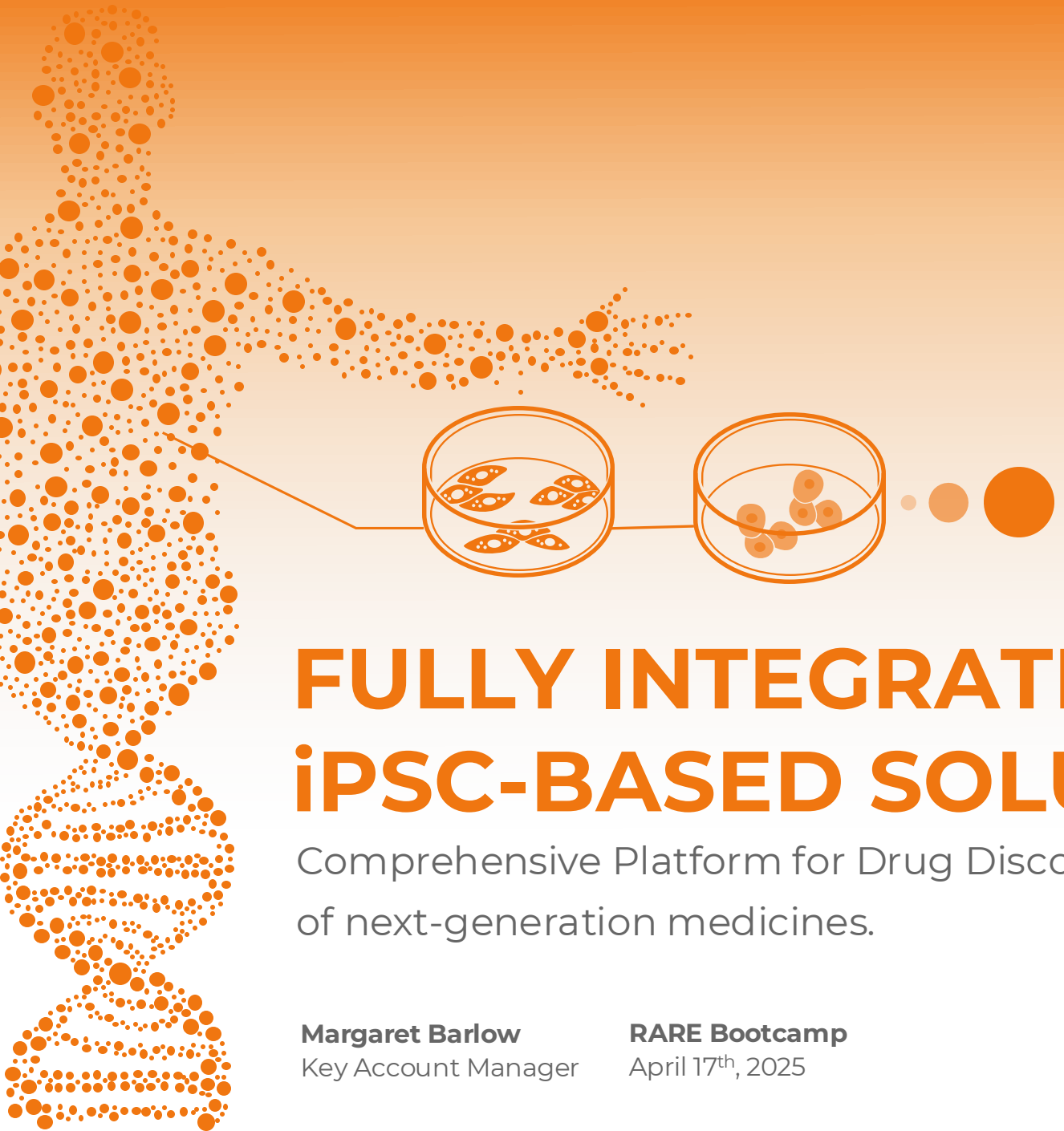
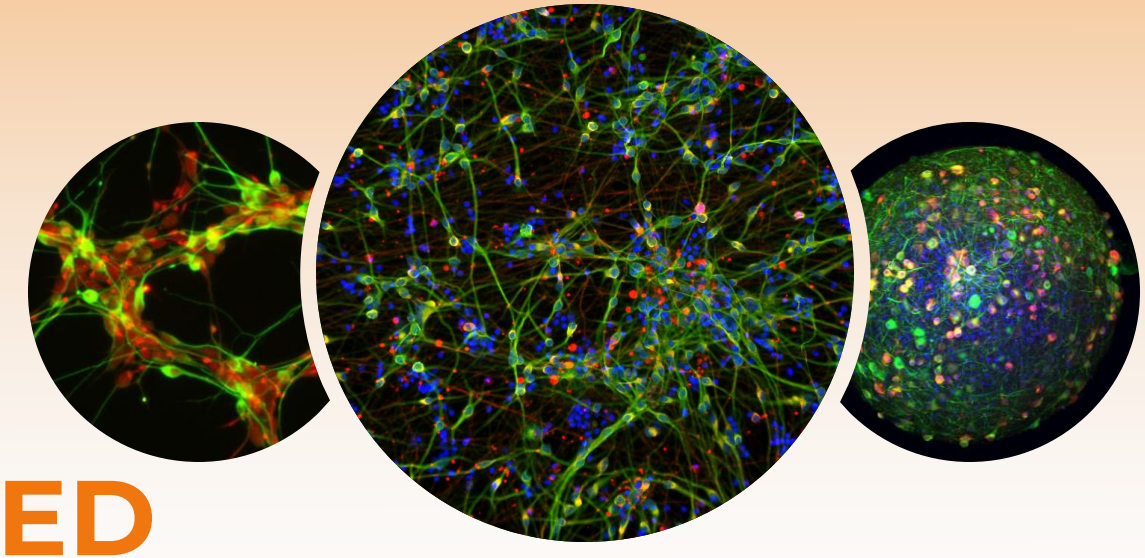
Better Human Models, **Better Discoveries.**





