Axcelead

Target ID/Validation
- Axcelead Target Discovery Engine -

Axcelead Target Discovery Engine

Client

Direction for Drug generation
- Strategy such as Therapeutic area
- Focused pathway
- Focused gene & Genome

GOAL

Generation of Drug target

AXCELEAD Drug Discovery Partners

【Validity】

① Data toward Direction

- Cell line
- Animal models
- Clinical samples
- Clinical information

(From Client or Axcelead)

Omics analysis

- Transcriptome
- Proteome
- Metabolome
- Lopidome

② Screening for Drug target candidate

- Enrichment analysis. Network analysis
- Axcelead original “Finger Printing” analysis
- Scientific intelligence by AdisInsight & Cortellis

- Prioritization with legacy data from Takeda

FRONTEO
- Natural language processing by AI

【Reliability】

③ Validation study for Drug target candidate

in vitro/vivo model

④ Prioritization of Drug target candidate

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Next Generation Sequencing (NGS) and Bioinformatics

1.Acquisition of AmpliSeq data using NGS

- Global gene expression profile (applicable to frozen cells from 1well of 96-well plate)
- DNA mutation of specific genes （can handle clinical samples)
- Fingerprinting of compounds, ASOs

2.Visualization of omics data, making hypothesis from your data

- Using your data, and public data
  - ✓ Prioritization of chemotypes/compounds
  - ✓ Profiling of your compounds for repositioning
  - ✓ MOA verification of your compounds, ASOs, antibodies
  - ✓ Searching marker candidates
  - ✓ Evaluation of extrapolation to humans using DTC DNA genetic testing data
- Using single-cell RNA-seq data
  - ✓ Understanding cell variety, finding novel cell sub-populations, building differentiation trajectories
  - ✓ Searching cell surface markers

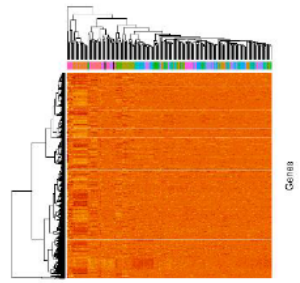
3. Consulting

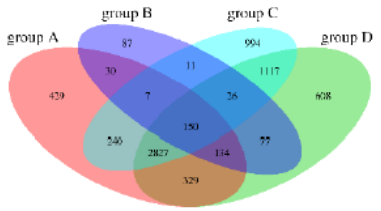
- Experimental design for omics data acquisition
- Wet validation
- etc.

Samples and omics data

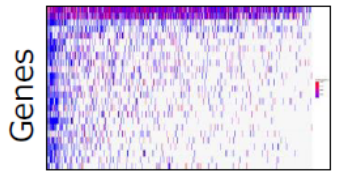
Patients | Animal modes | in vitro data → Original data | Data@public

Visualization・hypothesis





Heat map of mutations



❖ **We will support you from data acquisition, hypothesis making, through validation steps**

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Proteomics/Metabolomics

1. Metabolome and proteome wide-approach

- Our high-end platforms assess about **600 metabolites, 30 lipid classes, 7000 proteins and 15000 phosphorylation sites** from a single biological sample.
- This approach enable **a promising discovery of biomarker or mechanism of interest with time- and cost-saving** compared with the traditional narrow-target approach.

2. Our multi-omics platforms using mass spectrometry technology

- **Metabolomics platform** focuses on major metabolic pathways including carbohydrate, energy, lipid, nucleotide and amino acid metabolism.
- **Lipidomics platform** covers various simple and complex lipids by combination of unique one-step lipid extraction, liquid chromatography and high-resolution mass spectrometry technologies.
- **Proteomics platform** detect alteration of protein expressions and phosphorylation profile with high quantitativity by stable isotope labeling and peptide fractionation technologies.

❖ **Our omics technologies offer the efficient biomarker discovery and MoA analysis through metabolome- and proteome-wide approaches.**

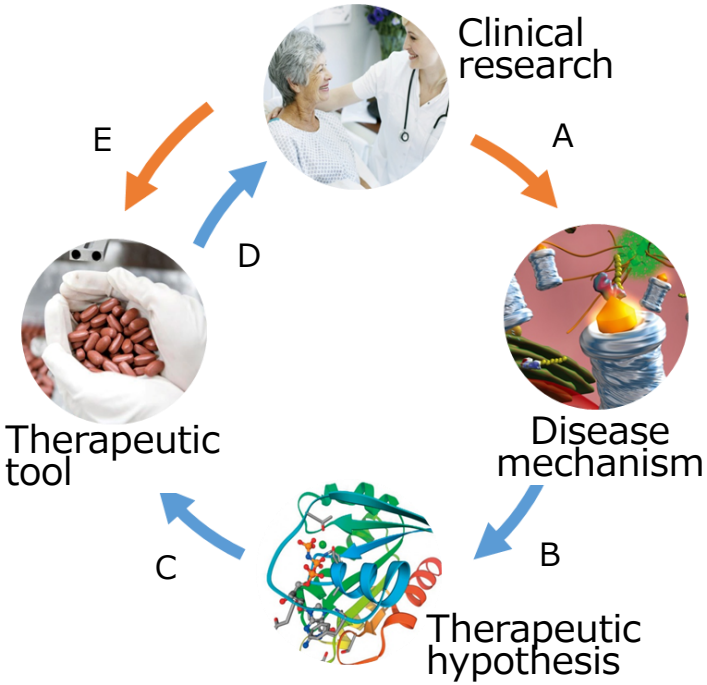


Fig. Applications of omics technologies for Drug Discovery and Development

A) Disease profiling
B) Therapeutic MoA analysis
C) Discovery of clinical biomarkers
D) Biomarker assessment in clinical study
E) Profiling of therapeutic response

