# NED 2026

ITHS | Institute of Translational Health Sciences
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NED 2026

# Patients in a Dish:

## How human specimen donations are becoming mini-machines for modeling disease and accelerating drug discovery and development

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University of Washington

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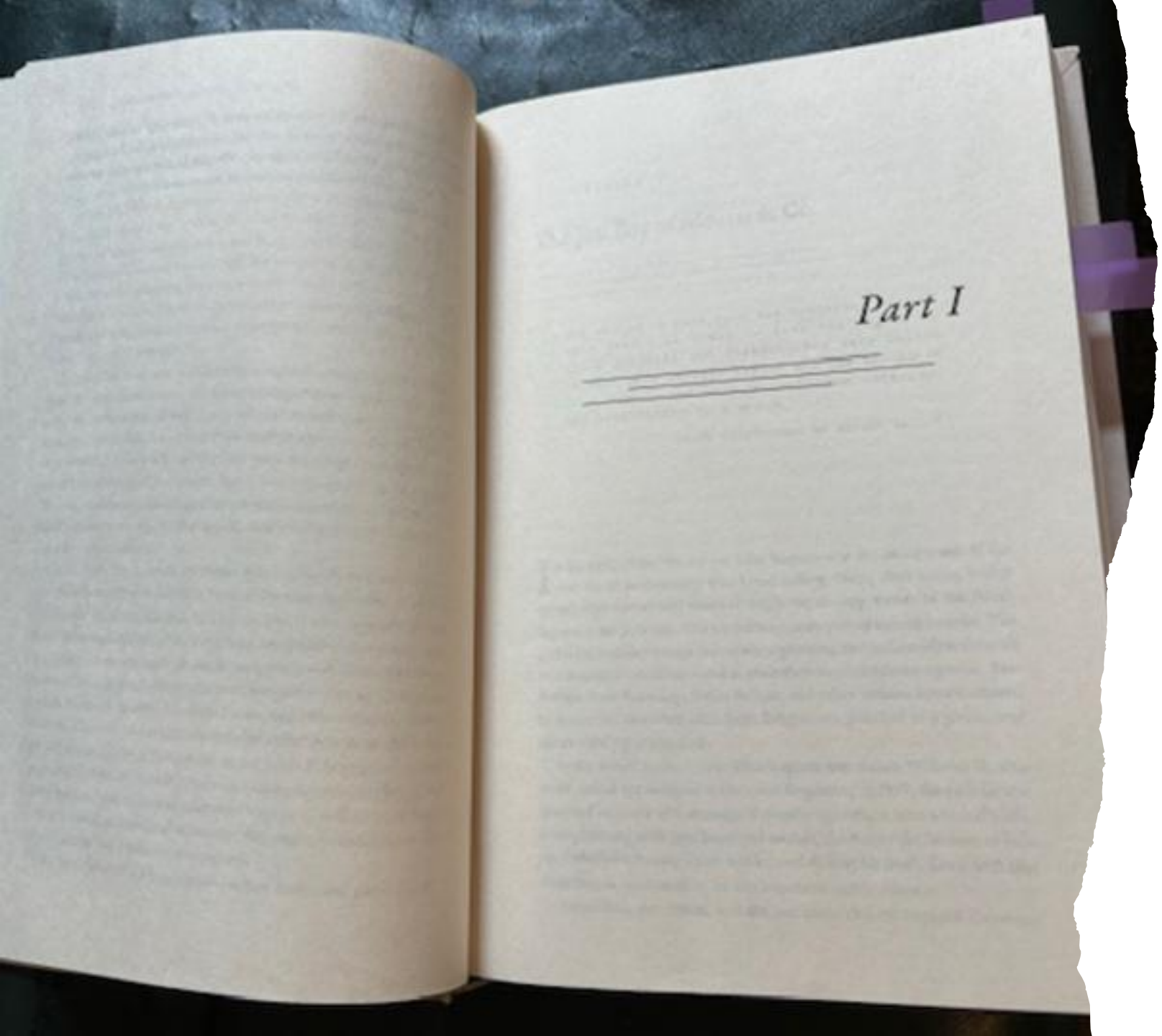



**Patients in a dish?**

**It all starts with Stem Cells**

# What does it mean to be a stem cell?






Differentiation

seed

Mature plant

# And in a human . . .



8-cell human embryo

Successful implantation & pregnancy






(Poster on the wall of Frida Kahlo's studio in Mexico City)

# Where do stem cells come from, and WHAT are their properties?

| Example Cell Type | Potency |
|---|---|
| Embryos | Totipotent – can make a complete human |
| Induced pluripotent stem cells | Pluripotent |
| Umbilical cord blood stem cells | Multipotent |
| Hematopoietic stem cells | Multipotent |
| Adipose stem cells | Multipotent |

Totipotent, pluripotent, and multipotent

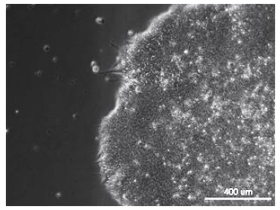
Can become (differentiate into):

- Any cell in the body
- Almost any cell in the body
- Cells in a particular “lineage” or tissue

# Generation of iPS cells



Somatic cells from a donor (Normal or patient), like blood, skin, urine-derived



# Shinya Yamanaka, MD, PhD
# 2012 Nobel Prize in Physiology or Medicine



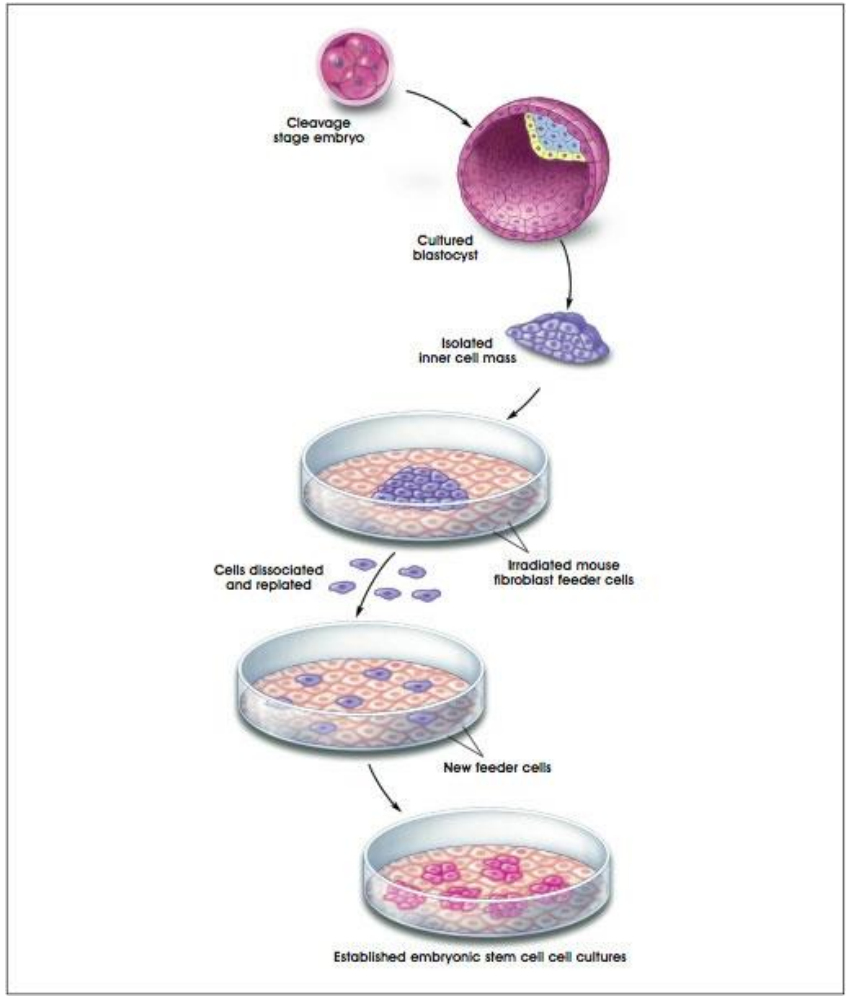
Dr. Yamanaka, senior investigator at the Gladstone Institutes, Director of the Center for iPS Cell Research and Application (CiRA) at Kyoto University



Dr. Yamanaka receiving the Nobel prize from Sweden's King Carl Gustaf

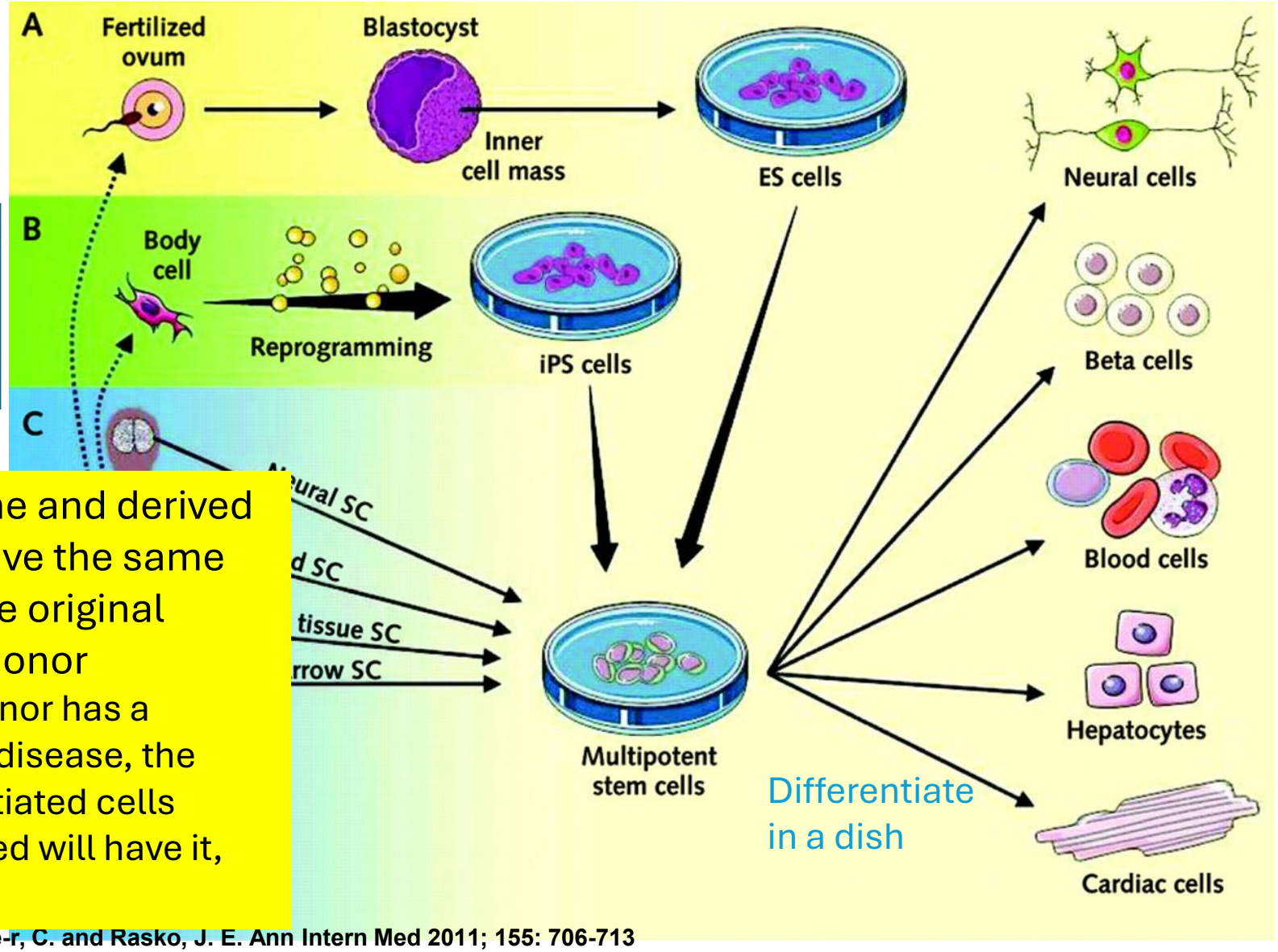
2012 Nobel for his discovery of how to transform ordinary adult skin cells into pluripotent stem cells like embryonic stem cells

# Generating a human stem cell line in the lab: embryo example



# Human stem cell lines can be used to generate different mature cell types for research studies

Blood specimen, skin fibroblasts, cells sloughed off in the urine




The iPSC line and derived cells will have the same genes as the original specimen donor

If the donor has a genetic disease, the differentiated cells generated will have it, too

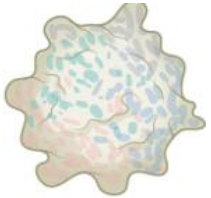
Note: these are often immature, progenitor cells

Powe-r, C. and Rasko, J. E. Ann Intern Med 2011; 155: 706-713

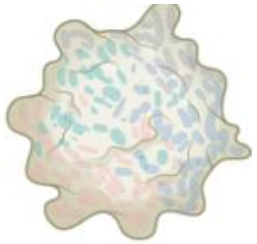
# Example: using stem cells to study the brain

- How do we study diseases of the brain?
  - Parkinson's, Alzheimer's, autism, psychiatric conditions
- Obtain a piece of someone's brain (brain biopsy)?
  - Invasive
  - May only be possible after death
- Animal brain
  - Provides clues, but is not fully representative of a human brain
  - Taking pieces out is invasive
  - Animal studies are expensive
- How about an artificial "brain in a dish"?
  - Could study iPSC-derived neurons in a dish
  - Can also generate an iPSC-derived human neural or cerebral "organoid"
    - Mimics certain brain functions
      - Contains a subset of the types of cells found in the brain
      - Self-organizing!





