



LEADING
REGENERATIVE
MEDICINE

**BIOTECH
SHOWCASE™
2014**

Innovation • Opportunity • Collaboration

JANUARY 13-15, 2014
SAN FRANCISCO, CA, USA
Parc 55 Wyndham San Francisco - Union Square



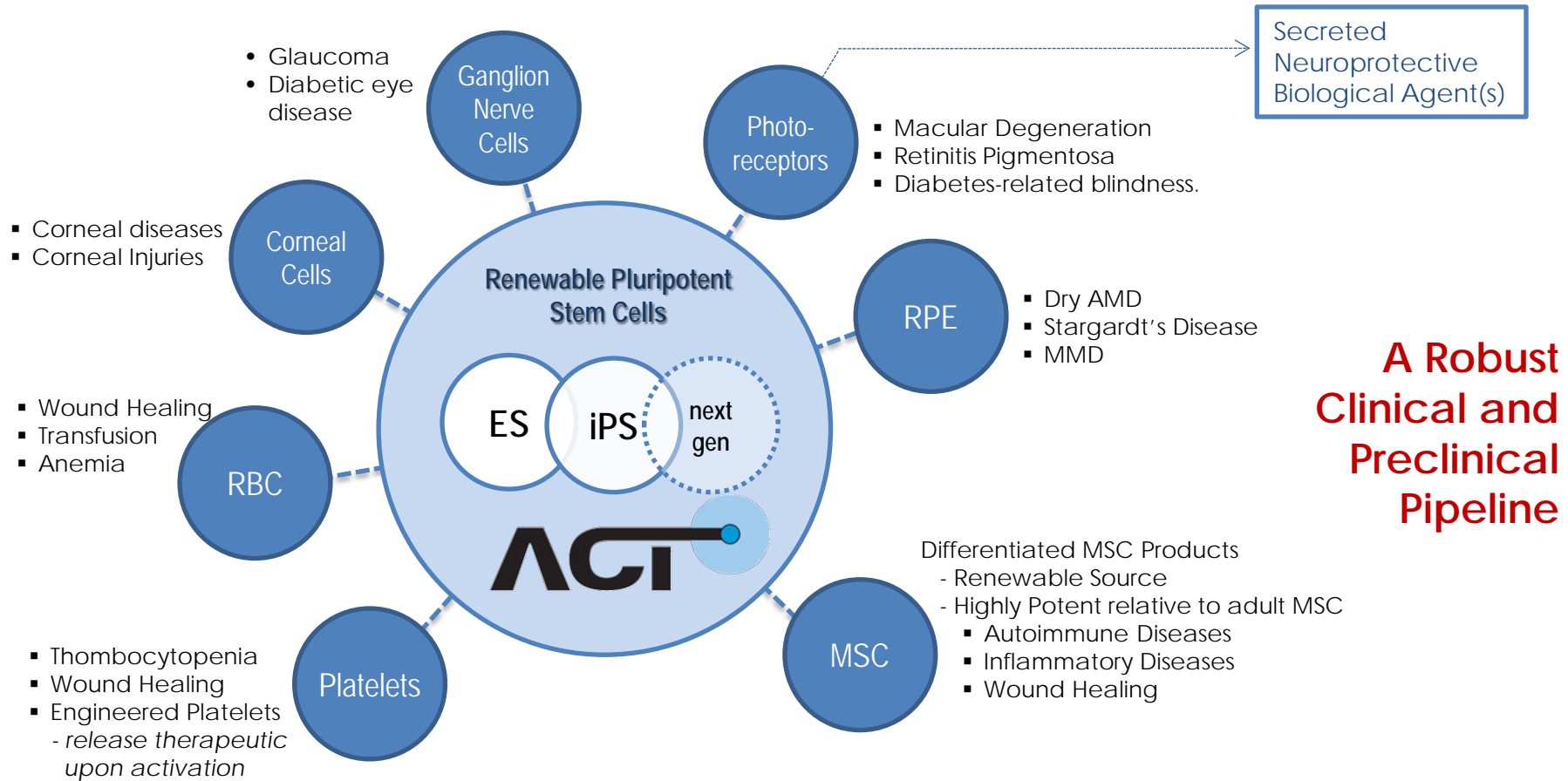
Cautionary Statement Concerning Forward-Looking Statements

This presentation is intended to present a summary of ACT's ("ACT", or "Advanced Cell Technology Inc", or "the Company") salient business characteristics.

