AXCELEAD
Drug Discovery Partners

AXCELEAD

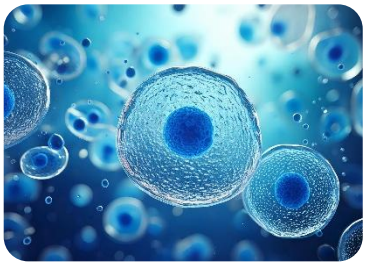
Advancing Rare Disease Research with an Integrated Disease Model Platform

Comprehensive support for understanding rare-disease pathophysiology, assay development, and assessing drug efficacy.

1. Patient-derived Models*

Identification of disease-specific pathways and targets

2. iPS Cell Disease Models



Evaluation of disease phenotype recapitulation and disease-specific responses

3. Genetically Engineered Animal Models

Functional validation and in vivo efficacy confirmation

1.“Patient-derived models” accurately capture disease pathophysiology

Models derived from patient samples provide the foundation for target identification, efficacy evaluation, and biomarker discovery.

Biomarker and patient stratification signature discovery

Extraction of signatures reflecting treatment response and disease progression, supporting patient stratification

Elucidation of disease mechanisms

Identification of disease-specific pathways through multi-omics and pathology analyses of patient samples

Proof of Mechanism (POM)

Quantification of target engagement and downstream signaling changes to demonstrate on-target drug activity

Patient Sample Analysis Drives Six Key Elements of Drug Discovery

Target validation

Confirmation of expression, activity, and relevant cell types in patient samples to support clinical validity

Mechanism of Action (MOA)

Integration of gene expression, immunophenotypes, and cell-network data to clarify pathways

Efficacy evaluation

Establishment of assays using cellular and molecular readouts from patient samples to enable in vitro–in vivo translation

Technology Platform ● Pharmacology ● Omics ● Pathology ● Bioinformatics

*Patient sample source will be discussed with the client.

2. “Cell-based disease models” reliably recapitulate patient-specific phenotypes and genetic backgrounds

Using patient-derived or mutation-engineered iPS cells, we evaluate disease-relevant phenotypes and differential drug responses. This supports a deeper understanding of disease biology.

Establishment and evaluation of patient-mutation knock-in iPS cells at Axcelead

Genome Editing
- CRISPR/Cas9
- Design of editing strategy
- Verification of gene editing (sequencing, etc.)

Quality Check
- Pluripotency confirmation
- Trilineage differentiation assay
- Karyotype analysis
- Mycoplasma testing

Differentiation
- Differentiation to CNS cells, etc.
- Evaluation of differentiation efficiency (e.g., ICC, FCM)
- Cell banking

Pharmacological Assay
- Disease-specific expertise
- Assay development
- Efficacy evaluation

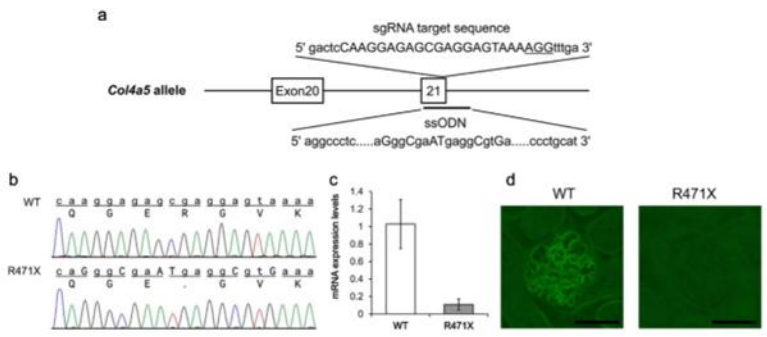
3. “In vivo disease models” recapitulate disease pathology and enable efficacy evaluation

Animal models engineered with disease-associated gene SNPs enable in vivo target validation and drug-efficacy studies, strengthening confidence in therapeutic hypotheses.

Generation of genetically engineered animals at Axcelead

- Extensive experience in generating KI mouse and rat models with 1 bp–40 kbp insertion sizes
- Generation of lethal-mutation models enabled by temporal and spatial control of expression
- Rapid KI mouse generation using CRISPR/Cas9 (50 mice in ~9 months)

Generated mice carrying a point mutation in type IV collagen, similar to Alport syndrome patients



Patient-like phenotype
- Glomerular basement membrane damage
- Severe albuminuria
- Decreased GFR, etc.

Biochem Biophys Rep. 2018;17:81–86.
doi:10.1016/j.bbrep.2018.12.003.

Axcelead Drug Discovery Partners, Inc.
https://www.Axcelead-us.com

WEB LinkedIn

ADDP_0035E_202603