FULLY INTEGRATED iPSC-BASED SOLUTIONS

Comprehensive Platform for Drug Discovery & R&D of next-generation medicines.

Margaret Barlow
Key Account Manager

RARE Bootcamp
April 17th, 2025

iXCells BIOTECHNOLOGIES

WHO WE ARE

Introducing iXCells Biotechnologies

We **accelerate drug discovery** with **accessible human cell models** and **patient iPSC-derived cells**. By bridging the translational gap, we help researchers bring **new therapies to market faster** and more efficiently.

Headquarters
San Diego, CA

Established
2014

2015 **Pioneers in iPSC-Derived Cells** Commercialization

300+ **Human iPSC-Derived Cells Lots** with 100+ donors and 27+ cell types

475+ **Projects and Products Delivered** to **top 30 global BioPharma** companies

100+ **iPSC Lines and Rare Disease Projects**, with 50+ patient foundations supported; Corporate Sponsor of N=1 Collaborative (since 2023)

iXCells BIOTECHNOLOGIES

OUR MISSION

Our Mission is our Purpose

Better Human Models.

Healthier Futures.

We empower life sciences researchers and patient foundations to **advance personalized therapies** and **accelerate the discovery** of **better medicines** for a **healthier future.**

Better Medicines.

WHY US

A Better, Targeted Approach to Drug Development

Better Disease Models
Biologically accurate models that reflect patient-specific disease conditions

Better Targets and Biomarkers
Identifies disease-specific targets and biomarkers by condition and patient subgroup

Better drug candidates
Improves preclinical validation and highlights drug repurposing opportunities

Better efficacy & toxicity testing
Human-relevant models improve predictability of therapeutic effects and safety.

Better Medicines
Patient-specific characteristics identified through cellular and molecular analysis of their biological materials

Better Efficacy and Toxicology Testing
Human *in vitro* models closely mimic the human in vivo environment

Patient-derived cells provide better disease models, aligning drug development closely with human disease biology

WHAT WE DO

Pioneering Cell Technology

We specialize in iPSC generation, gene editing, and cell differentiation. As pioneers in iPSC-derived motor neurons, we support drug discovery with a strong focus on rare and neurological diseases, driving innovation in personalized medicine.

PRODUCTS

- Human iPSC lines and iPSC-derived cell types from both healthy and patient sources
- Human & animal primary cell types
- Companion products: Cell culture media & reagents

CUSTOM SERVICES & CELL-BASED ASSAYS

- iPSC Generation, Genome Editing, iPSC Differentiation, Custom Primary Cell Isolations
- Development of Disease Models: “Disease in a Dish”
- Immunoassays, Neuronal Assays, etc.

