January 29, 2016

Janet Rossant, PhD – President and Scientific Director

Economic Burden of Disease

Chronic and degenerative diseases cost in Canada
$190 billion annually
(Public Health Agency of Canada 2010)

Direct costs for hospitals, doctors and drugs come in at
36% ($68 billion)

Indirect costs including disability and premature death account for
64% of all costs ($122 billion)

Heart disease affects
1.3M Canadians, costing more than $22B every year and is the number one cause of death in men and women
(PHAC 2009)

The economic burden of Diabetes in Canada is about $12 billion per year, affecting 2.4 million individuals with high rates of complications and comorbidities
(CDA 2010)

- These diseases are based on the breakdown of cells and tissues – cells and tissues that can be regenerated by stem cells
- One regenerative medicine treatment holds the potential to save billions of dollars

Ontario Institute for Regenerative Medicine

- Established in 2014 by Ministry of Research and Innovation, funded by Ontario Government
- Not-for-profit research and commercialization institute

Vision: To revolutionize the treatment of degenerative diseases, making Ontario a global leader in the development of stem cell-based products and therapies

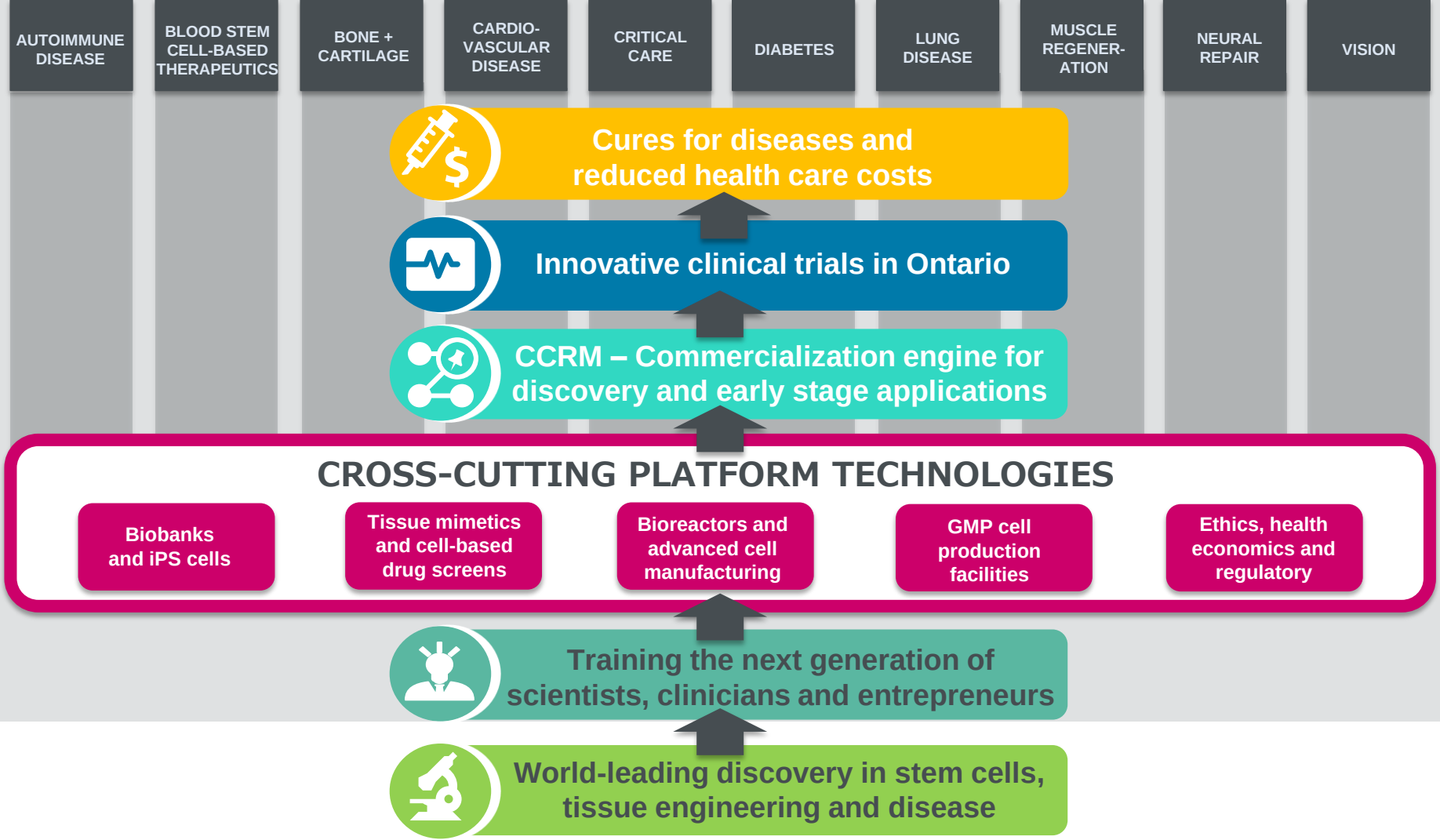
Lead the translation of stem cell and regenerative medicine research into improved health and economic growth in Ontario

Enhance excellence in stem cell and regenerative medicine research in Ontario

Educate and engage the public about the excitement and impact of stem cell science and regenerative medicine

Why Ontario?