# “New Approach Methodologies (NAMs)” Organoids



- iPSC-derived in a laboratory dish
- Self-assembling
- Scientists can create organoids representing many  tissue/organ types
  - Very different “cocktails” to create the different types of organoids
- Organoids are not “mini-organs” and have some limitations:
  - Do not retain all of the cell types of the organ
  - Do not have vasculature
  - Core may be necrotic because it is not exposed to flow
- HOWEVER, organoids do enable us to study diseases, biochemical pathways
- Organoids can be “strung together”
- Like the iPSC line, the organoids will have the same genes as the original specimen donor
  - If the donor has a genetic disease, the organoid will have it, too

# How we make organoids: cerebral organoid example



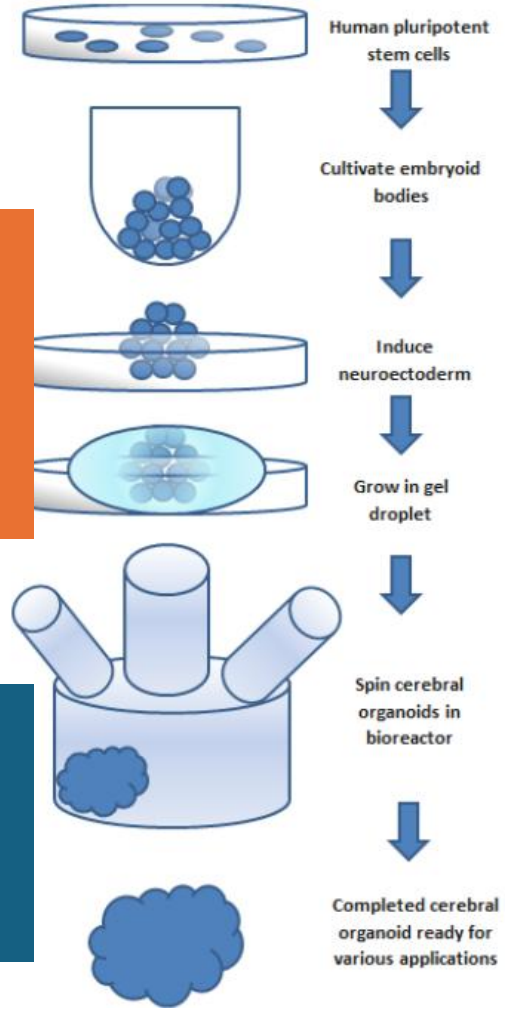

Obtain mature cells, like skin cells or blood cells

**Is this an actual brain? Can it think?**

A flask containing human cerebral organoi

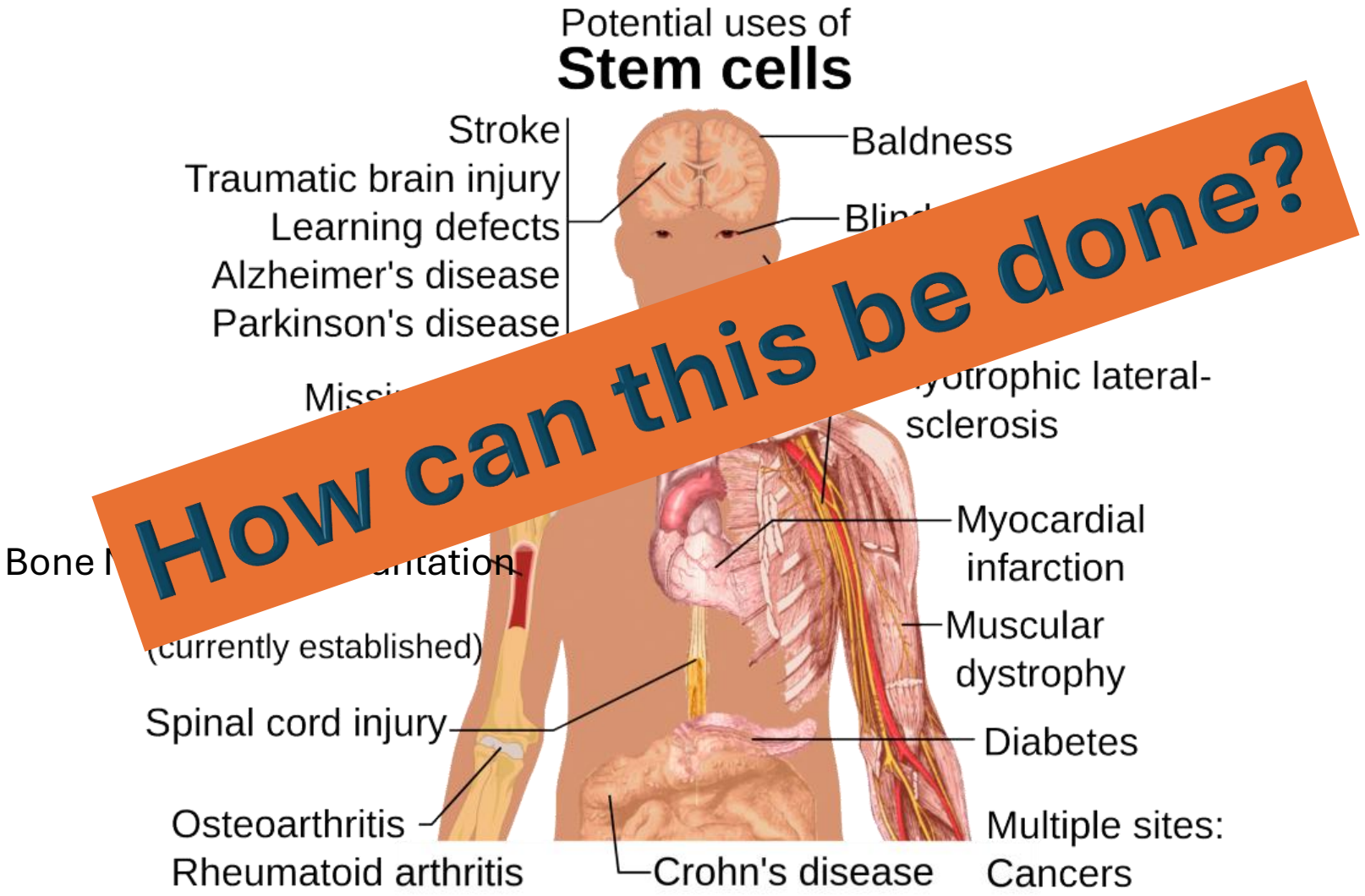


**No – it only contains a subset of brain cells**




Self-assembling!

# What if we could treat or even cure diseases by transplanting stem cell-derived organs?




Thanks to Dr. David Mack, University of Washington

# Efforts to make tissues: kidney example

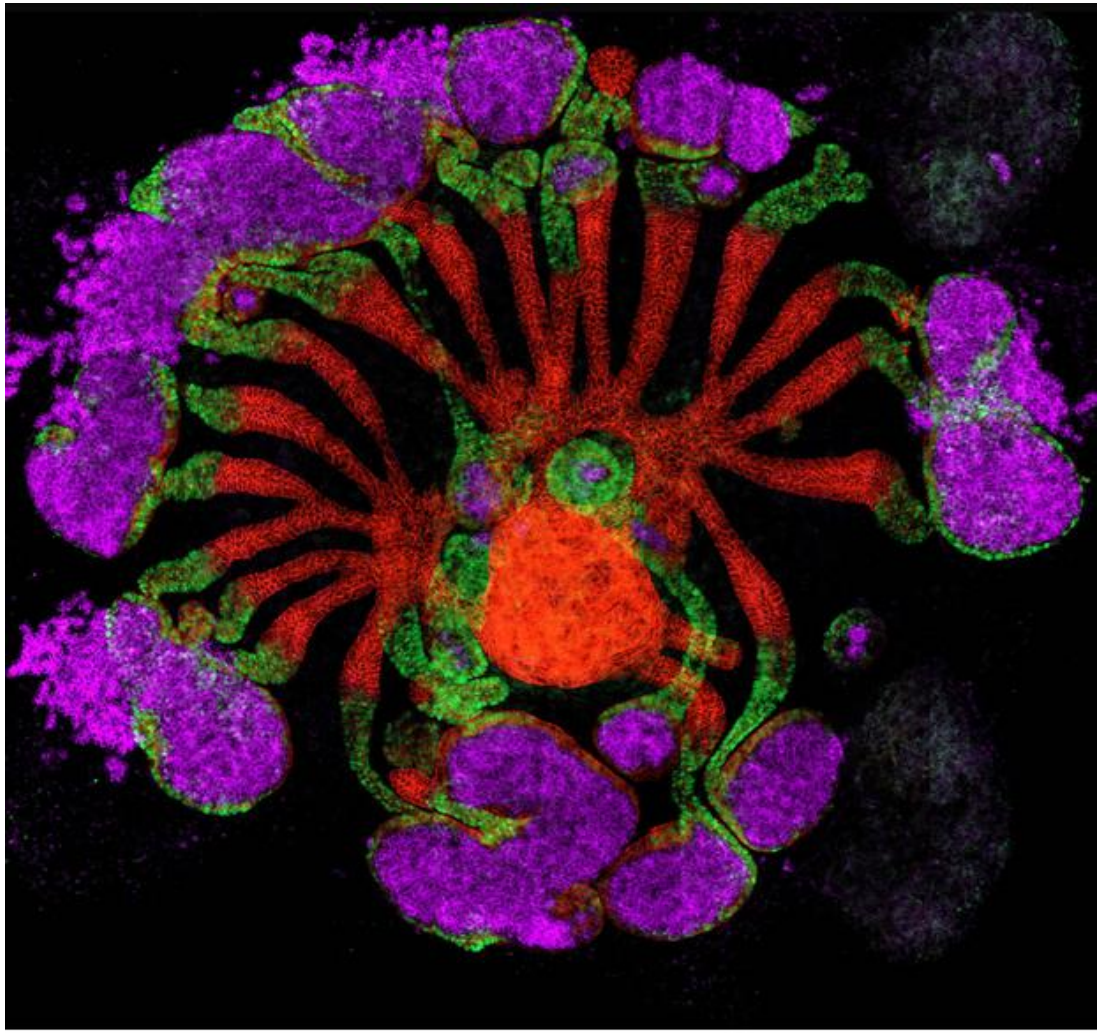
Science

## Scientists make most authentic kidney replicas so far

Lab-grown organoids reproduce some of a kidney's internal structure and function

17 SEP 2025 · 11:00 AM ET · BY MITCH LESLIE

Mouse kidney organoid
1 millimeter wide
Recapitulates some of the intricate internal organization of the kidney
Has filtering units and collecting ducts
Transplanted into mice: makes urine!

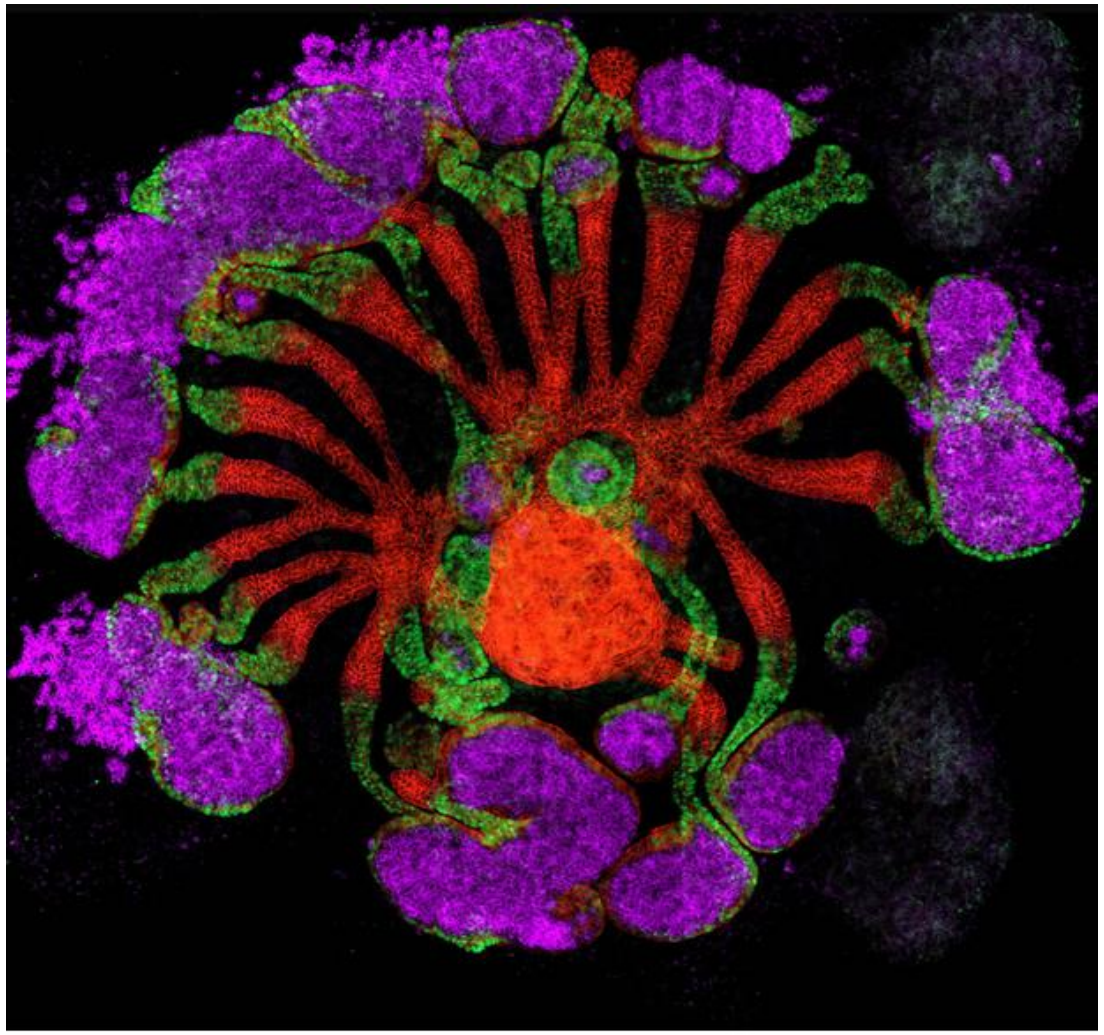


Using human stem cells, researchers grew this organoid that features glomeruli (purple), the blood-filtering subunits of a real kidney. ZHONGWEI LI

# BUT . . .



# This is not a kidney



Using human stem cells, researchers grew this organoid that features glomeruli (purple), the blood-filtering subunits of a real kidney. ZHONGWEI LI

It’s not a mature kidney; the cells are immature, progenitor cells

Plus, this is mouse tissue. The researchers couldn’t quite replicate this with human stem cells.