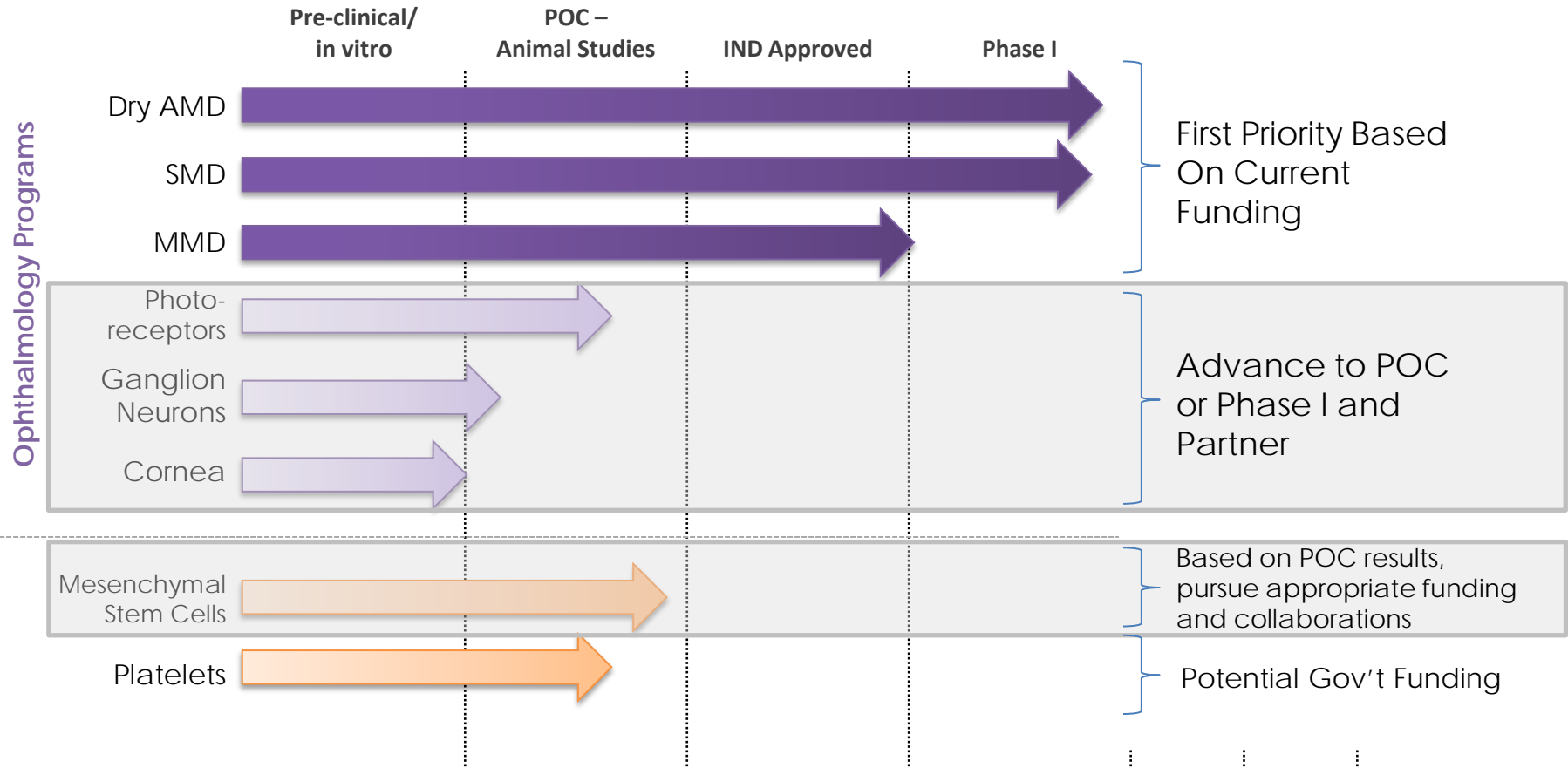
The information herein contains "forward-looking statements" as defined under the federal securities laws. Actual results could vary materially. Factors that could cause actual results to vary materially are described in our filings with the Securities and Exchange Commission.

You should pay particular attention to the "risk factors" contained in documents we file from time to time with the Securities and Exchange Commission. The risks identified therein, as well as others not identified by the Company, could cause the Company's actual results to differ materially from those expressed in any forward-looking statements.

