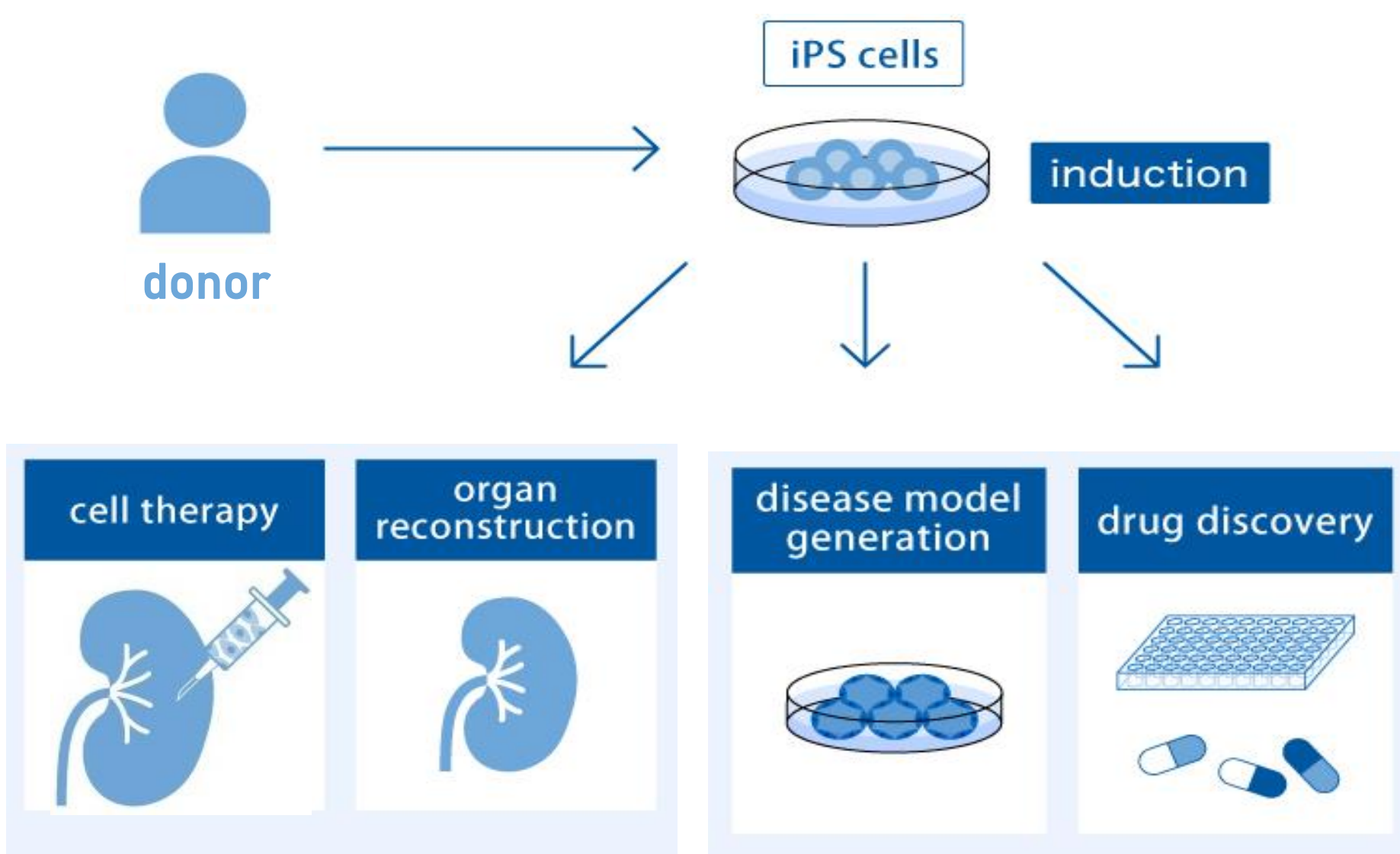


Cell Therapy and Drug Discovery from iPS Cell-Derived Renal Cells

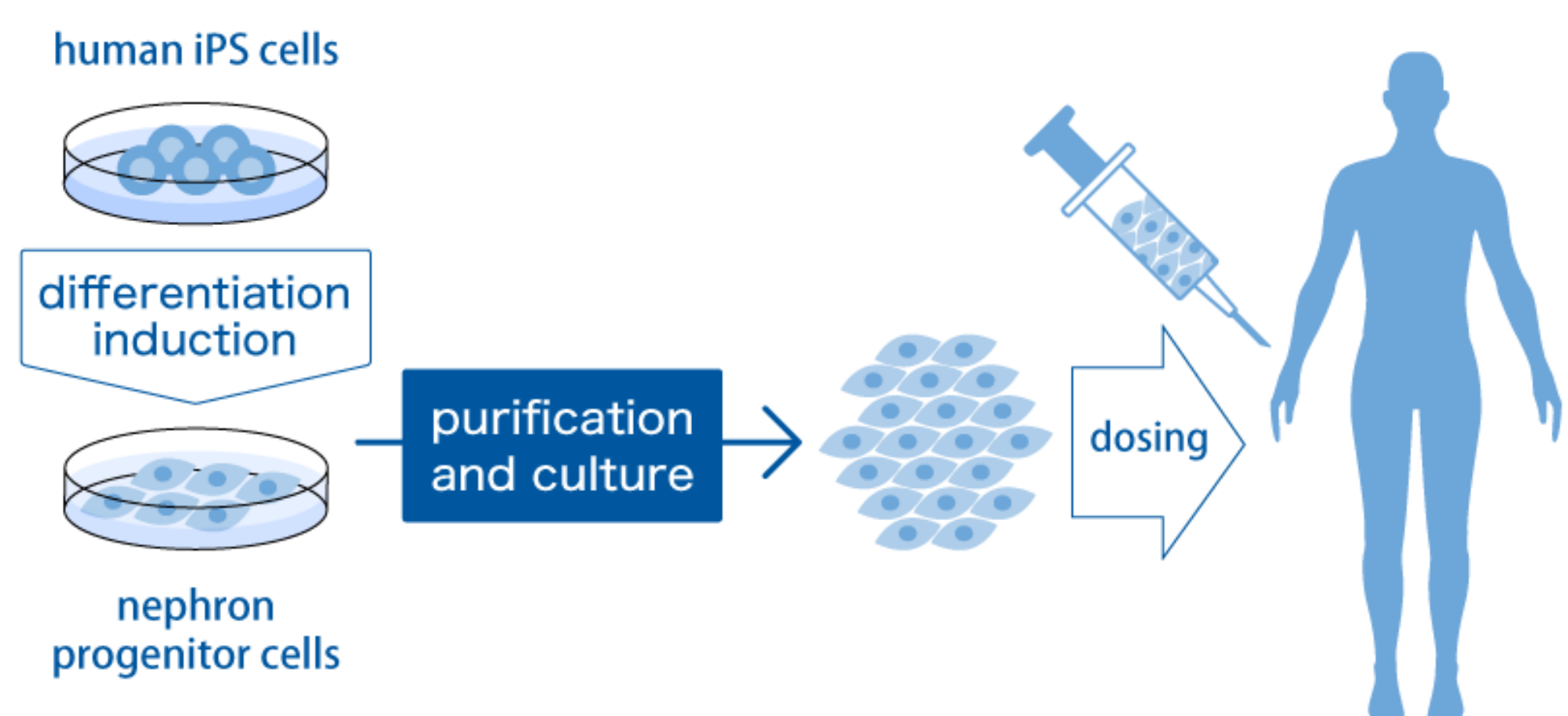


- ▶ Developing cell therapy for chronic kidney disease and small molecule drugs for autosomal dominant polycystic kidney disease (ADPKD)
- ▶ Focusing on kidney. Liver and pancreas are next our focus
- ▶ Established in 2019 to implement the seeds of Prof. Osafune of the Center for iPS Cell Research and Application, Kyoto University

Product: Cell Therapy for Chronic Kidney Disease (iNPC)