# What would we like to able to do?

- What if we could make replacement organs in the lab?
  - Kidneys, livers, lungs
- What if we could make implantable heart patches to compensate for scarred heart tissue?
- What if we could make pancreatic beta cells that could be implanted into patients with diabetes?
- What if we could make neurons that could be implanted into the brains of patients with Parkinson's disease or Alzheimer's disease?
- What if we could make new photoreceptors that we could transplant into patients with different kinds of blindness?
- Researchers are working in all of these areas and MORE

➢START with stem cells and make TISSUES

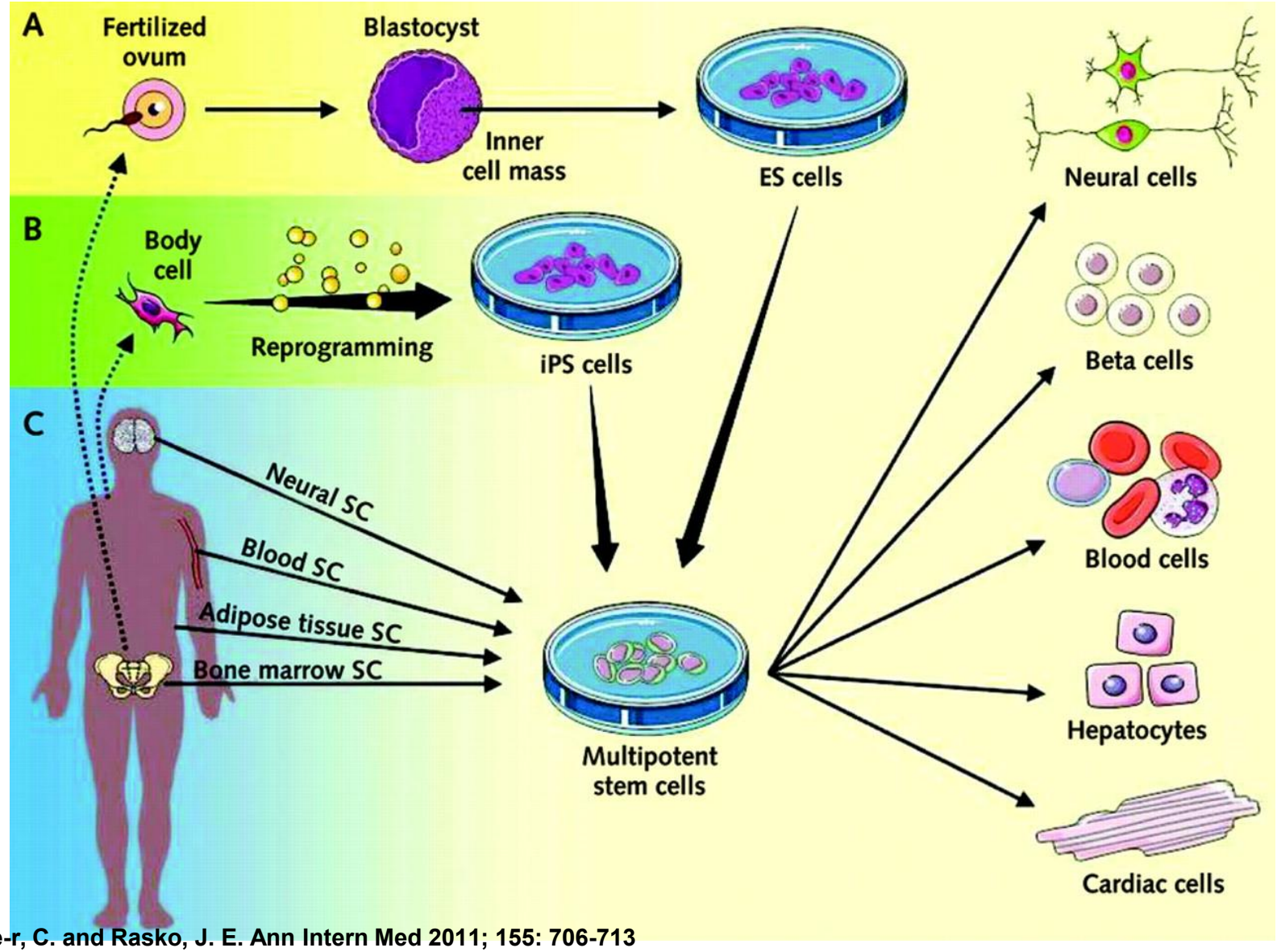
We are not there yet, but we are making progress!

# What's needed to get from stem cells to replacement organs or tissue patches or cell therapy?*

- An understanding of how to make each tissue type
  - Mature the cells – the stem cell derived tissues/organoids that we can make in the lab are often progenitors, immature
  - Incorporate all the cell types that are involved in the organ
  - Method needs to be replicable, reliable
- A clear understanding of the disease itself
  - Mechanisms of action
  - Biochemical pathways
- Be able to scale up to the number of cells and size of the tissue or organ (for transplant) that would be needed
- Figure how to safely deliver the cells and organs
- Understand what it takes for engraftment

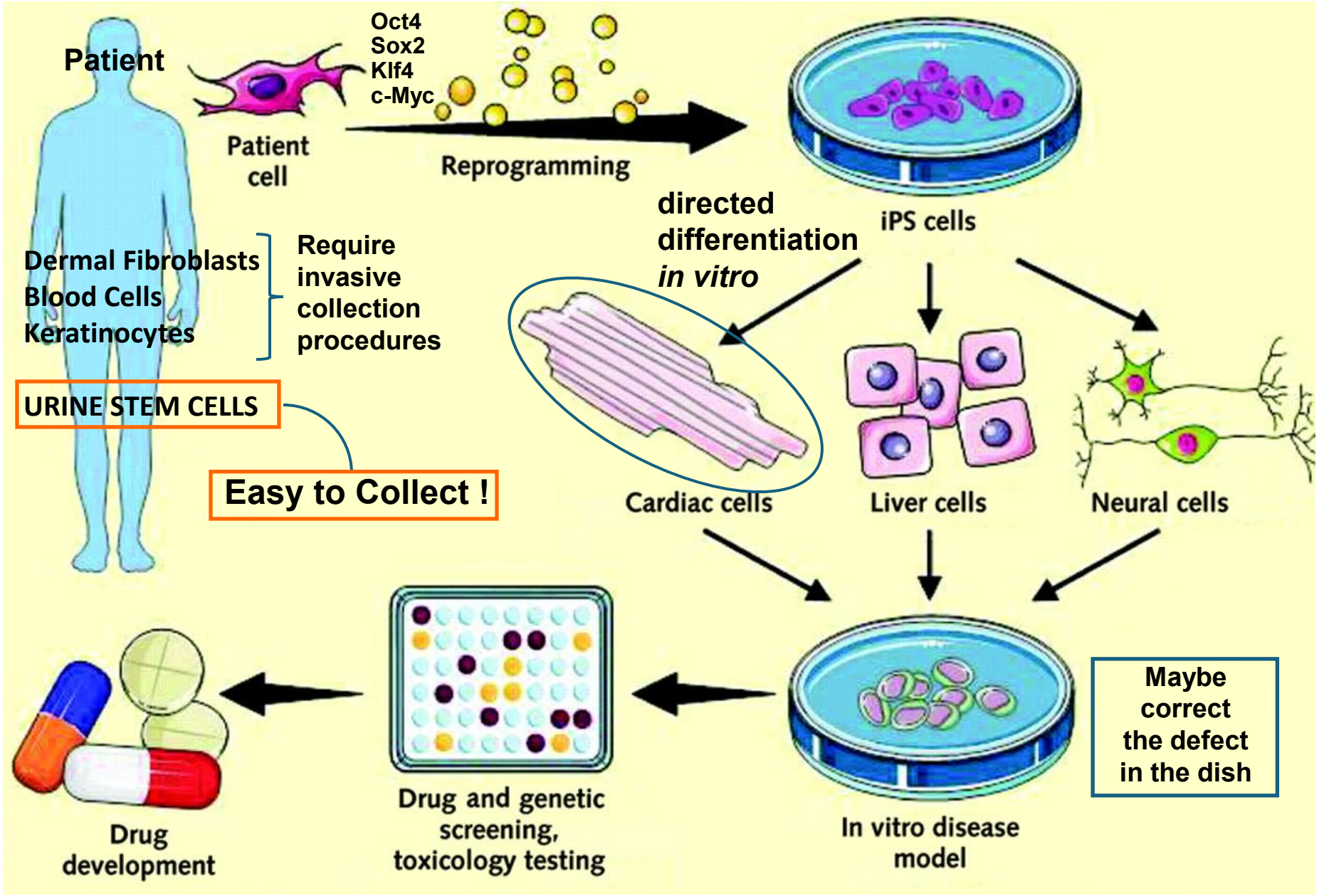
*Just a few issues! There are many!

