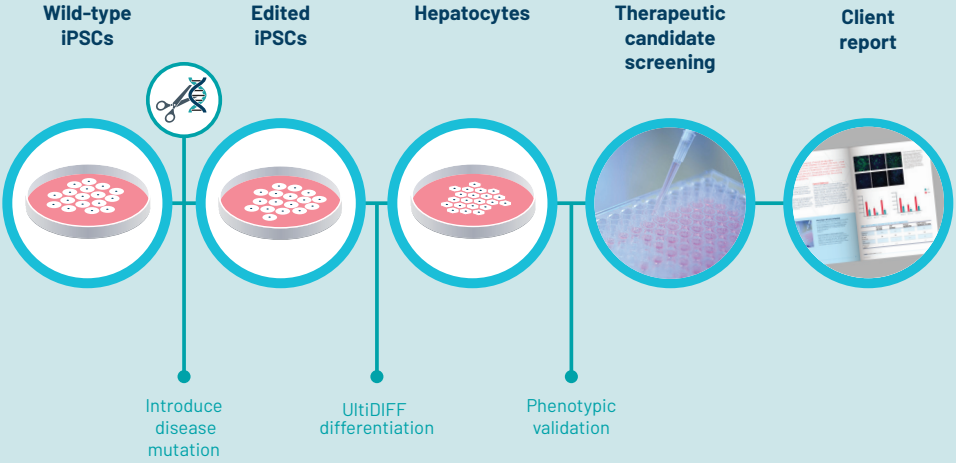
Our in-house CRO service offering provides expertise from our seasoned team of scientists who can help optimize experiments enabling the rapid generation of pre-clinical data for hit-to-lead, lead optimization and candidate selection. We also offer several cryopreserved cell vials that can be utilized for your own internal research or evaluation. Our cryopreserved cells exhibit excellent recovery after thawing, maintain morphology, display key functionality markers, and offer longer assay windows than primary human cells.

Learn more about our cryopreserved models

UltiDIFF technology platform

Our core technology platform, UltiDIFF, has been developed to optimize the large-scale generation of iPSC-derived hepatocytes with field leading purity and functionality. These cells can be employed for drug screening, using both diseased and Wild-Type hepatocytes.

DefiniGEN have the capability to generate diseased hepatocytes through two avenues. Firstly, by collecting somatic cells from a diseased patient, maintaining their genetic defect through regeneration to iPSCs, and then utilizing the UltiDIFF process. Secondly, for rarer mutations, we can employ gene editing tools like CRISPR/Cas9 to introduce specific mutations into iPSCs, generating the diseased cell lines.



Models

DefiniGEN have developed a number of in-house exemplar rare disease models, these include:

- Alpha-1 antitrypsin deficiency (A1ATD)
- Wilson's disease
- Progressive familial intrahepatic cholestasis type 2 (PFIC2)
- Ornithine transcarbamylase (OTC) deficiency

Each of our exemplar models is supported with disease circuit verification, differentiation capability to end cell type, and phenotypic validation data. In addition, our A1ATD and Wilson's disease models feature phenotypic recovery and compound response data.DefiniGEN offers the ability to develop custom models for screening or characterizing lead compounds for indications that have previously been difficult to study.

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