iPSCore CORE.Reprogramming CORE.Edit CORE.Differentiation

INTRODUCING iPSCore PLATFORM

One Platform, Every Solution

iXCells BIOTECHNOLOGIES

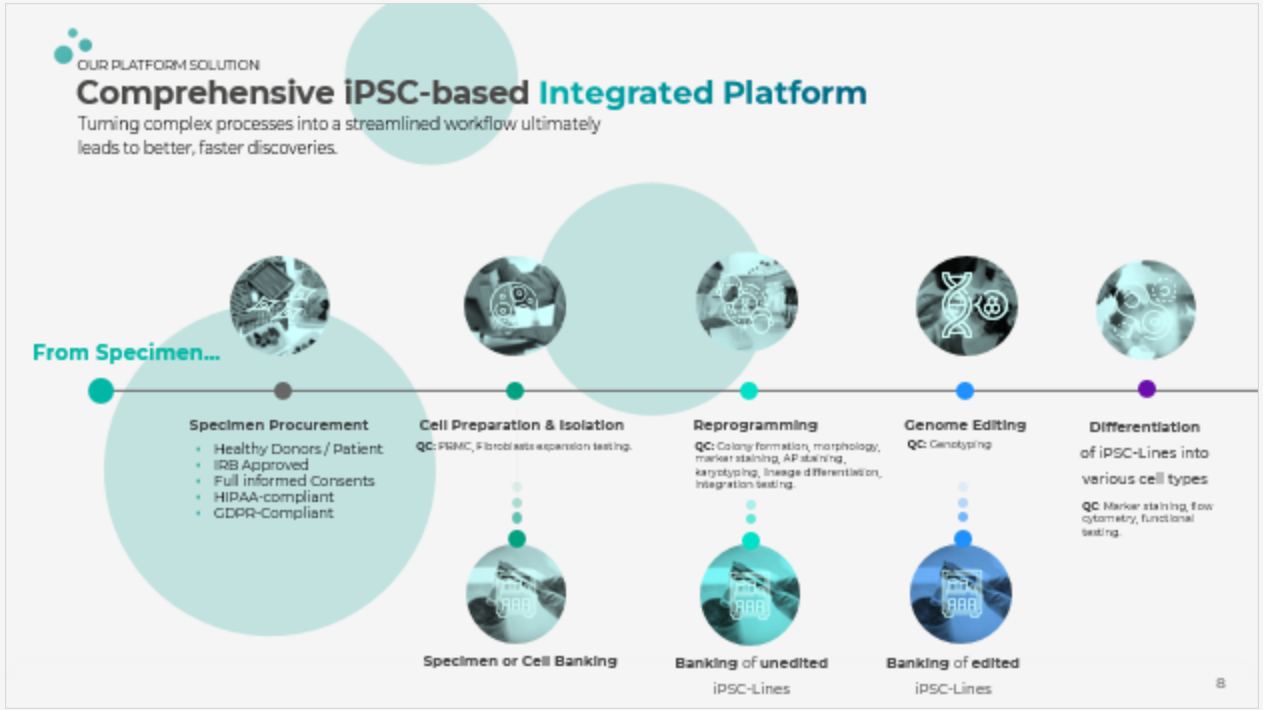
iPSCore

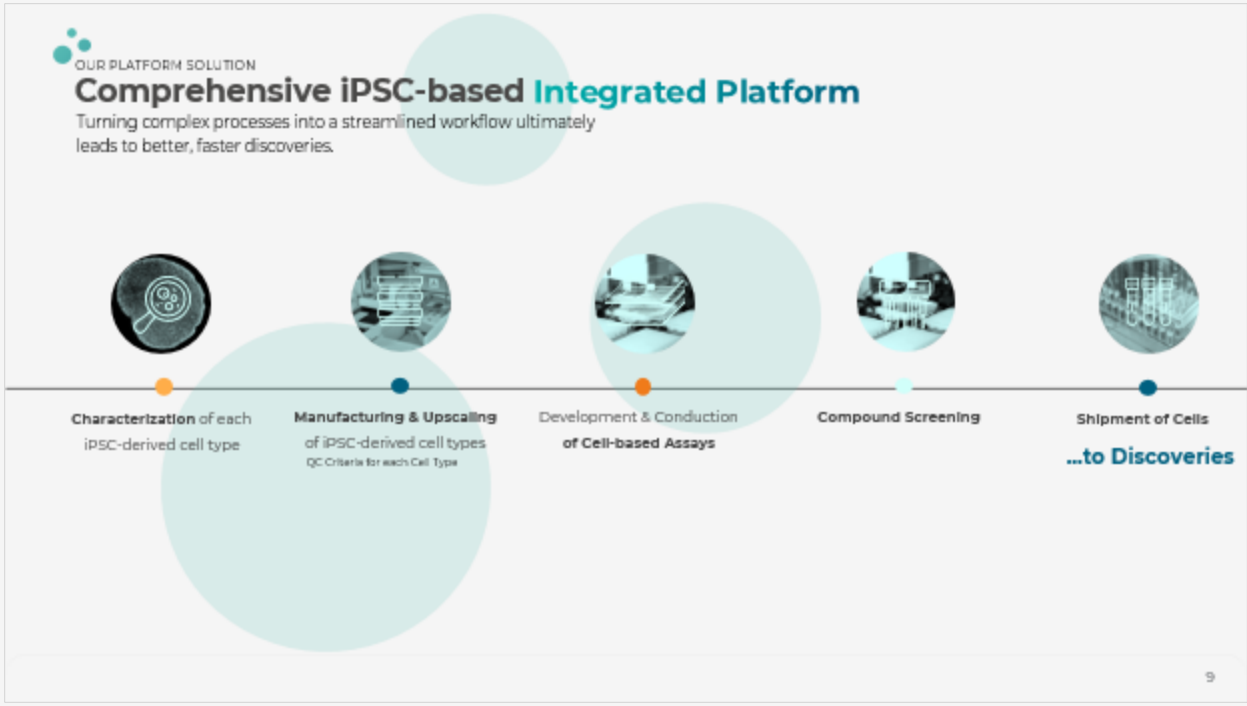
Better Human Models, Better Discoveries.