- World-leading scientific discoveries in stem cell and regenerative medicine for 50+ years
- Collaborative network of over 170 scientists across Ontario including stem cell biologists, biomedical engineers and translational clinicians
- Established commercialization partner - the Centre for Commercialization of Regenerative Medicine
- World-class clinical infrastructure + expertise in implementing cell therapy trials
- Favourable regulatory environment and philanthropic community


AUTOIMMUNE DISEASE
BLOOD STEM CELL-BASED THERAPEUTICS
BONE + CARTILAGE
CARDIO-VASCULAR DISEASE
CRITICAL CARE
DIABETES
LUNG DISEASE
MUSCLE REGENER-ATION
NEURAL REPAIR
VISION
Cures for diseases and reduced health care costs
Innovative clinical trials in Ontario
CCRM – Commercialization engine for discovery and early stage applications
CROSS-CUTTING PLATFORM TECHNOLOGIES
Biobanks and iPS cells
Tissue mimetics and cell-based drug screens
Bioreactors and advanced cell manufacturing
GMP cell production facilities
Ethics, health economics and regulatory
Training the next generation of scientists, clinicians and entrepreneurs
World-leading discovery in stem cells, tissue engineering and disease

Regenerative Medicine Trials in Ontario 2015

Brain and/or nerves

Phase II using MSCs for Multiple Sclerosis: Toronto*

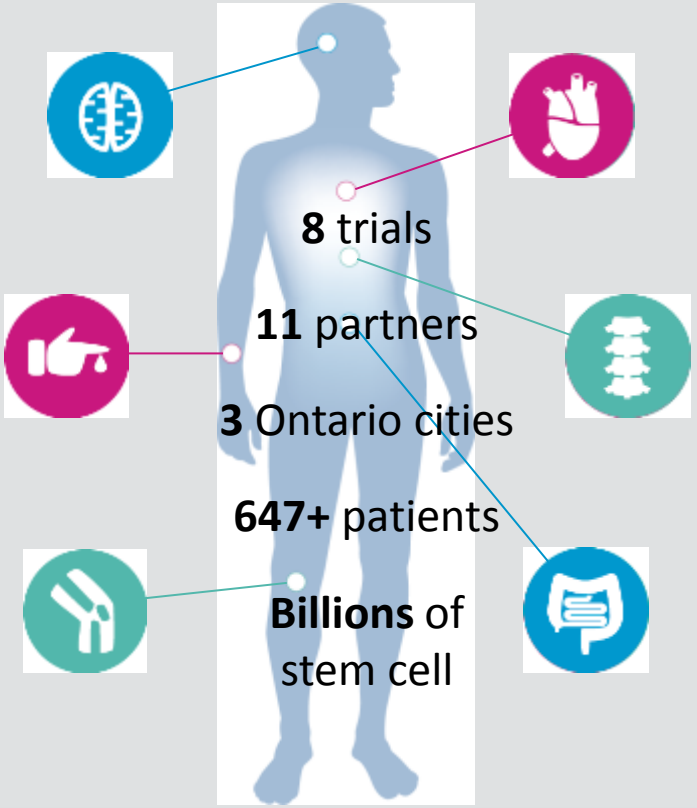
Phase III using diabetes drug to stimulate brain repair for malignant brain tumours: Toronto*

Blood

Phase I using immune cells for blood cancer relapse after bone marrow transplantation: Toronto*

Joints

Phase I-II using stem cells mid- to late-stage knee osteoarthritis; Toronto*



Heart

Phase II using genetically modified stem cells following heart attack: Ottawa and Toronto*

Phase II using MSCs for advanced heart failure with left ventricular assist device: Toronto*