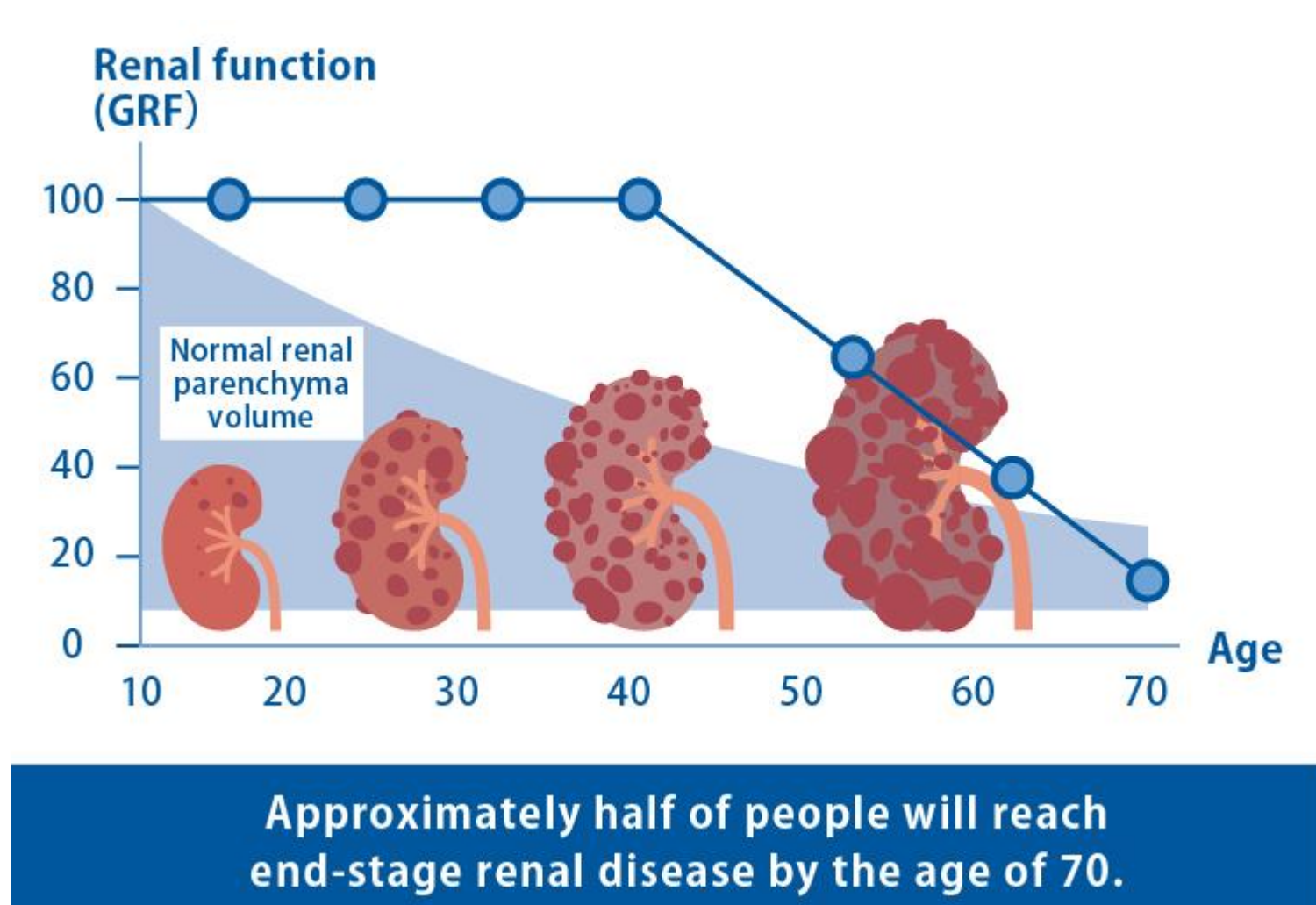
Cost of Dialysis
 Japan : **\$50K/patient**
 US : **\$100K/patient**