In vitro validation study

◆ Cell lines form human
◆ Fresh peripheral blood

Tumor Cells TA: Oncology
PBMC TA: Inflammation
T・B cells TA: Immunology
Other cell lines

◆ iPS derived cell lines

Cardiac Cells TA: CV
Endothelial Cells
Neural Cells TA: CNS
Macrophages TA: Inflammation
Hepatocytes TA: Metabolism

Platform for In vitro study

◆ **Tool compound**

Agonist/ Antagonist

(Purchased/ADDP original)

◆ **Genetically modification**

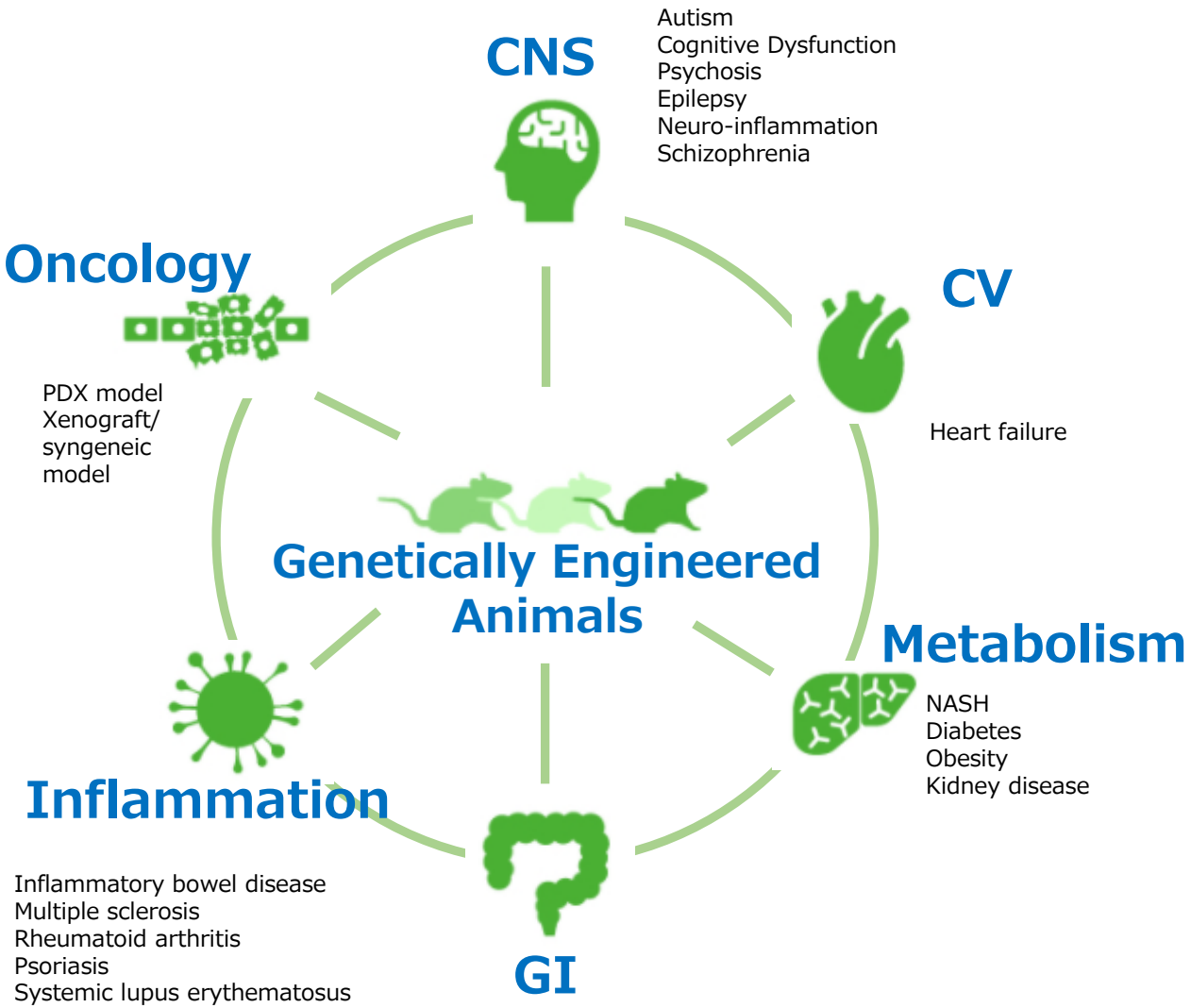
siRNA/shRNA (KD)

CRISPR/Cas9 (KO/KI)

◆ **One stop service**

Research for Seamless by specialist

In vivo validation study



Platform for In vivo study

- **Tool compound**
  - Agonist / Antagonist
  - (Purchased/ADDP original)
- **Genetically modification**
  - CRISPR/Cas9 (Berkeley license) (KO/KI)
  - 4 Mo to generate F0-Homo KO
- **One stop service**
  - Research for Seamless by specialist