# Stem cells and their derivatives do enable us to study diseases and move toward organ creation




Powe-r, C. and Rasko, J. E. Ann Intern Med 2011; 155: 706-713

# AND we can and are using stem cell technologies for drug development

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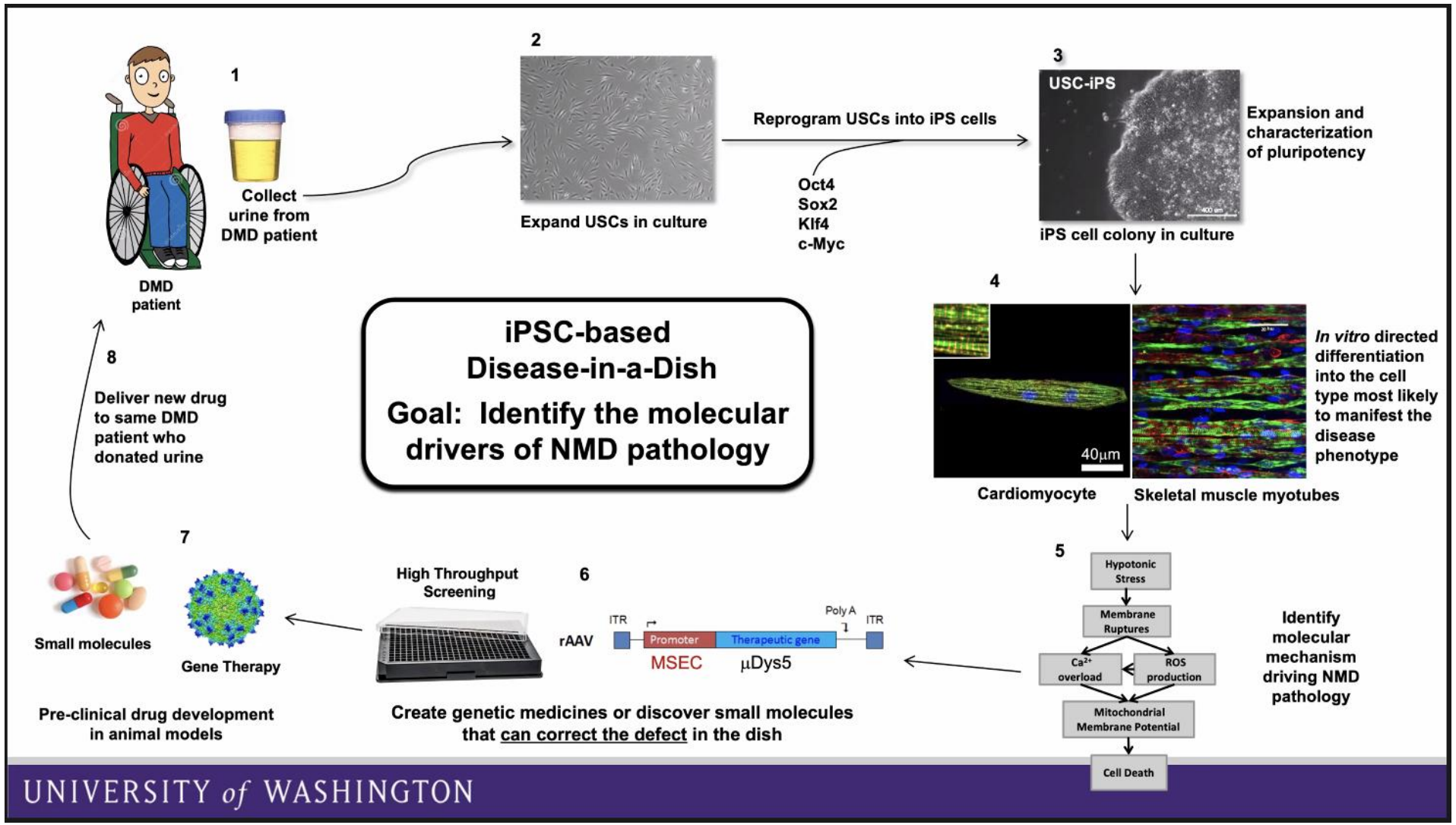
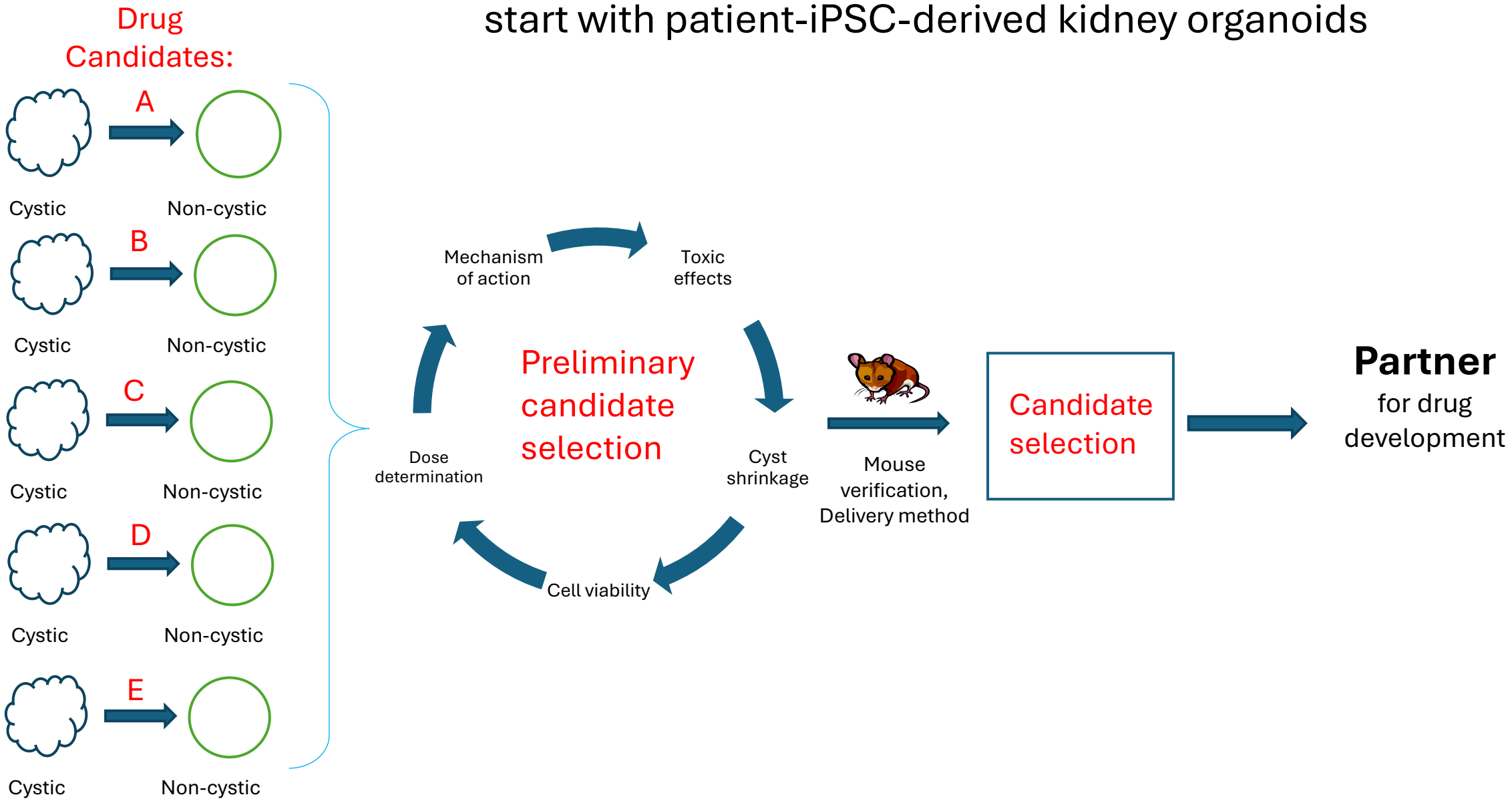
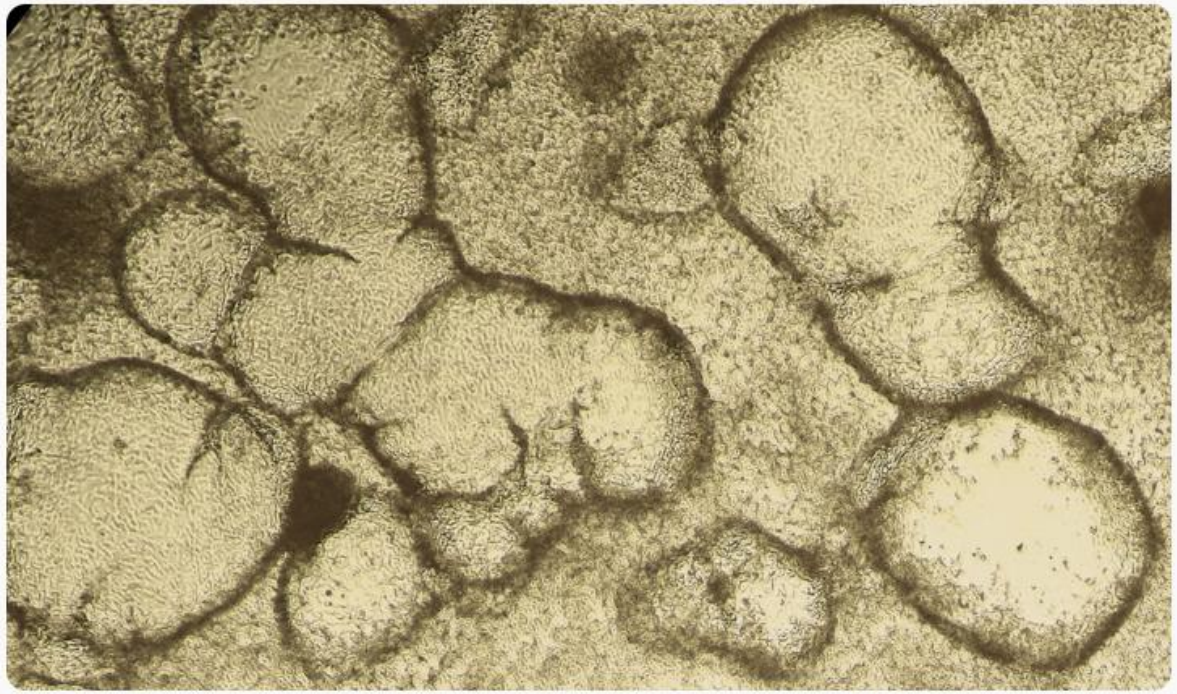



Thank you to Dr. David Mack for this slide

# Example: candidate molecule screening using kidney organoids for Polycystic Kidney Disease (PKD) start with patient-iPSC-derived kidney organoids



# Example: gut organoid

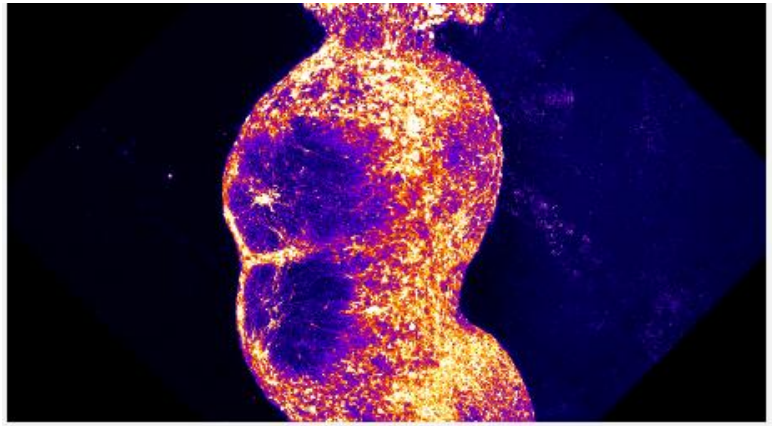


## Patient-derived Gut Organoids

Stem cell-derived gut organoids retain the same features as those of the patients. Intestinal organoids are superior to other intestinal models in drug discovery and screening. Gut organoids can be used to develop a high-throughput gut-in-a-dish platform and gut-on-a-chip microphysiological system.

## Benefits

- Retain the same features of patients.
- Recapitulate the human gut microenvironment.
- It can proliferate and propagate for several passages.
- Suitable for studying chronic gut diseases such as IBD, colorectal cancer, intestinal inflammation.
- Excellent model for drug screening, evaluating the pharmacokinetic of drugs.



April 09, 2025

## By re-creating neural pathway in dish, Stanford Medicine research may speed pain treatment

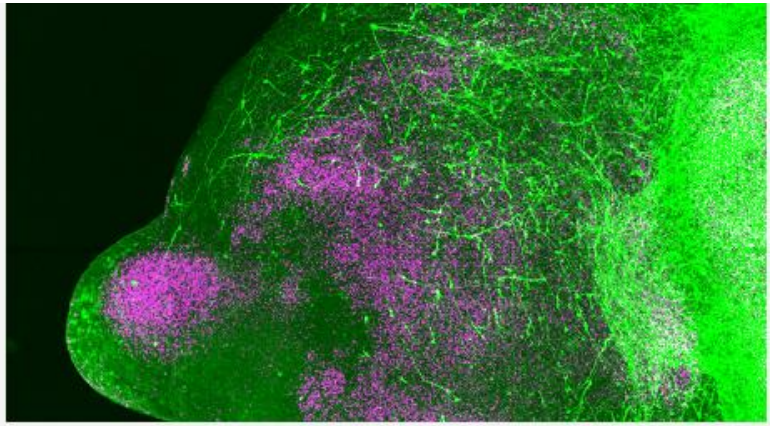
Stanford Medicine scientists have rebuilt, in laboratory glassware, the neural pathway that sends information from the body's periphery to the brain, promising to aid research on pain disorders.



April 24, 2024

## Brain organoids and assembloids are new models for elucidating, treating neurodevelopmental disorders

Stanford Medicine research on Timothy syndrome — which predisposes newborns to autism and epilepsy — may extend well beyond the rare genetic disorder to schizophrenia and other conditions.



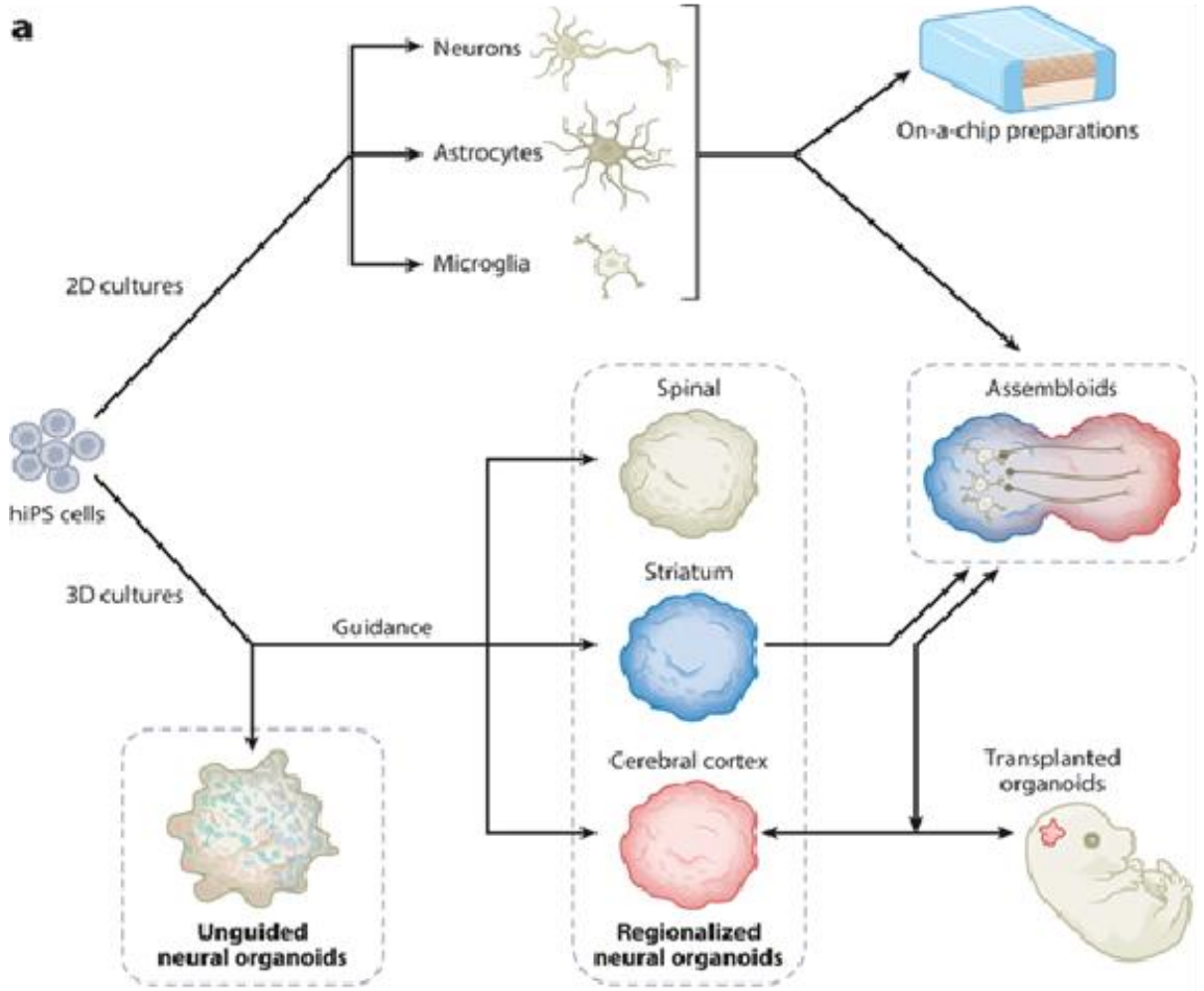
September 27, 2023

## Impact of genes linked to neurodevelopmental diseases found

Combining two cutting-edge technologies, researchers revealed the impact of a multitude of genes that are associated with neurodevelopmental disorders, including autism, but whose effects on human brain development were previously unknown.