Cost of End-Stage Renal Disease
 Japan : **\$10B**
 US : **\$50B**



- ▶ **Nephron progenitor cells (NPCs) are cells that give rise to nephrons**
 - ▶ NPCs improve the function of damaged kidneys and were firstly identified by Prof. Osafune
- ▶ **iNPCs are NPCs induced from allogeneic human iPSCs**
 - ▶ Transplant iNPC (RN-032) into renal subcapsular space
- ▶ **Currently, no known cure for chronic kidney disease other than kidney transplantation**
 - ▶ The number of donated kidneys is 20,000 in US and 2,000 in Japan, far from sufficient
 - ▶ Patients with end stage renal disease are forced to undergo dialysis

Product: ADPKD Therapeutics from iPS Drug Discovery



Disease-specific iPS cell-derived human ADPKD disease model



- ▶ **Autosomal dominant polycystic kidney disease (ADPKD) is the most common life-threatening genetic disease caused by a single gene mutation**
 - ▶ Cystic formation in kidney is a hallmark of ADPKD, eventually needing dialysis
 - ▶ Tolvaptan is currently the only approved therapeutic option but has large unmet medical needs due to a strong side effect
- ▶ **RAR agonist tamibarotene (RN-014) was selected as the candidate for clinical development**
 - ▶ RAR (retinoic acid receptor) agonists effectively inhibited the cyst enlargement in our model
 - ▶ Conducting Phase II clinical studies from Jan 2024

Rege Nephro Co., Ltd.

Management Team



Akifumi Morinaka, PhD, MBA

Representative Director, CEO

- ▶ Venture investment at JAFCO
- ▶ Drug discovery and new business creation at Astellas Pharma
- ▶ PhD from Univ. of Tokyo

JAFCO



Kenji Osafune, MD, PhD

Founder, Chief Scientific Advisor

- ▶ Professor of CiRA, Kyoto Univ.
- ▶ Stem cell research at Harvard Univ.
- ▶ MD from Kyoto Univ. and PhD from Univ. of Tokyo



Katsuhisa Yamaguchi, CPA

Board Director, CFO

- ▶ CFO of Sandoz Japan
- ▶ Finance director of Chugai Pharma UK
- ▶ Finance manager of GSK Japan

SANDOZ
a Novartis company



Toshihiro Ishikiriama

Board Director

- ▶ CEO of Japan Vaccine
- ▶ CFO of GSK Japan
- ▶ Board Director of JCR Pharma



Tsuyoshi Nakano

General Manager, Development

- ▶ Head of clinical development of GSK Japan
- ▶ Global development at Tanabe Pharma
- ▶ More than 60 product licenses



Yoshifumi Kobayashi, PhD

General Manager, Production

- ▶ Cell processing at Astellas Pharma, iPS Academia and iPS portal
- ▶ PhD from Hiroshima Univ.



Achievements

- ▶ **Cell therapy for chronic kidney disease**
 - ▶ Obtaining pre-clinical POCs in mouse CKD models
 - ▶ Development of manufacturing processes
 - ▶ Preparing for non-clinical studies
- ▶ **ADPKD therapeutics**
 - ▶ Obtaining pre-clinical POCs in mouse ADPKD models
 - ▶ Conducting Phase 2 clinical trial
- ▶ **Collaboration Research**
 - ▶ AstraZeneca, NIKKISO, SHIMADZU, Mitsui Chemicals, etc.
 - ▶ **Abu Dhabi Stem Cells Center**



Financing

- ▶ **Seed round \$3.5M in 2020 and Series A \$10.5M in 2022**
- ▶ **Top tier VCs in Japan as lead investors**
 - ▶ Kyoto University Innovation Capital, JAFCO, and MUFG capital
- ▶ **Top tier life science companies as investors**
 - ▶ FUJIFILM, Asahi Kasei, SHIMADZU, and M3
- ▶ **Obtained major grants**
 - ▶ AMED (Japan Agency for Medical Research and Development)
 - ▶ ViCLE (\$4M) and Regenerative Medicine (\$0.5M)
 - ▶ Kyoto prefecture, Kyoto city, etc.



Research and develop various solutions against kidney diseases,
improving QOL among patients suffering from kidney diseases,
and contribute to society

<Inquiry>

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