OUR PLATFORM SOLUTION

Comprehensive iPSC-based Integrated Platform

Turning complex processes into a streamlined workflow ultimately leads to better, faster discoveries.





*iPSCore carves the path for a **paradigm shift in drug discovery** through its integrated modular approach*

OUR PLATFORM SOLUTION

Comprehensive iPSC-based Integrated Platform

Turning complex processes into a streamlined workflow ultimately leads to better, faster discoveries.

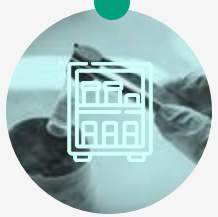
From Specimen...

Specimen Procurement

- Healthy Donors / Patient
- IRB Approved
- Full informed Consents
- HIPAA-compliant
- GDPR-Compliant

Cell Preparation & Isolation

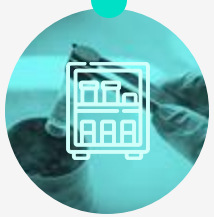
QC: PBMC, Fibroblasts expansion testing.



Specimen or Cell Banking

Reprogramming

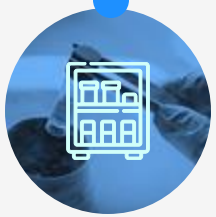
QC: Colony formation, morphology, marker staining, AP staining, karyotyping, lineage differentiation, integration testing.



Banking of **unedited** iPSC-Lines

Genome Editing

QC: Genotyping



Banking of **edited** iPSC-Lines

Differentiation

of iPSC-Lines into various cell types

QC: Marker staining, flow cytometry, functional testing.

OUR PLATFORM SOLUTION

Comprehensive iPSC-based Integrated Platform

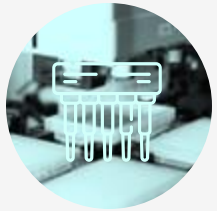
Turning complex processes into a streamlined workflow ultimately leads to better, faster discoveries.

Characterization of each iPSC-derived cell type

Manufacturing & Upscaling of iPSC-derived cell types

QC Criteria for each Cell Type

Development & Conduction **of Cell-based Assays**



Compound Screening

Shipment of Cells

...to Discoveries

UNDERSERVED INDICATIONS

Rare Diseases: Turning Complexity into Breakthroughs

50%
of the people affected by rare diseases are **children.**

30
million people suffer from rare disease in the US & 350 million **worldwide.**

11,000+
rare disease & disorders have been identified.

>80%
are caused by **faulty genes,** highlighting the need for treatment over prevention.