https://med.stanford.edu/pascalab/press/releases.html

# What can we do with neural organoids?




Drug screening – e.g., for toxicities, efficacy

Different organoids communicate - Observe neural pathways

In vivo functionality – Neural organoid engrafts into rodent brain

Dr. Sergiu Pasca, Stanford

Rebecca J. Levy and Sergiu P. Paşca. From Organoids to Assembloids: Experimental Approaches to Study Human Neuropsychiatric Disorders. Annual Review of Neuroscience. 2025;48:363-379 https://doi.org/10.1146/annurev-neuro-112723-023232

## Why transplant neurons or neural organoids into the brain of a mouse?

Towards a cure for the rare disease, Timothy Syndrome (Sergiu Pasca lab, Stanford)

iPSC-derived neurons were implanted into brains of neonatal rats

**2022**

After transplantation of intact human cortical organoids derived from either healthy or Timothy syndrome subjects into different sides of neonatal rats' brains, the human tissue integrated into the rat's brain both anatomically and functionally. This revealed numerous differences in the development and behavior of transplanted "healthy" versus "Timothy syndrome" neurons.



Non-Timothy syndrome neurons grew larger and more branched in rats

**2024**

Used organoid, assembloid and rat-transplantation experiments to demonstrate a therapeutic strategy that might correct defects in Timothy syndrome neurons.



Timothy syndrome neurons in rats were smaller and less branched

https://med.stanford.edu/news/all-news/2024/04/timothy-syndrome.html

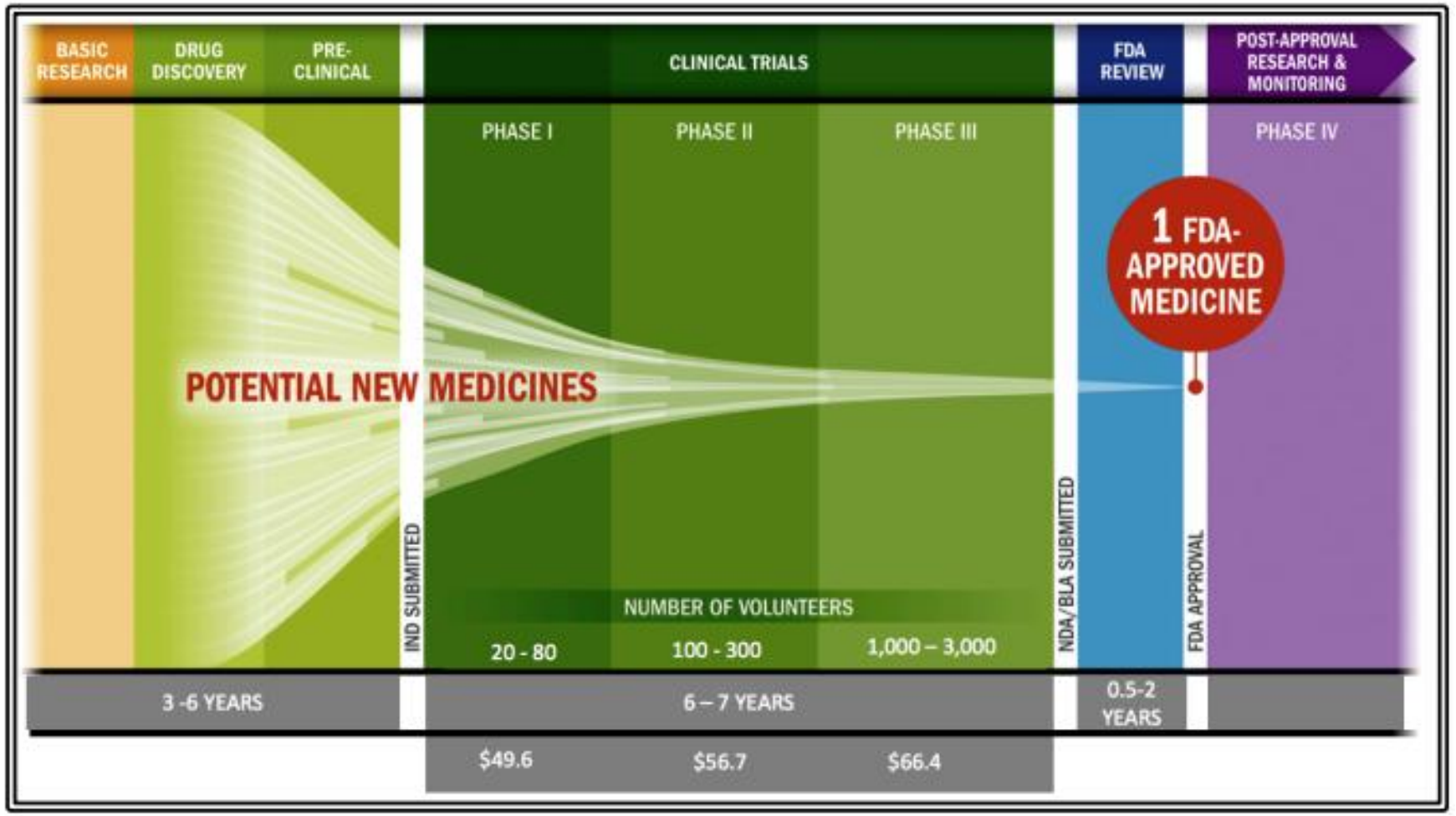
**Not only can iPSC technologies help us study and model disease, but they also may help speed and streamline drug development!**





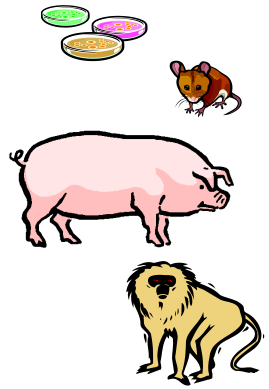


# Drug development pipeline – high rate of failure! Stem cell therapies can help with this!




Source: https://hsph.harvard.edu/wp-content/uploads/2025/10/ghd-043_polio_vaccine.pdf; Adapted from PhRMA, US Food and Drug Administration drug infographic and Di Masi JA, Grabowski HG, Hansen RW. Innovation in the pharmaceutical industry: new estimates of R&D costs. J Health Econ. 2016.

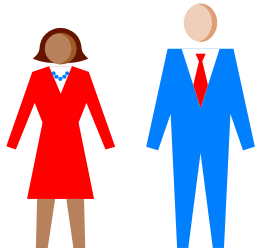
# FDA's Typical Phased Approach to Drug Development



Non-clinical
Preclinical

Sponsor submits data to FDA – asks permission to test the drug in humans

If FDA doesn't say "no"



Phase 1

Lab & animal studies:
Drug Discovery
Identify toxicities
Hone manufacturing

Safety:
Side effects (short-term),
Dosing studies (probably 20 or fewer patients)

100 (?)
Patients



Organ Toxicity

Submit all your data to FDA

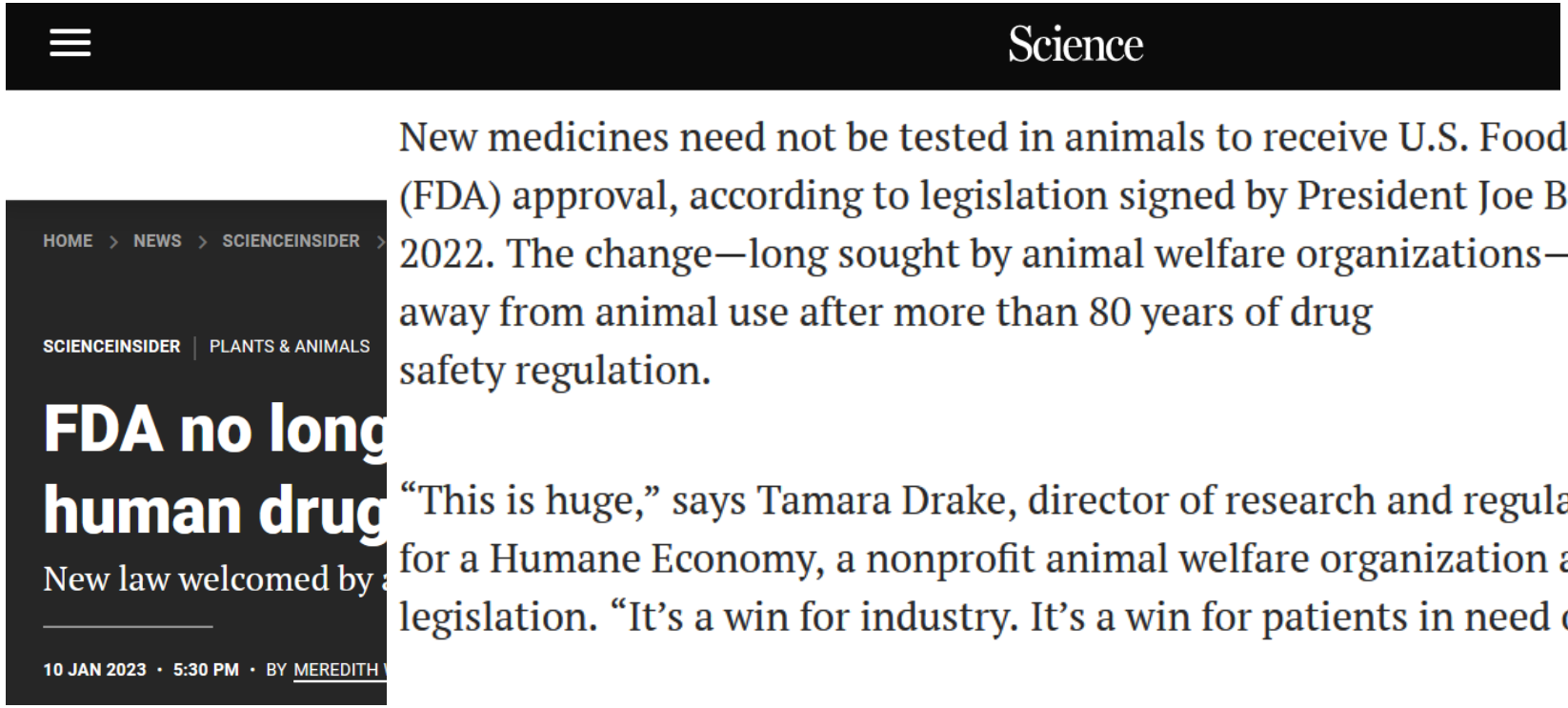
Approval!



Efficacy Failures

Patients

# Sea change in drug development




New medicines need not be tested in animals to receive U.S. Food and Drug Administration (FDA) approval, according to legislation signed by President Joe Biden in late December 2022. The change—long sought by animal welfare organizations—could signal a major shift away from animal use after more than 80 years of drug safety regulation.

"This is huge," says Tamara Drake, director of research and regulatory policy at the Center for a Humane Economy, a nonprofit animal welfare organization and key driver of the legislation. "It's a win for industry. It's a win for patients in need of cures."

In place of the 1938 stipulation that potential drugs be tested for safety and efficacy in animals, the law allows FDA to promote a drug or biologic—a larger molecule such as an antibody—to human trials after either animal or nonanimal tests. Drake's group and the nonprofit Animal Wellness Action, among others that pushed for changes, argue that in clearing drugs for human trials the agency should rely more heavily on computer modeling, "organ chips," and other nonanimal methods that have been developed over the past 10 to 15 years.