Spine

Phase I/II using neural stem cells for cervical spinal cord trauma: Toronto*

Digestive system

Phase III using MSCs to induce remission of Crohn's disease: London and Toronto

* OIRM researchers leading trial

OIRM Priorities: 2016

- Funded 4 new Disease Teams moving forward to the clinic :
  - Heart regeneration with stem cell–derived heart muscle cells
  - Repairing white matter in the brain following disease or injury in children
  - A stem cell approach to regenerate the injured spinal cord
  - Cellular therapy for septic shock
- Supporting 12 New Ideas grants, 6 postdoctoral fellowships, clinical trials and other workshops, patient and public engagement

Partnerships

- Building research and commercial partnerships locally, nationally and internationally:
  - Multiple academic and hospital research institutes across Ontario
  - Health charities
  - Other provincial and federal institutes
  - International partnerships with China and Japan

Canada-Japan Joint Research Program in Epigenetics of Stem Cells 2013-2018

- Five year funding for three teams to carry out collaborative research in stem cells and disease applications

Epigenetics driving new approaches to stem cell and leukemia therapies

CANADA:

John Dick, Princess Margaret Cancer Centre and University of Toronto

Gordon Keller, McEwen Centre for Regenerative Medicine and University of Toronto

Mathieu Lupien, Princess Margaret Cancer Centre and University of Toronto

JAPAN

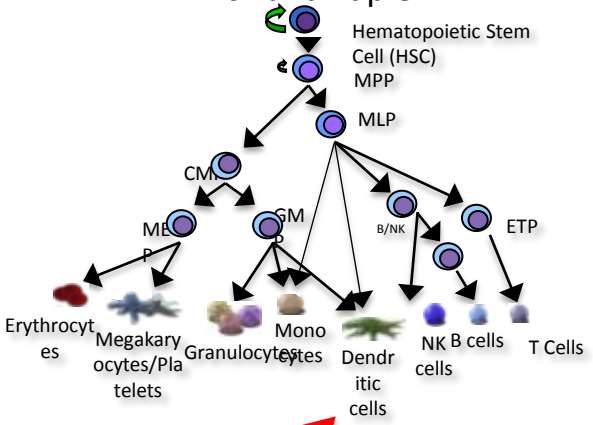
Hiromitsu Nakauchi, Tokyo University

Seishi Ogawa, Kyoto University

Epigenetics driving new approaches to stem cell and leukemia therapies

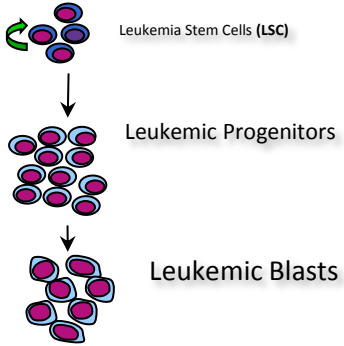
NORMAL-Gold Standard

Dick and Lupien



LEUKEMIA

Ogawa and Dick

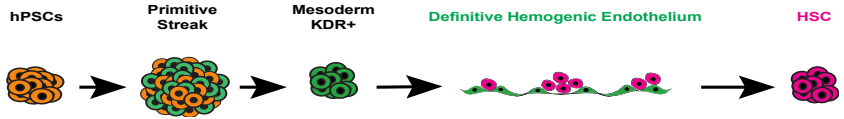


Shared personnel and populations

Epigenetic and transcriptional guidance to overcome barriers

Developmental approaches to HSC generation ex vivo

Keller



Teratoma-based HSC generation

Nakauchi

Genetic and epigenetic hierarchies distinguishing pluripotent and trophoblast stem cells

CANADA:

Janet Rossant, Hospital for Sick Children and University of Toronto

Bill Stanford, Ottawa Health Research Institute and University of Ottawa

Brian Cox, University of Toronto

JAPAN

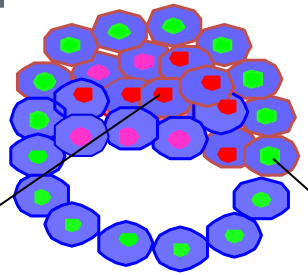
Hitoshi Niwa, Kumamoto University

Minoru Ko, Keio University

Satoshi Tanaka, Tokyo University

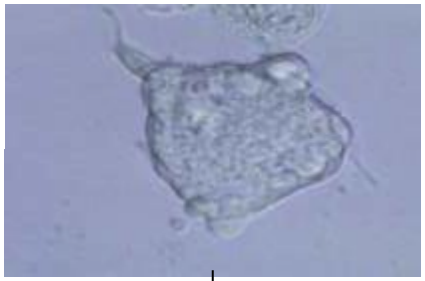
Genetic and epigenetic hierarchies distinguishing pluripotent and trophoblast stem cells