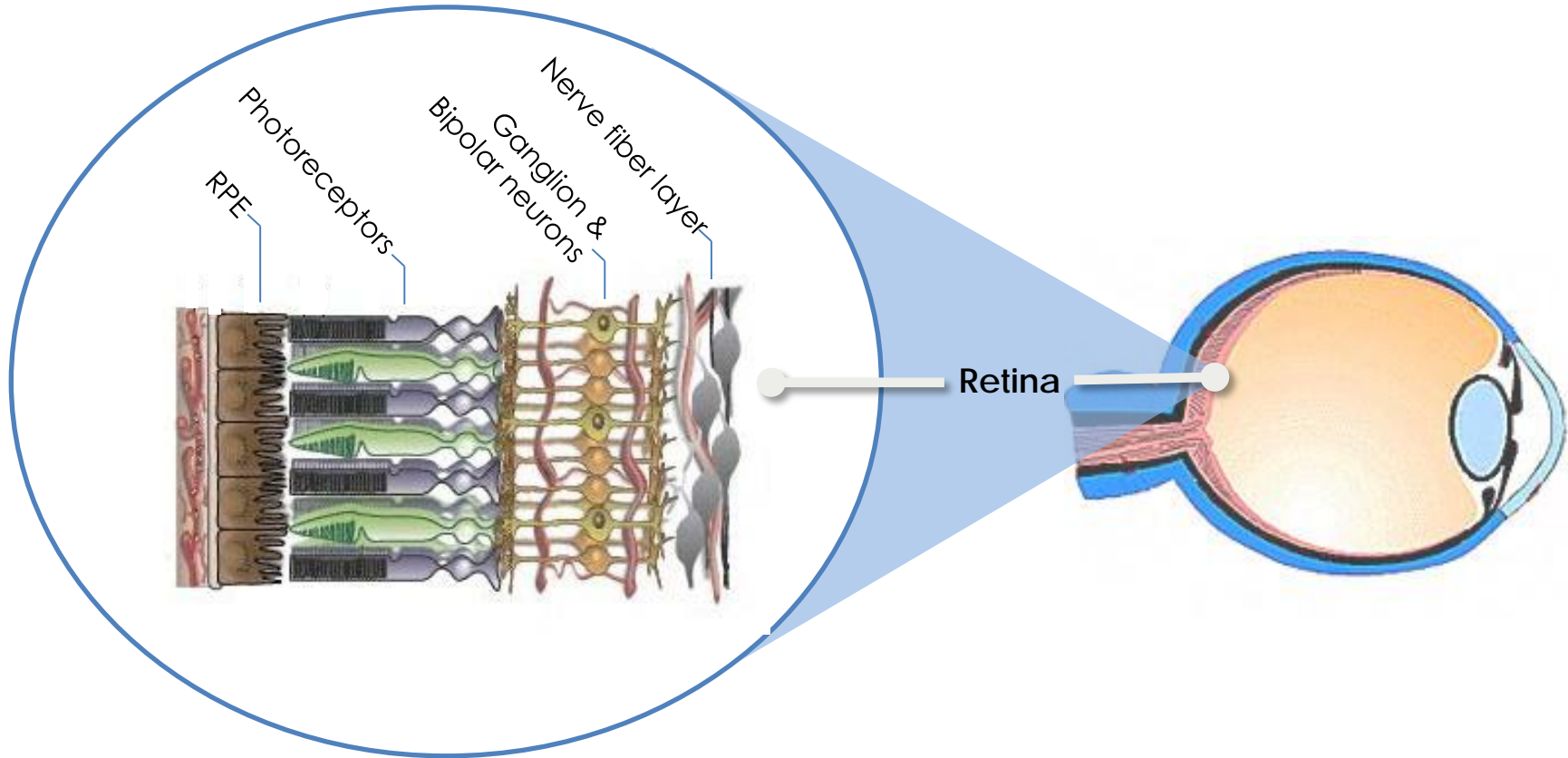
Renewable Pluripotent Stem Cell Platform



Robust Development Pipeline Provides Multiple Opportunities to Commercialize and Partner

