Introduction of Regenerative Medicine Unit and Takeda-CiRA Joint Program

Seigo Izumo, M.D.
Global Head of Regenerative Medicine Unit
Mission of Regenerative Medicine Unit

*We aspire to become an industry leader in regenerative medicine by bringing definitive therapies to patients with life threatening disease.*
Takeda’s External Collaborations in Cell & Gene Therapy

**GI**
- **TiGenix**
  - Cx601 for complex perianal fistula in Crohn’s disease

**T-CiRA Joint Program**
- **Kyoto University**
- **Yokohama City University**
- **Riken**

**Oncology**
- **GAMMADELTA**
- **NOILE-IMMUNE BIOTECH**
  - Novel T cell platform for a broad range of cancers
  - Yamaguchi University
  - National Cancer Center

**Sanford Consortium**
- **Sanford Consortium for Regenerative Medicine**
  - Salk Institute for Biological Studies
  - UC San Diego

**CNS**
- **K Pharma**
  - Keio University
In 2015, Takeda and Center for iPS Cell Research and Application (CiRA) at Kyoto University agreed to start a ten-year-long research collaboration (T-CiRA Joint Program)
Takeda-CiRA Joint Program: Unique academia-industry collaboration

1. Long term (10 years) commitment by both CiRA and Takeda
2. Over 100 researchers at one site.
3. Aim: To deliver cell therapies and drug discovery using human iPS cells
4. "Reverse" academia-industry collaboration in the center of a drug company's research facility.
5. Co-ownership of Intellectual Properties (50:50)
The Framework of T-CiRA Collaboration and Clinical Candidate Development

**CiRA**
- iPS cell science / Technology
- Targets / Prototype assays
- PIs / Postdoctoral researchers

**Takeda**
- R&D Know-how
- Facility / Equipment
- Researchers
- Drug Discovery Platforms
- Budget

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**T-CiRA Joint Program in Shonan Research Center**
- Cell Therapy
- Gene Therapy
- Drug Discovery
- Drug Safety and Efficacy

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**Support by Takeda Group**
- Screening library
- Pharmacology
- Safety
- Early clinical trial etc.

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**Partnerships**
( Co-development, New-co, Joint Venture, etc)

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**Candidate Cell**
- Candidate Cell

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**Candidate Compound**
- Candidate Compound

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**Cell Therapy**
- Cell Therapy

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**Gene Therapy**
- Gene Therapy

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**Drugs Biologics**
- Drugs Biologics

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**IPs not exercised by Takeda**
**T-CiRA Collaboration**

-Dedicated Space in Shonan Research Center-

**T-CiRA Program**

- Located in Fujisawa, Japan
- Approximately 7,500m² total floor area
- More than 100 scientists from academia and Takeda
- All projects use iPSC technologies
- Considered as a branch of CiRA by Kyoto University
### Cell Therapy Group

- **Taro Toyoda** (CiRA, Kyoto Univ.)
  - Type 1 diabetes

- **Yoshinori Yoshida** (CiRA, Kyoto Univ.)
  - Heart failure

- **Shin Kaneko** (CiRA, Kyoto Univ.)
  - T-cell therapy for cancer
  - Immune tolerance for organ transplantation

### Drug Discovery Group

- **Haruhisa Inoue** (CiRA, Kyoto Univ.)
  - Amyotrophic Lateral Sclerosis (ALS)

- **Yoshinori Yoshida** (CiRA, Kyoto Univ.)
  - Cardiomyopathy

- **Hidetoshi Sakurai** (CiRA, Kyoto Univ.)
  - Intractable muscular disease

- **Tadashi Suzuki** (RIKEN)
  - Drug discovery for NGLY1 deficiency

### Platform Technology

- **Makoto Ikeya** (CiRA, Kyoto Univ.)
  - A new research platform with neural crest cells

- **Takanori Takebe** (Yokohama City Univ.)
  - Organoid technology

- **Akitsu Hotta** (CiRA, Kyoto Univ.)
  - Genome editing therapy for Duchenne muscular dystrophy
Game-Changing Therapeutics to be Delivered from the T-CiRA Program

T-CiRA aims to enter clinical trials within 3-4 years and submit approval applications within 9 years.

- **Cell Therapy**
  - Outcome
    - Tumor regression or cure
    - Improved survival

- **Drug Discovery for ALS**
  - Outcome
    - Recovery of motor function
    - Suppression of neuronal degeneration

- **Cell Therapy**
  - Outcome
    - No need for heart transplantation
    - Prevention of death

- **Gene Editing**
  - Outcome
    - Treatment of muscular dystrophy
    - Treatment of myopathy

- **Drug Discovery**
  - Outcome
    - Prediction of liver toxicity
    - Drug screening using mini-organ

- **Islet**
- **Neuron**
- **Cardiomyocyte**
- **Hepatocyte**
- **Skeletal Muscle**
- **T-cell**
- **Differentiation**
- **iPS Cell**
Transformation of Shonan Research Center into an Open Innovation Hub

2017.3

Shonan Research Center

CNS Research

Regenerative Medicine Unit

T-CiRA

Platforms and Pre-Clinical Support Functions
Transformation of Shonan Research Center into an Open Innovation Hub

2017.3
- Shonan Research Center
  - CNS Research
  - Regenerative Medicine Unit
  - T-CiRA
  - Platforms and Pre-Clinical Support Functions

2017.10
- Shonan Health Innovation Park
  - CNS Research
  - Regenerative Medicine Unit
  - Pharma Science
  - T-CiRA
  - Axelead Drug Discovery Partners
  - SochiaPharma, Cardurion
    EVPs: Seedsupply, ChromaJean, etc
  - Noile-Immune, K-Pharma
  - Open Labs: Other tenants
  - Business Support (e.g. legal, IP, Finance, IT etc.)

Takeda Research
Open Innovation Hub
T-CiRA: A New Form of Academia-Industry Collaboration at Shonan Health Innovation Park

T-CiRA

A joint program between Takeda and Center for iPS Cell Research and Application (CiRA) that will change the future of medicine using iPS cells

URL:https://www.takeda.com/t-cira/