# Global Shift to Human-Relevant Drug Development

## Advancing Human-based Technologies for Next-Generation Therapeutics



**2021**

**FDA Modernization Act 2.0**
Removes mandate for animal testing in DD

**EMA Regulatory Science Strategy**
Promotes integration of NAMs in Drug Development

**2022**

**FDA Modernization Act 3.0**
Call to establish NAMs qualification process in nonclinical testing

**2024**

**FDA to Phase out Animal Testing for mAb & other Drugs**

**NIH to Prioritize Human based research**

**2025**

**UK Roadmap for Phasing out Animal Testing**

**NIH funds $87M for Standardized Organoid Modeling (SOM) Centers**

**EU European Commission Research Action NAMs**

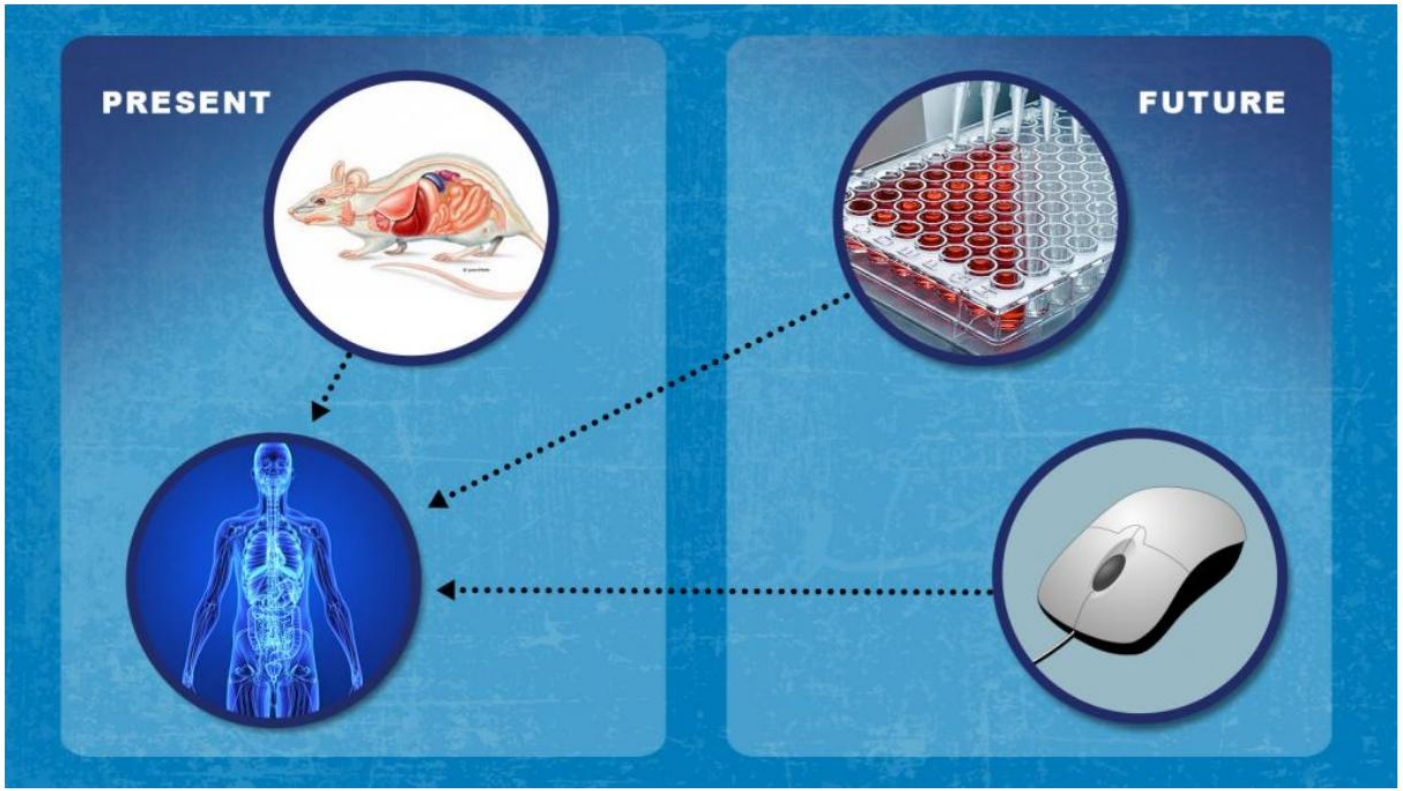
**2026**

**EU Roadmap for Phasing out Animal Testing**

Credit: Thank you to ISSCR for this slide

# FDA wants new tools to speed drug development!  And they'll help you!



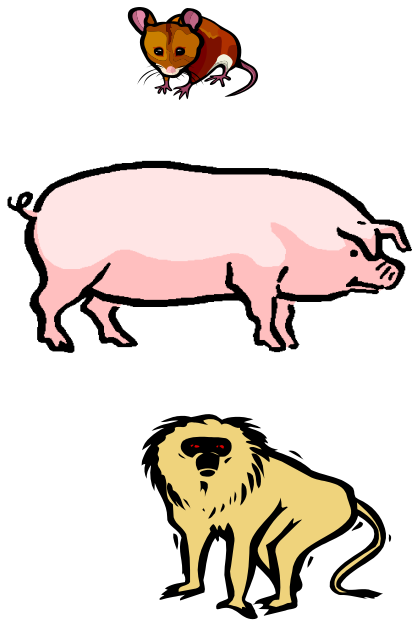

NAMs = New Alternative Methodologies = New Approach Methodologies

Can we replace animal testing with something else that might be more relevant and predictive of safety, toxicity, and efficacy?

New Approach Methodologies (NAMs) – Organoids and “organ-on-chips”



human organoids



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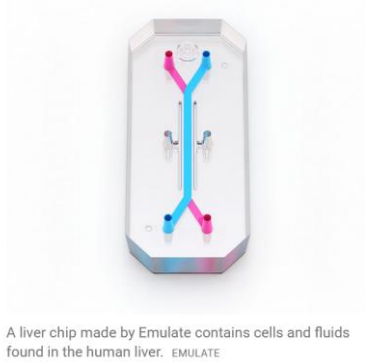


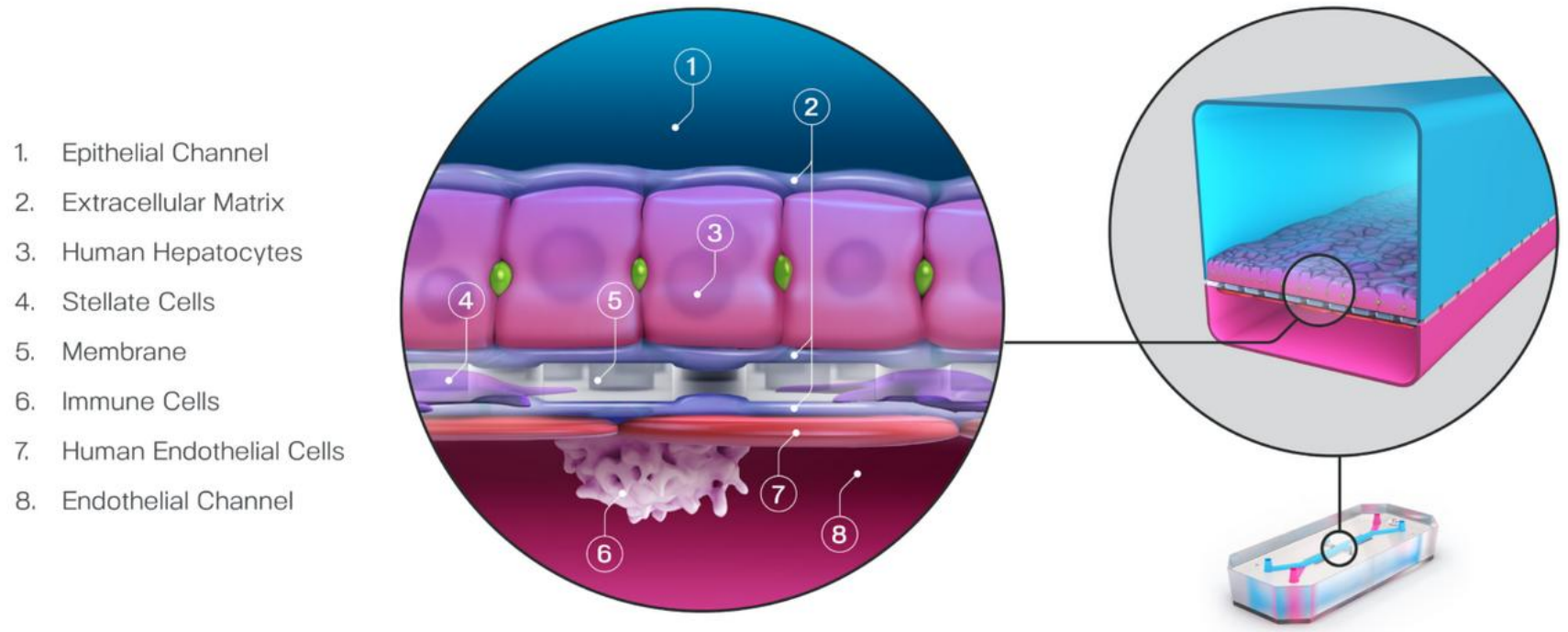
A liver chip made by Emulate contains cells and fluids found in the human liver. EMULATE

(USB-stick-sized)

Human
“organs-on-chips,” also known as
“Microphysiological systems,” or “MPS”

# Devices in Drug Development
# Organ-on-a-Chip technology

emulate PRODUCTS SERVICES APPLICATIONS RESOURCES ABOUT SUPPORT GET A QUOTE

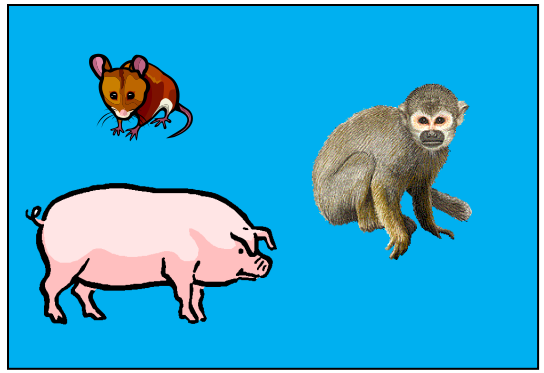



Schematic of the quad-culture Liver-Chip
Emulate human Liver-Chip (emulatebio.com)

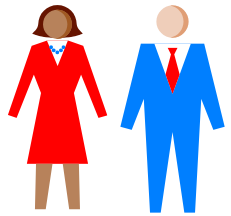
# NAMs in drug development

- Microphysiological systems use a microscale cell culture platform for in vitro modeling of functional features of a specific tissue or organ of human origin
- “Liver-chip” – identify liver toxicities – “drug-induced liver injuries” or “DILI”
- “heart-chip” – identify cardiotoxicities
- Can even link multiple organoids in a laboratory system

# Getting to the first clinical trial



IND



Phase 1

Substance of the IND + proposed clinical trial

Preclinical studies:

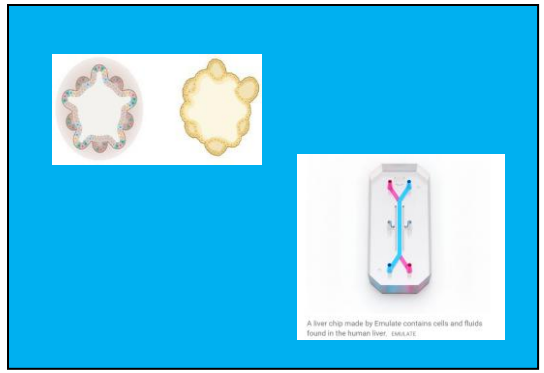
Laboratory & animal studies: Safety & Efficacy

Manufacturing studies:

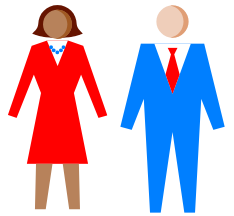
Protocol development and refinement for: cell growth, purification, testing, characterization

Safety:
Side effects (short-term),
Dosing studies (probably 20 or fewer patients)

# Getting to the first clinical trial



IND



Phase 1

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- Safety & Efficacy

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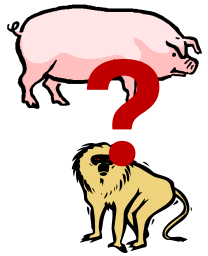
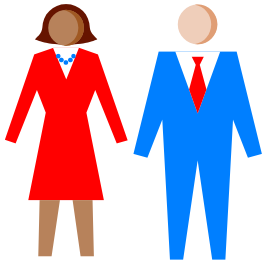
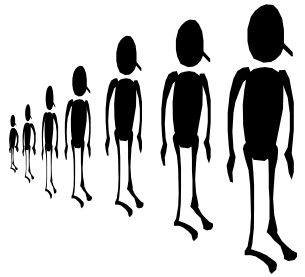
# Potential Variation on FDA's Phased Approach