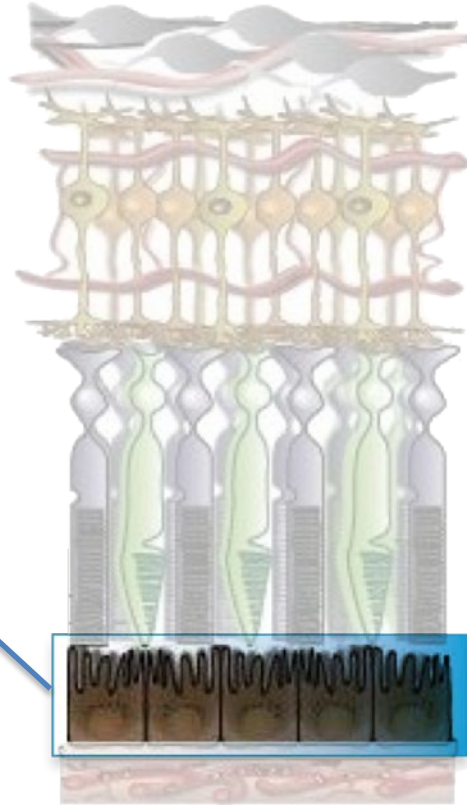


Structure of the Retina



Life Support to Photoreceptors

RPE Layer has
**multiple
critical roles**
in the
**health and
function**
of photoreceptors and
the retina as a whole.



Provides nutrients and growth factors

- photoreceptors see no blood

Recycles Vitamin A

- maintains photoreceptor excitability

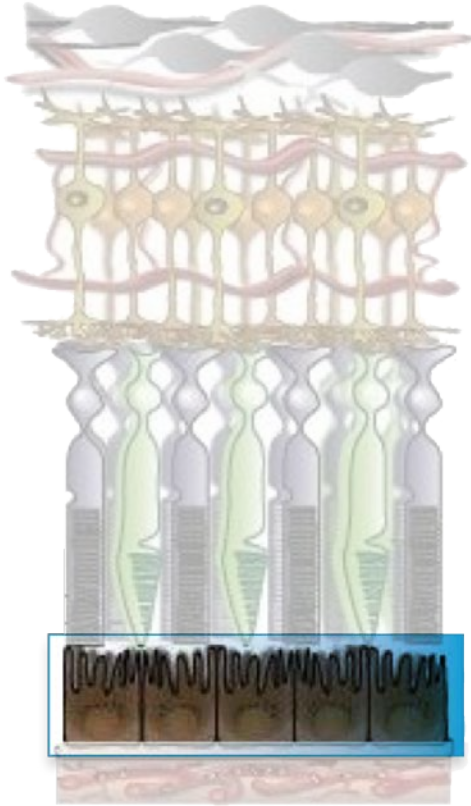
Detoxifies photoreceptor layer

Maintains Bruch's Membrane

- natural antiangiogenic barrier
- immune privilege of retina

Absorbs stray light / protects from UV

Life Support to Photoreceptors

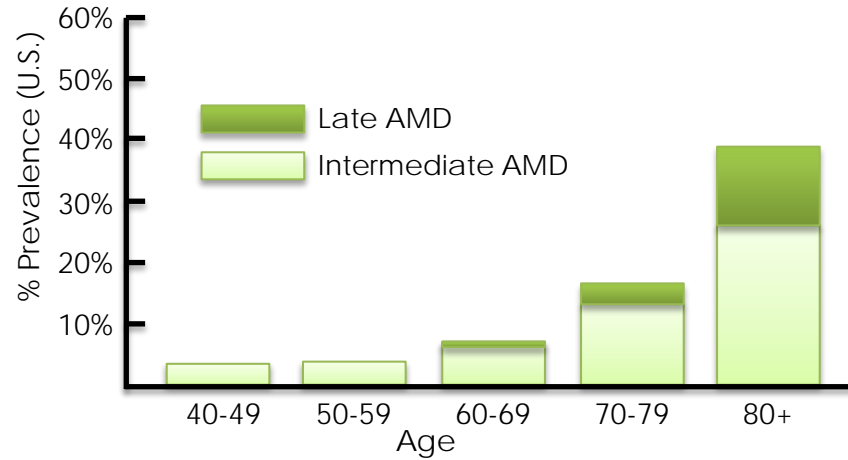


Failure of RPE cells
results in many
degenerative diseases

Stargardt's disease
Myopic Macular Dystrophy
Age-related macular degeneration (AMD)