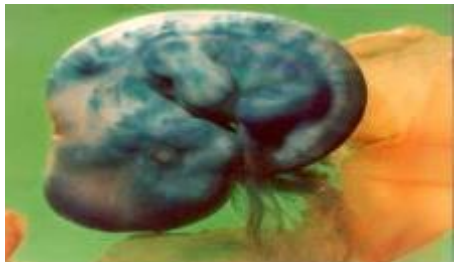
Stem cells from the mouse blastocyst



trophectoderm
epiblast progenitor
primitive endoderm progenitor

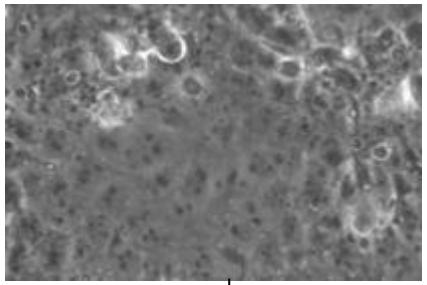
Pluripotent ES cells

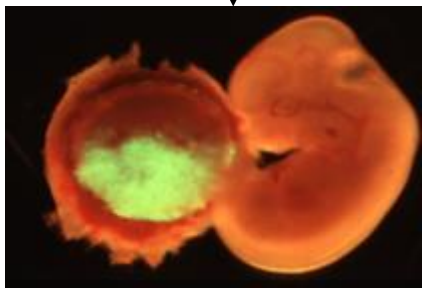




Overexpress TS factors
Change epigenetics
Express microRNAS

Placental specific trophoblast stem cells





Can we define genetic and epigenetic networks that distinguish ES and TS cells and use to develop human TS cells?

Directing Cellular Identity Towards Progenitor Cell Therapies

CANADA:

Andras Nagy, Lunenfeld-Tanenbaum Research Institute and University of Toronto

James Ellis, Hospital for Sick Children and University of Toronto

Jacques Tremblay, Universite de Laval

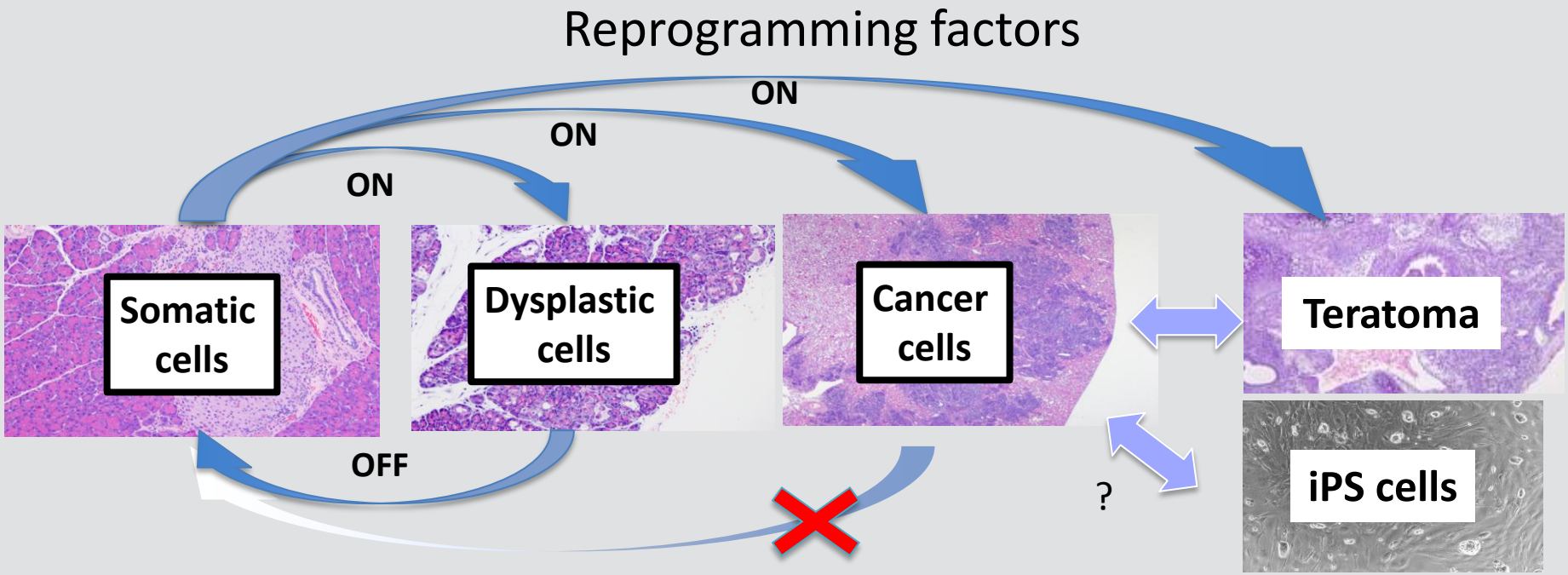
JAPAN

Yasuhiro Yamada, CiRA, Kyoto University

Yoichi Shinkai, RIKEN

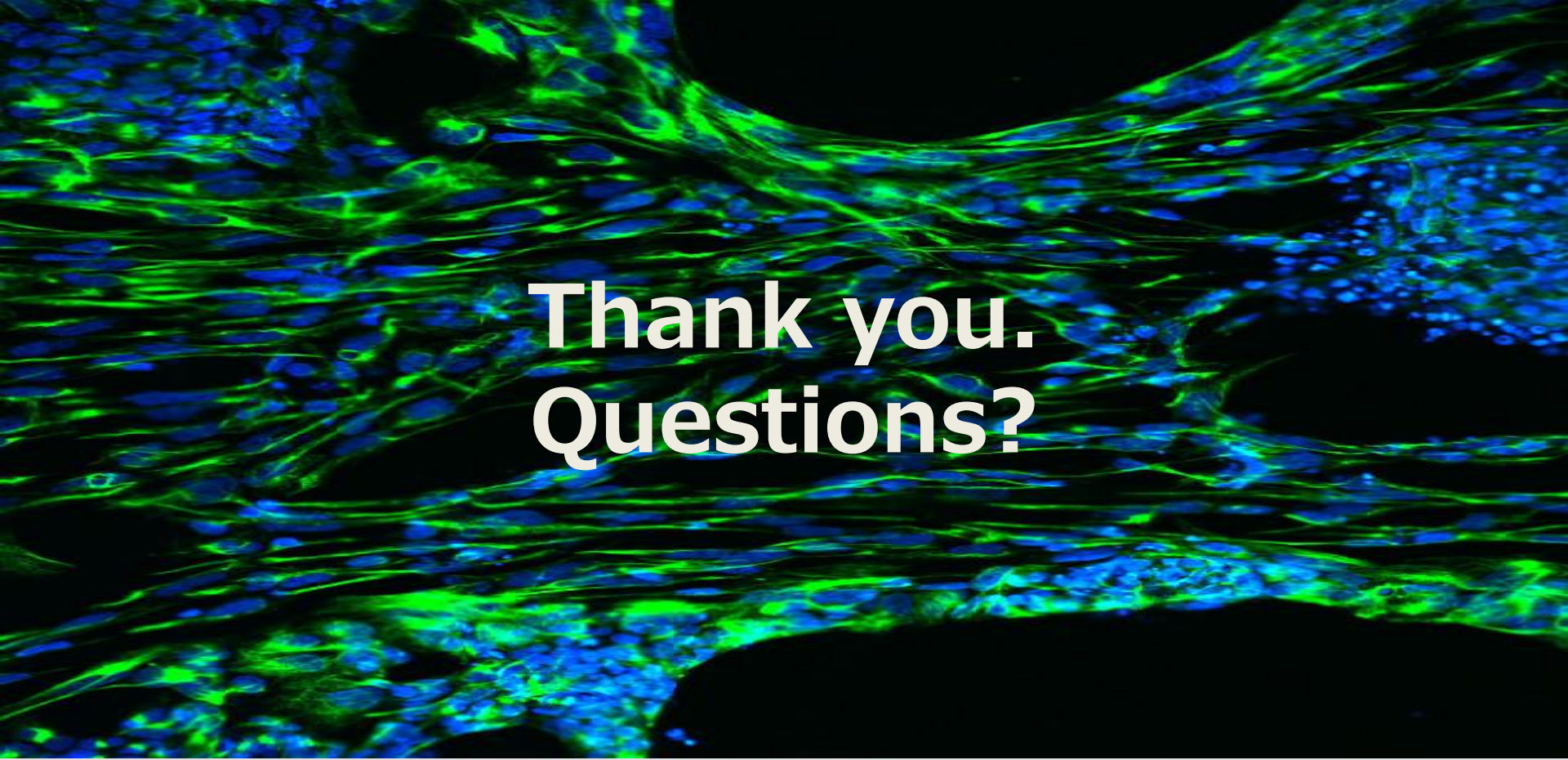
Knut Woltjen, CiRA, Kyoto University

Successful and failed reprogramming and its relationship to cancer



Ongoing Japan-Canada Regenerative Medicine Interactions

- Exchange of trainees
- Canadian stem cell–trained faculty in Japan
- Academic partnerships around stem cell discoveries
- Commercial partnerships around stem cell IP
- New opportunities for partnered innovative cell therapy trials


Thank you.
Questions?