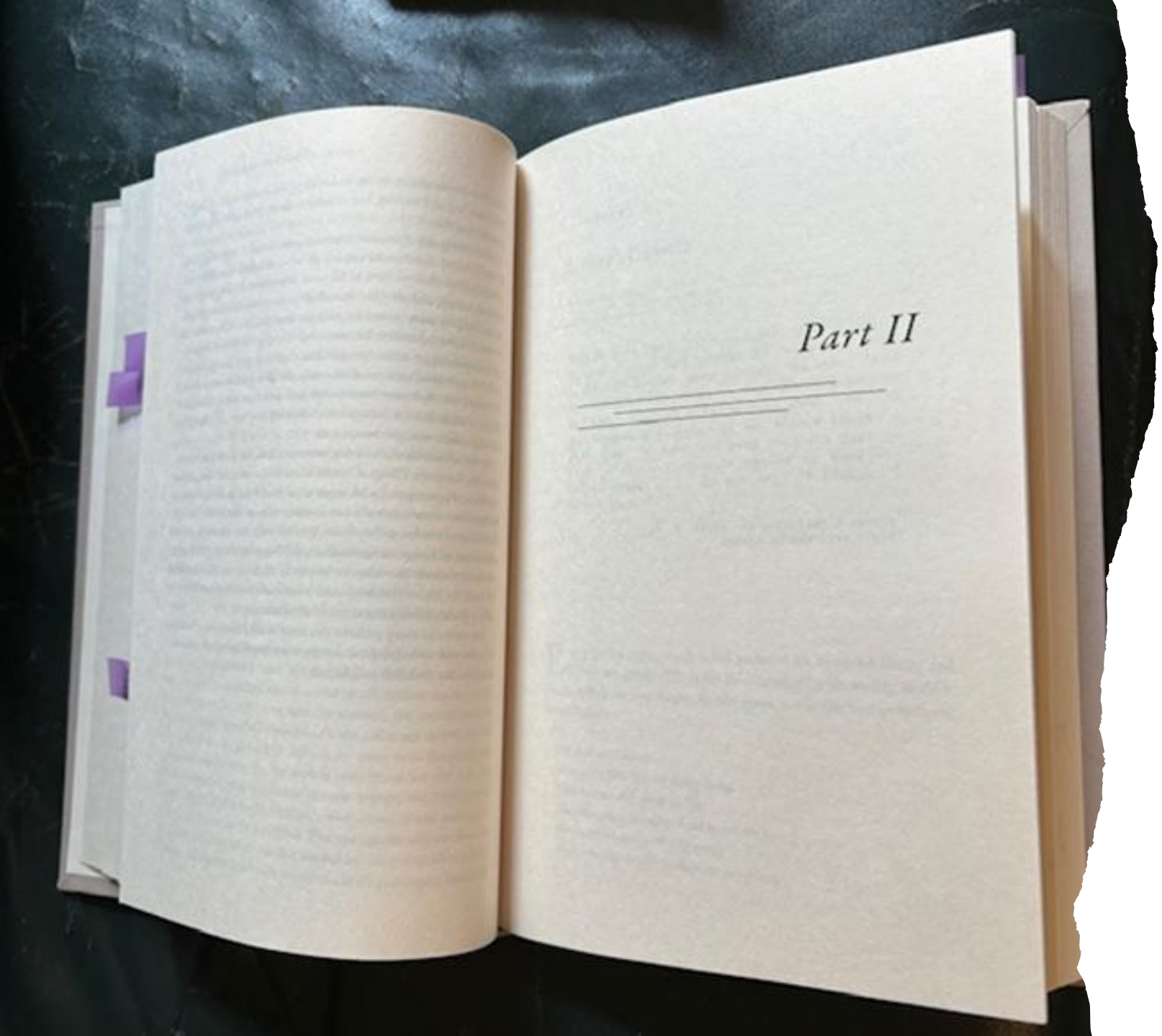
FDA Approval!



| Nonclinical | Phase 1 | Phase 2 | Phase 3 | Phase 4 |
|---|---|---|---|---|
| Lab & MPS<br>Drug Discovery<br>Identify toxicities<br>Refinement<br>Use fewer animals?<br>Get better results? | Safety:<br>Side effects (short-term)<br>Dosage<br>20-80<br>Human Volunteers | Safety (side effects) & Efficacy<br>100-300<br>Patient volunteers | Safety & Efficacy, Including Long-term<br>1000's of<br>Patient volunteers | Drug is now on the market. Continue to collect data on Safety (side Effects) |

Wait a minute! Hold the bus!
Where do the human specimens even come from?






# Sources of human specimens for stem cell and regenerative medicine research

# Who provides cells for these studies or even for transplant?

- Healthy normal donors
- Patients with various conditions

# Human Specimens for Stem Cell Research - The Mother Lode



Embryos

Human embryonic stem cell lines

Fetal cells

Cells sloughed off in the urine

Skin Fibroblasts

Whole Blood

Blood cells

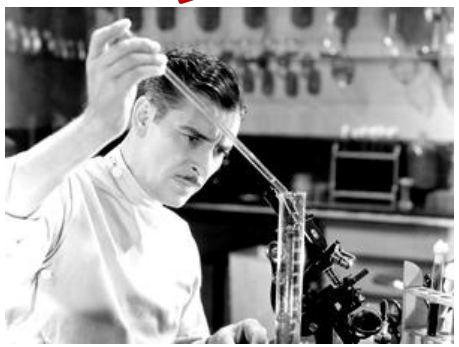
Biopsies

Bone marrow

(Etc.)

How do we get these?







# Where do specimens come from?



- From direct interactions with patients
  - Research studies
- From other investigators who interacted with patients
  - Biorepositories
- Leftover, discarded specimens after clinical procedures
  - Biopsies
  - Surgeries
- Sources of some stem cells
  - Fetal cells from abortions
  - Embryos leftover from *in vitro* fertilization procedures

# Consent matters!

## Havasupai Indian Tribe

- Understood that they were donating specimens to Arizona State University for diabetes research
- Leftover specimens remained in the freezers at ASU for years
  - Postdocs, others used the specimens for research on mental illnesses, tribal origins
- Tribal members found out and said they would never have agreed to that research
- Led to a lawsuit, monetary penalty, reclaiming of remaining specimens by tribe



New York Times

## Henrietta Lacks

- Cervical cancer cells obtained at surgery, made into the HeLa line
  - Henrietta Lacks never was asked for consent, nor informed of the cell line
- Academics, pharmaceutical companies made many discoveries, products
- Millions of dollars for researchers
- Henrietta Lacks family was not informed, has lived in poverty, even without health insurance



Henrietta Lacks: Twenty years after her death, her family learned that her cells lived on in a lab. Credit: SPL

# How should we inform specimen donors about stem cell research?

- How specific should we be?
- How specific can we be?
- What are the risks of the research to the donor?
  - Obtaining the sample may be a trivial medical procedure.
  - But what about everything else we will do with the specimen?

➢We must explain WHAT iPSCs are!

# Purpose of the Study & iPSC explanation

- We are doing a research study on neuromuscular diseases. We would like to understand the molecular effects of neuromuscular diseases on various body organs, including the heart and nerve cells as well as other tissues and organs.

- Because it can be difficult and risky to obtain tissues, especially heart and other muscle cells, as well as nerve tissues, we have developed a new technology to enable us to make copies of these mature cell types in a laboratory dish. First we obtain cells that are easy for the patient to provide. For this study we are asking patients to provide a urine specimen as a source of cells. We can remove the cells from the urine and treat them in the laboratory in a certain way in order to turn them into primitive cells called "induced pluripotent stem cells," also known as iPS cells. We allow the iPS cells to multiply and divide, creating an iPS cell line of identical cells. We can then add various molecules to a portion of the iPS cells in a laboratory dish in order to direct the iPS cells to develop into heart cells, nerve cells, skeletal muscle cells, or any other type of mature cell found in the human body. The heart and other mature cells created in this way carry the same defect as the corresponding cells that are in the patient. By studying these laboratory-derived cells, we hope to learn how the disease affects the organs of the body. We might also use the laboratory-made cells to test medicines that we think might be candidates for patient treatments in the future.

# Another way I've explained iPSCs & organoids

- Another kind of cell line that we may make from your sample is called an "induced pluripotent stem cell line" also known as an iPSC line. We make iPSC lines from blood, skin, or urine-derived cells. Using certain conditions and chemicals, we "turn back the clock" on the blood, skin, or urine-derived cells, making them into more general, primitive cells. We can then re-mature the iPSCs into specific cell types, like heart cells, nerve cells, kidney cells, muscle cells, or any other kind of cell type in the body. This allows us to study the effects of genetic changes on different types of cells, without having to get a biopsy from organs such as the heart or kidney. We can also create tiny three-dimensional structures in a laboratory dish to see how different cell types interact, similar to the way that different cell types within an organ would interact. Because the cells and 3-D structures will have been developed from a sample from you, their genes will be identical to yours. iPSCs and cells and 3-D structures created from them allow us to study the effect of an abnormal gene on the function of the cells and 3-D structures.

# What we may do with the stem cells

- One possible research use of the stem cell lines might involve changing some of their genes. Another possible research use might involve placing some stem cells into laboratory animals to learn whether and how the cells develop into a specific cell type. In addition, the stored stem cell lines might be used in the future for research related to the development of cell therapies. For example, we are learning how to make mature cells and tissues from stem cells, with the hope that someday we can develop stem cell-derived tissues that could be transplanted into patients with serious diseases. These are just a few common examples of what might happen to the specimen-derived stem cells. But there are many other future possible research uses that are simply unknown at this time. A committee at the University of Washington will review the research studies to ensure that they are scientifically sound and meet ethical standards.

# Genomics – this WILL be done!*
# And genomics data will be shared!**

- We will isolate DNA (genetic material) from the cell lines and perform gene sequencing. For these studies, a subset or even your whole genome may be sequenced and analyzed and used to study your disease and other disorders. In whole genome studies, all or most of the genes in the cells are sequenced and analyzed. This allows researchers to understand the cells, and study links to many diseases and conditions.

- We will provide the gene sequence data and medical information about you, such as evidence of heart disease, and the history of your disease, to a national database at the National Institutes of Health (NIH), the primary agency in the US that funds biomedical research. The NIH has developed restricted data banks for the collection and sharing of the results of genetic studies. The NIH data bank stores the genetic information and makes it available to qualified researchers from the government, academia, or commercial institutions who are also conducting research on any number of topics. Any genetic information that we share will be sent with only a code number attached. Your name and other identifiable information will never be shared. You will not receive the results from any of these additional studies that are performed using your genetic data. You will not be able to withdraw your information after it has been submitted to the NIH data banks.

*Be sure to use the most current language required by your IRB

**Even if you are not NIH-funded, publications often required this

# Information we may need about you

- We may obtain some medical information from your medical records and/or by asking you to fill out a brief health questionnaire.  For study purposes we are interested in your gender, age, diagnosis (if known), and symptoms (particularly evidence of heart problems, muscle weakness, neuromuscular disease, and any molecular diagnostic information [such as whether you have a known, specific mutation]).

# Stem cell research:  Key points for the donor to know

- Things to know about induced pluripotent stem cell lines from the donor's samples
  - The cell lines may live indefinitely
  - We may mature them into other types of cell types
  - We may put the cells, or derivatives made from them, into animals
  - We may share the cell lines with other researchers
- Withdrawal
  - If we share your sample we cannot get it back
- We will sequence the DNA of the cell lines
  - The DNA sequence of the cell lines and derivative cell lines will match your DNA sequence
- Sharing of sequence data
  - Researchers may share sequence data with other researchers. Sequences may be uploaded to research databases and/or in publications.
- Commercialization
  - "Your specimens may lead to the development of commercial products. There are no plans to provide compensation to you."

# RISKS, STRESS, OR DISCOMFORT

- Risks of urine collection:  Providing a urine specimen may be embarrassing.

- Risk of blood draw:  You may feel faint, lightheaded, or nauseated.  There will likely be temporary pain at the location in which the needle breaks the skin.  Rarely, an infection occurs.

- Risk of production of induced pluripotent stem cells:  iPSCs are an immortalized cell line and their lifespan is indefinite.  Some people are uncomfortable with this concept.

- There is a very small risk of loss of confidentiality.  Your specimen and the iPSC line we derive from it will be coded.  Your identity will be kept secret.

- As mentioned above, the genetic information will be sent with only a code number attached. There are many safeguards in place to protect your information while it is stored in data repositories and used for research.

# We sure to be clear with the donor

- iPSCs are inherently identifiable, not “de-identified,” because DNA can be linked back to a specific individual
  - Relatives may also be identified
- Genetic analysis, such as whole genome sequencing, will reveal disease risk – will the donor be re-informed?
  - Disease risk may be relevant to relatives, as well
- Treatments for others may be derived from the donation
  - “Allogeneic” cell therapy treatments
  - These may be commercializable, and the developer will make money
    - The specimen donor will not receive financial benefits
- iPSC lines may be shared and used by other investigators for a wide range of research studies that cannot be completely predicted
  - Including research involving putting human cells into animals
- Artificial gametes – sperm or oocytes (eggs) - can be derived from iPSCs

# WTC-11 Consent form

IRB NUMBER: 10-02521
IRB APPROVAL DATE: 10/11/2010
IRB EXPIRATION DATE: 10/10/2011

**UNIVERSITY OF CALIFORNIA, SAN FRANCISCO**
**CONSENT TO PARTICIPATE IN A RESEARCH STUDY**

**Study Title: Induced Pluripotent Stem Cells for Genetic Research**

This is a medical research study. Your study doctors Bruce Conklin MD, Deepak Srivastava MD, and Melvin Scheinman MD from the UCSF Departments of Medicine and Pediatrics will explain this study to you.

Medical research studies include only people who choose to take part. Take your time to make your decision about participating. You may discuss your decision with your family and friends and with your health care team. If you have any questions, you may ask your study doctor.

You are asked to take part in this study because you carry a gene mutation that may cause a heart disease or an increased risk of a heart disease or because you are a healthy relative of a person that carries a gene mutation for a heart disease.

**Why is this study being done?**

The purpose of this study is to learn more about heart development and disease. A new technique has been discovered which enables skin cells to be changed to any cell type of the body, including heart cells. This technique has been referred to as "stem cells from skin cells", or "induced pluripotent cells". This research focuses on studying heart cells that come from the skin of people with a heart disease or with an increased risk of getting heart disease and from their healthy relatives.