Age-Related Macular Degeneration will Soon Take on Aspects of an Epidemic

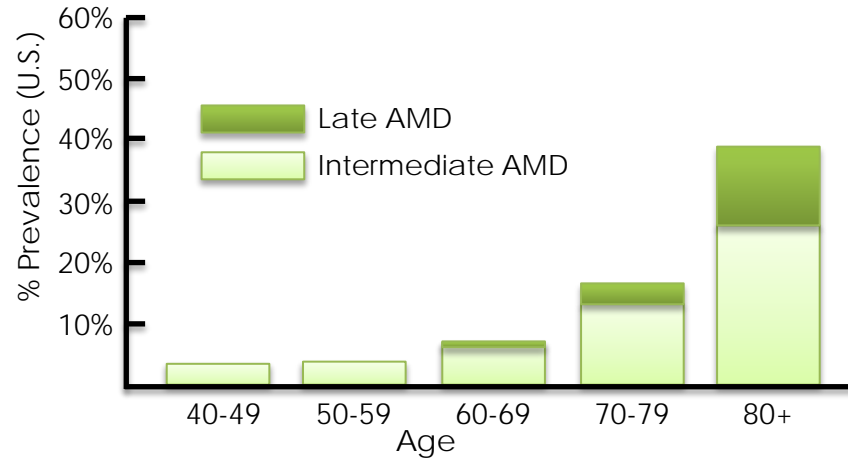
Exponential rise in prevalence and incidence rates with age, with prevalence rates of late AMD quadrupling per decade



Data from <http://www.nei.nih.gov/eyedata/> and
U.S. Census Bureau Publication "65+ in the United States", P23-209

Age-Related Macular Degeneration will Soon Take on Aspects of an Epidemic

Exponential rise in prevalence and incidence rates with age, with prevalence rates of late AMD quadrupling per decade



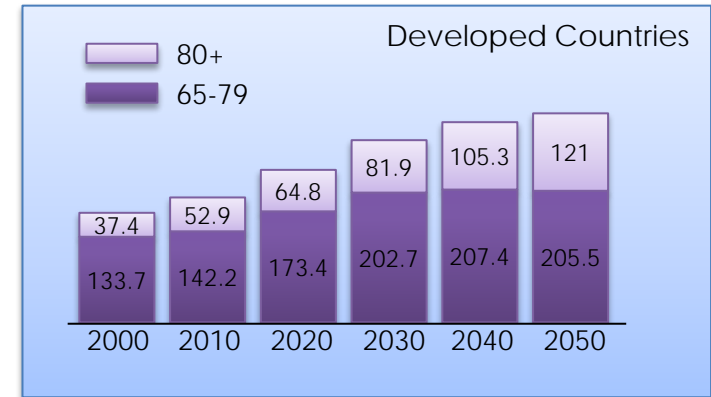
Data from <http://www.nei.nih.gov/eyedata/> and
U.S. Census Bureau Publication "65+ in the United States", P23-209

Age-Related Macular Degeneration will Soon Take on Aspects of an Epidemic

There are currently >30 Million American and European AMD patients. This is projected to exceed 50 Million patients by 2025

Wong et al. *Lancet* January 2014

The projected number of people with age-related macular degeneration in 2020 is 196 million, increasing to 288 million in 2040.



"macular degeneration will soon take on aspects of an epidemic"
- former Director of the National Eye Institute Dr Carl Kupfer

Data from <http://www.nei.nih.gov/eyedata/> and U.S. Census Bureau Publication "65+ in the United States", P23-209

Cell Therapy for RPE, Achievable by a Small Company

Small dosage size

- less than 200K cells

Immune-privileged site

- minimal immunosuppression

Ease of administration

- no separate device approval

Unique measuring and observation environment

- measurable endpoints

Significant unmet medical need

GMP Process

GMP process for differentiation and purification of RPE

- Virtually unlimited supply from stem cell source
- Optimized for large scale manufacturing

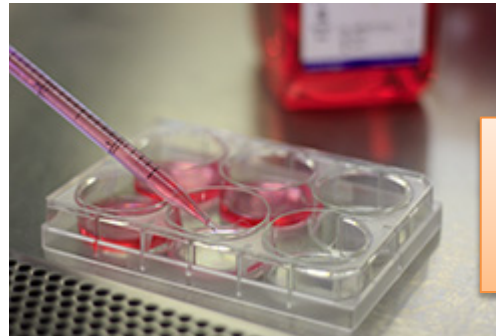
Product Cold Chain is Easily Scaled for Global Sales