#Patient Foundations & Advocacies Served
50+

#Rare Disease Related Projects
100+

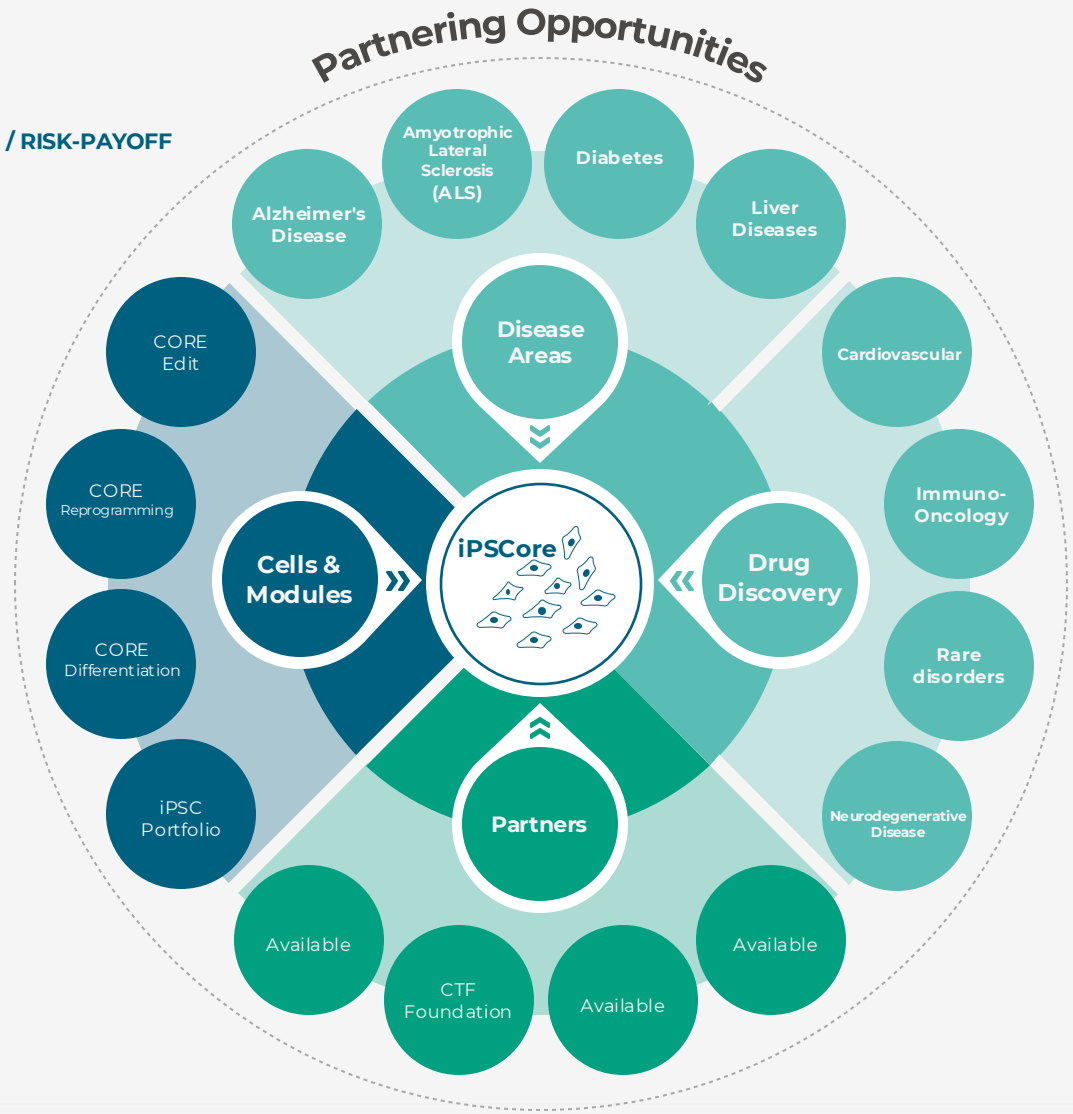
#Patient iPSC Lines Generated
30+

#ASOs Screened
3000+

#Repurposing Drug Screened
1000+

PARTNERSHIP OPPORTUNITIES

Partnering for Better Medicines



HUMAN

- Animal-Free, Human-Centric Models
- High relevance to human disease at screening stage

APPLICATIONS

- Drug screening / Phenotypic screening
- Biomarker discovery
- Test Drug Mechanism of Action (MoA)
- *in vitro* toxicology

RELEVANCE

- Disease-specific pathophysiology
- Disease-relevant phenotypic read-out
- Biologically relevant

TRANSLATIONAL

- Functional manifestation of disease
- Drug candidate
- *Clinical-trials-in-a-dish*

*A fully integrated platform helping (bio)pharma **redefine health** and **disease***

Better Human Models, **Better Discoveries.**

iPSCore

Thank You!

Margaret Barlow
Business Development

READY FOR BETTER DISCOVERIES?

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