**Tissue Banking Permission (initial one line)**

_X_ I give my permission to have my skin tissue cells frozen and stored for future research studies.

_____ I do not give my permission to have my skin tissue cells frozen and stored for future research studies.

**Future Contact Permission (initial one line).**

**_X** I give permission for researchers may contact me about future medical studies and/or follow-up studies.

_____ I do not want to be contacted for future medical studies and/or follow-up studies.

**If you wish to be in this study, please sign below:**

_____ ____WTC Signed here
Date Participant's Signature for Consent

_____ _____
Participant's name printed Date of birth

**ADDENDUM TO UNIVERSITY OF CALIFORNIA, SAN FRANCISCO CONSENT TO PARTICIPATE IN A RESEARCH STUDY – GENETIC DATA SHARING**

**Study Title: Induced Pluripotent Stem Cells from Genetic Research in Adults**

Previously, you provided your consent for a skin biopsy to be collected and used to generate induced pluripotent stem cells and study a gene mutation associated with heart disease. At the time you consented to this research study, you also gave your permission for other researchers to use your coded, de-identified cells and data for unspecified future research.

Some researchers who are studying your cells would like to collect and share genetic data broadly with other researchers.

It may be possible to re-identify de-identified genetic data, even if access to data is controlled and data security standards are maintained. If you choose to share your genetic data, your confidentiality cannot be guaranteed. There is a possibility that re-identified genetic data could be used to discriminate against you or your family members. There may be unknown future risks as well.

For this reason, we are seeking your consent regarding how researchers can share your genetic data. Please select one of the options below describing how you consent for your genetic data to be shared:

☐ My genetic data may be shared in an open and publicly accessible repository or in a controlled-access data repository that provides access to other researchers.

☐ My genetic data may be shared in a controlled-access data repository that provides access to other researchers, but is not accessible to the general public.

☐ I do not consent to the sharing of my genetic data in an open or controlled-access data repository.

# Typical consent for surgery for clinical purposes

**CONSENT FOR ADMINISTRATION OF ANESTHESIA AND FOR PERFORMANCE OF OPERATIONS OR OTHER PROCEDURES**

**PATIENT**: I ☐ DO DO NOT wish the services of a translator.

1. **DIAGNOSIS (medical terminology):** _____

   **DIAGNOSIS (common terminology):** _____

2. **OPERATION OR PROCEDURE (medical terminology):** _____

   OPERATION OR PROCEDURE (common terminology): _____

   Benefits of the operation or procedure are: _____

   Common risks and complications of the procedure are: _____

   There may be unknown complications that have not been identified to date.

   Possible alternatives to the procedure and/or anesthesia are: _____

3. **PATIENT CONSENT** *(Line through any parts that are refused by the patient.)*

   A. *I hereby give my consent and authorize_____**(Name of Provider)** of ANMC to perform the above named operation or other procedure. All my questions have been answered to my satisfaction. I acknowledge that no promises or guarantees have been made to me as to the results that may be obtained.*

   B. *I consent to the performance of the operation or procedure and any emergent operations or procedures that are found to be necessary in the best judgment of the medical staff during the planned operation or procedure.*

   C. *I consent to the administration of anesthesia that is considered necessary in the judgment of the medical staff.*

   D. *I consent to the disposal by ANMC of any body tissues or parts which may be necessary to remove in accordance with applicable laws and regulations. I authorize the facility to retain, preserve and use for scientific or teaching proposes any tissue or specimens taken from my body.*

   E. *I consent to the admittance or participation of observers, vendors or supervised students in accordance with the policies and procedures of the ANMC.*

   F. *I understand that photographs and movies may be taken of this operation or procedure which may be viewed by personnel undergoing training at this or other facilities. I consent to the viewing of such movies and photographs for scientific or educational purposes, provided my identity is not revealed by the pictures or written information accompanying them.*

4. **COUNSELING PROVIDER**: I have counseled this patient as to the nature of his/her condition, the proposed procedure, the benefits, the risks, alternatives and expected results.

   **Signature of Provider Securing Consent:**_____

5. **PATIENT**: I understand the diagnosis, the proposed operation or procedure, the benefits, the risks and the alternatives, and the expected results.

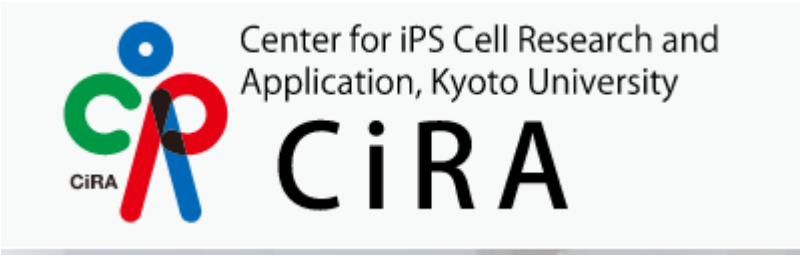
**Witness's Signature:** _____ **Patient's Signature**: _____ **Date**: _____ **Time**: ____ **AM** ☐ **PM** ☐

# Broad consent

D. *I consent to the disposal by ANMC of any body tissues or parts which may be necessary to remove in accordance with applicable laws and regulations. I authorize the facility to retain, preserve and use for scientific or teaching proposes any tissue or specimens taken from my body.*

Is this enough to tell the patient?

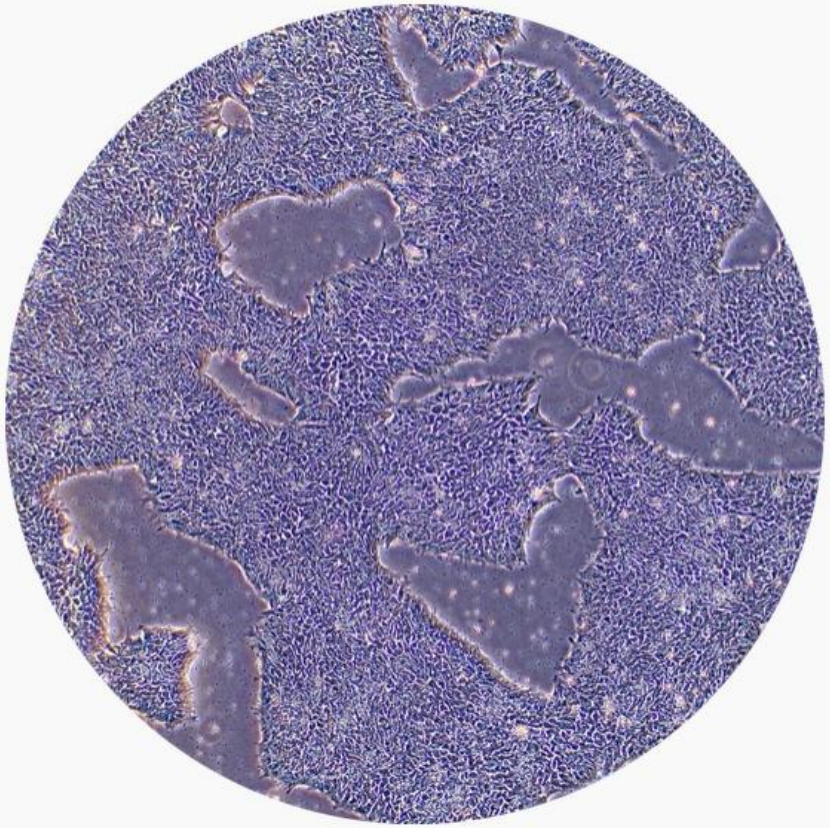
# iPSC biobank as a supply of cells for therapeutic purposes: Japan




- Japanese population is fairly homogeneous
  - Limited HLA types (human leukocyte antigen)
- Project: Collected cells from healthy donors with homozygous HLA
- Developed homozygous iPSC lines and created a biobank
- These cell lines are expected to lower the risk of immune rejection in transplants after donor-patient matching
- Characterized cell lines using manufacturing standards for clinical products
- Can supply cell lines quickly to medical care institutions and research institutions in Japan and overseas when required
- Estimates that these iPSC cell stocks could match 40% of the Japanese population

https://www.cira-foundation.or.jp/e/research-institution/ips-stock-project/index.html

# iPSC



## Human Induced Pluripotent Stem Cells (HiPSCs)

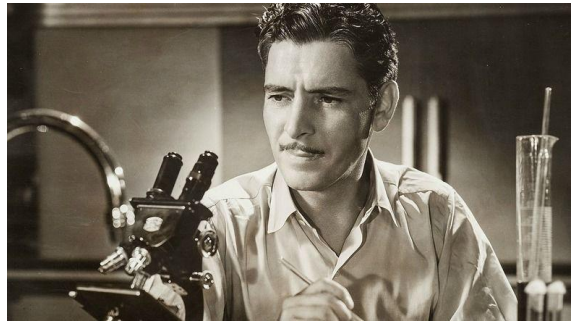
Human iPSC-derived pluripotent stem cells provide a renewable and genetically defined source of human cells for basic research, disease modeling, drug discovery, and regenerative medicine applications. These cells are generated under standardized conditions and retain the full capacity to differentiate into cell types of all three germ layers.

## Benefits

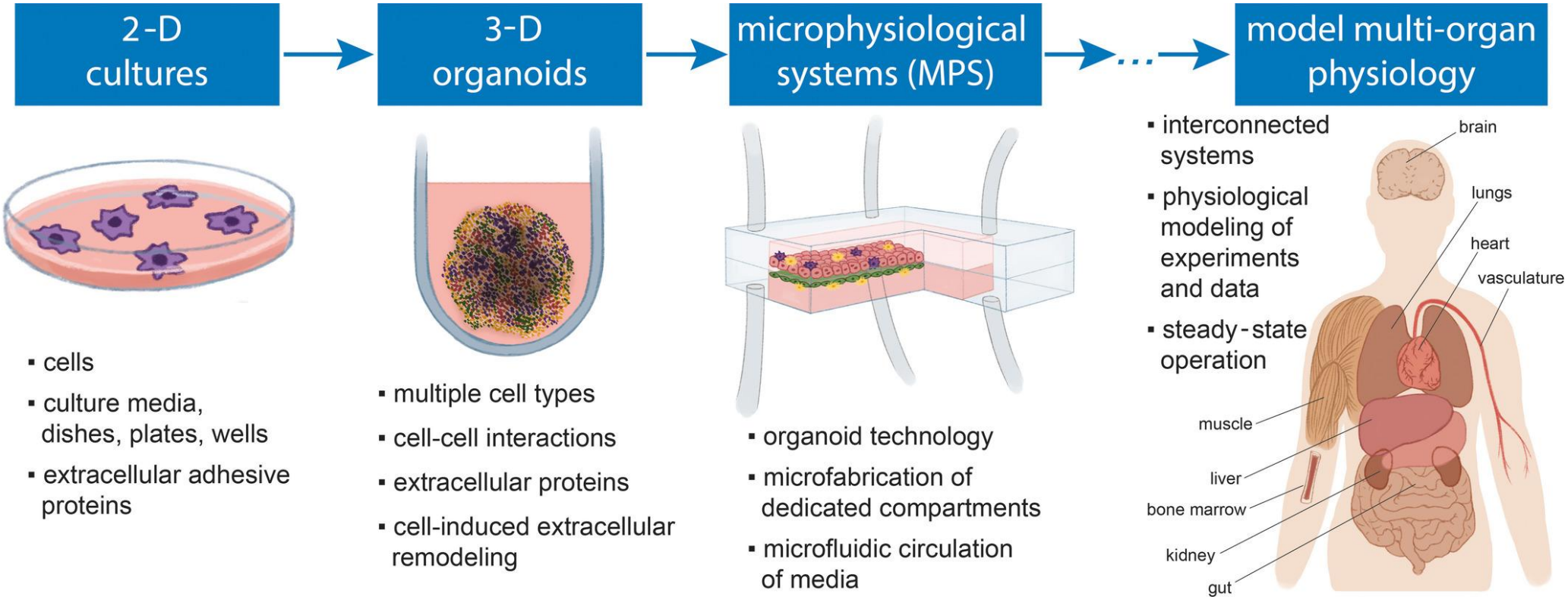
- ✔ High-quality, well-characterized pluripotent stem cells
- ✔ Robust expansion capacity for large-scale experiments
- ✔ Retain full differentiation potential into ectoderm, mesoderm, and endoderm lineages
- ✔ Suitable for genome editing, disease modeling, and cell-based therapy research

# Lessons learned – Even if you work at the lab bench, and not with patients, ASK:

- What is this cell line that I'm working with?
- Where did it come from?
- Who is it from?
- Was informed consent given for use of the cell line?
  - Can I see it?
- What did the donor give consent to?
  - Types of research
  - Sequencing
  - Development of commercial products
- What am I allowed to do with this cell line?



# If you know experiments like this may be done with the specimen donation, the donor should be informed.




Liver Microphysiological Systems for Predicting and Evaluating Drug Effects
**Clin Pharma and Therapeutics, Volume: 106, Issue: 1, Pages: 139-147, First published: 16 April 2019, DOI: (10.1002/cpt.1458)**


I'm growing mini human brains from stem cells.
Why? There's no shortage of mini human brains.
CartoonStock.com

# QUESTIONS?

ITHS | Institute of Translational Health Sciences
ACCELERATING RESEARCH. IMPROVING HEALTH.

NED 2026