ACT Cleanroom Suite



Ideal Cell Therapy Product

- Centralized Manufacturing
- Robust Release Assays
- Simple Handling

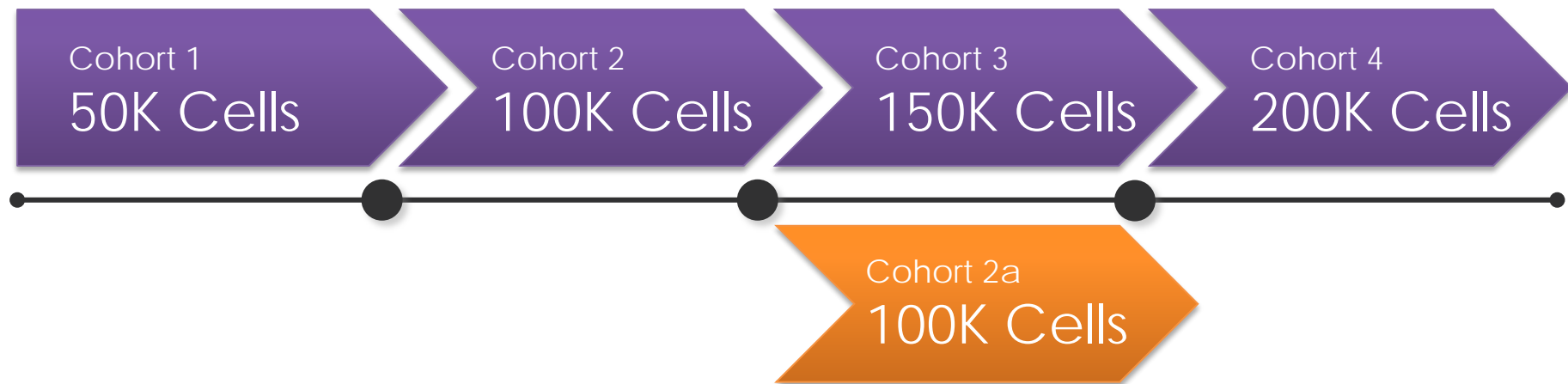


A single 6-well plate
can generate 50-
100 doses

First Treatments informed a more aggressive strategy to treat “better vision” cohort, could lead to broader label and/or earlier approval

January 2013: FDA approved additional 4 patient “better vision” cohorts in each trial.

For Cohort 2a – can enroll patients with vision as good as **20/100**.



Clinical Trials being led by World Leaders in Ophthalmology



World renowned leadership to help us navigate the clinical path and ultimately support market launch

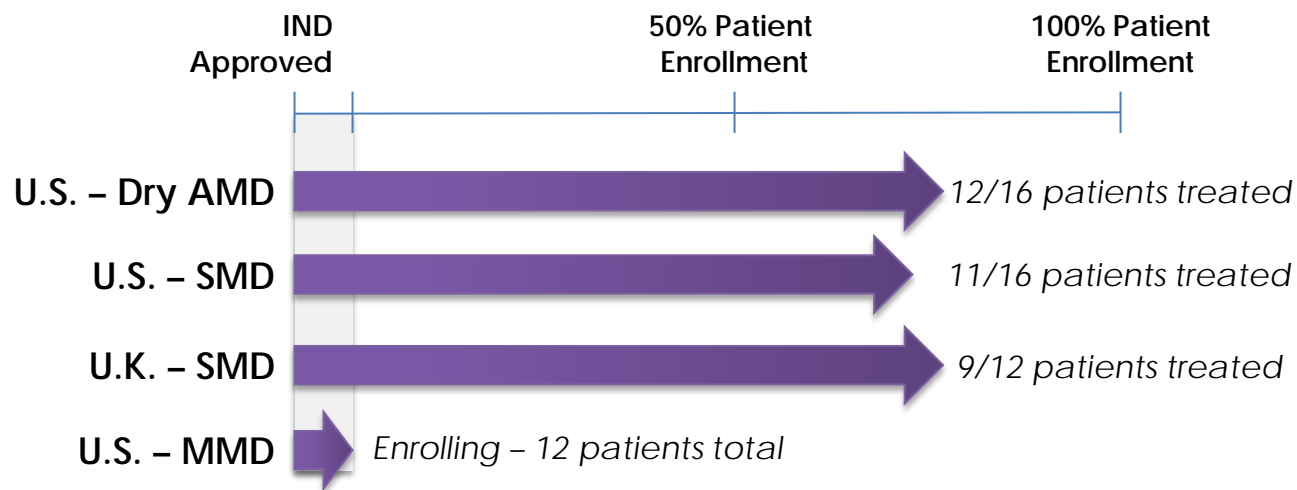
Surgical Overview

Procedure:

- 25 Gauge Pars Plana Vitrectomy
- Posterior Vitreous Separation
- Subretinal hESC-derived RPE cells injection
- Bleb Confirmation
- Day Surgery/Sedation only



Current Enrollment

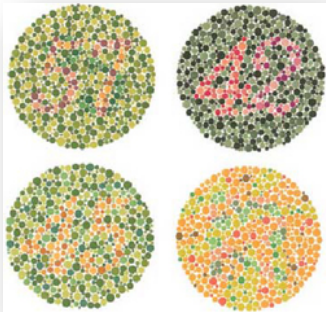


32 Patients Treated to Date

Phase I Trials Exceeding Expectations – no adverse events and persistence of cells

- No Adverse Events
- Persistence of cells
- Impact on Acuity

Recorded functional visual improvements in majority of patients.



- Increased letters on ETDRS Charts
- Color perception
- Contrast
- Low light vision



Preliminary Results

Recorded **functional visual improvements** in both patients.

- SMD Patient: Best corrected visual acuity improved from hand motions to 20/800 and improved from 0 to 5 letters on the ETDRS visual acuity chart in the study eye.
- Dry AMD Patient: Vision improved in the patient with dry age-related macular degeneration (21 ETDRS letters to 28).

32 Patients - up to 2 years of follow-up visits

- Measurable Improvements in Visual Acuity for Majority of Treated Patients
- Gains in visual acuity generally persist

SMD Patient #1

	BCVA	ETDRS (number of letters)
Fellow eye		
Baseline	Hand motion	0
1 week	Hand motion	0
2 weeks	Hand motion	0
3 weeks	Hand motion	0
4 weeks	Hand motion	0
6 weeks	Hand motion	0
8 weeks	Hand motion	0
12 weeks	Hand motion	0
Operated eye		
Baseline	Hand motion	0
1 week	Counting fingers	0
2 weeks	Counting fingers	1
3 weeks	Counting fingers	3
4 weeks	20/800	5
6 weeks	20/800	5
8 weeks	20/800	5
12 weeks	20/800	5

Visual acuity gains have persisted for more than 2 years

Expanding Clinical Programs

Myopia creates a higher risk of permanent vision loss due to **Myopic Macular Degeneration** (MMD)

- Severe near-sightedness causes elongation of the eyeball -- which can cause fissures in RPE layer.



**January 2013 - FDA Approved
MMD Phase I/II study**

Jules Stein Eye Institute (UCLA) and ACT

Second Generation RPE Cell Therapy Products

By engineering the master stem cell bank used to manufacture RPE cells, the transplanted RPE cells can express

- **Anti-angiogenic agents**

Reduce occurrence of choroidal neovascularization (wet AMD).

- **Complement factor D, Factor C5 and/or Factor C3 Inhibitors**

Activation of alternative complement pathway implicated in disease progression for certain patients

- **Anti β -amyloid agents**

Drusen deposits resemble amyloid deposits.

- **Anti-Inflammatory agents**

IL-1, IL-2, IL-3, and TNF- α antagonists

Recombinant Lipocortin – a potent anti-inflammatory protein

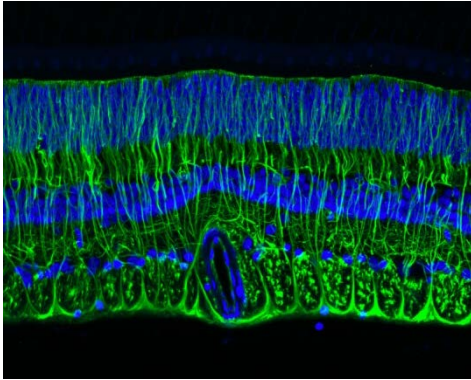
Intellectual Property – RPE Program

Dominant Patent Position for Treating Retinal Degeneration

- Broad Coverage for Manufacturing RPE Cells
- Broad protection of pharmaceutical preparations
Covers both RPE cell suspensions and scaffolded RPE layers.
- RPE Cells derived from other pluripotent stem cells – e.g., iPS cells

Keeping our
IP Lawyers
on their toes

- Careful Consideration of Literal Scope
- Preservation of Doctrine of Equivalents
- Constantly Mining Existing Filings
- Vigilantly Filing on Improvements

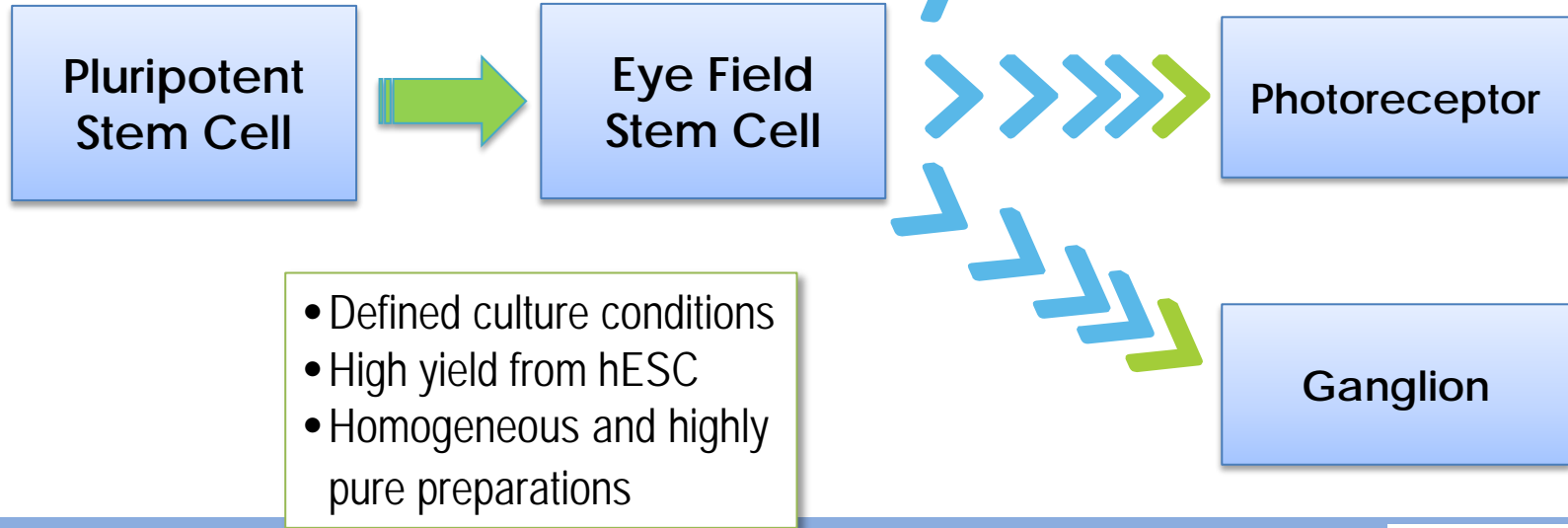


Neurosensory Retina

Photoreceptor and Ganglion Progenitor Cells

Ocular Program – Retinal Neural Progenitors

ACT has developed proprietary methods for deriving various cell types of the retina

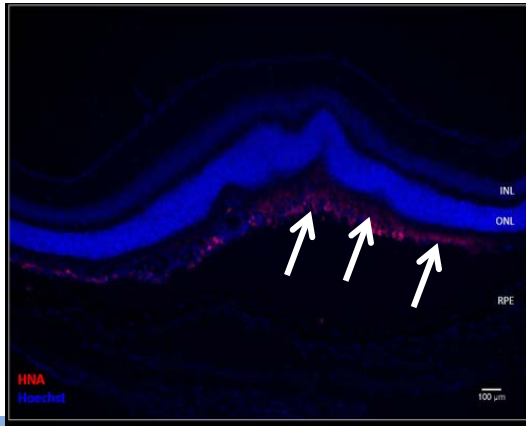


Photoreceptor Progenitor Cells

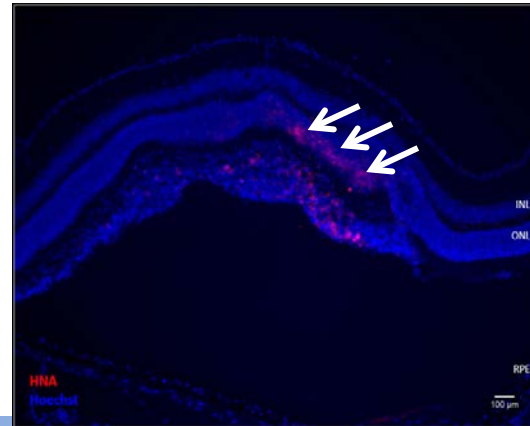
Sub-retinal injection of Photoreceptor Progenitor cells **promoted functional of recovery** photoreceptor function in animal models of photoreceptor loss

↳ Observed incorporation of human photoreceptor cells into Outer Nucleated Layer

1 week after subretinal transplantation



3 week after subretinal transplantation

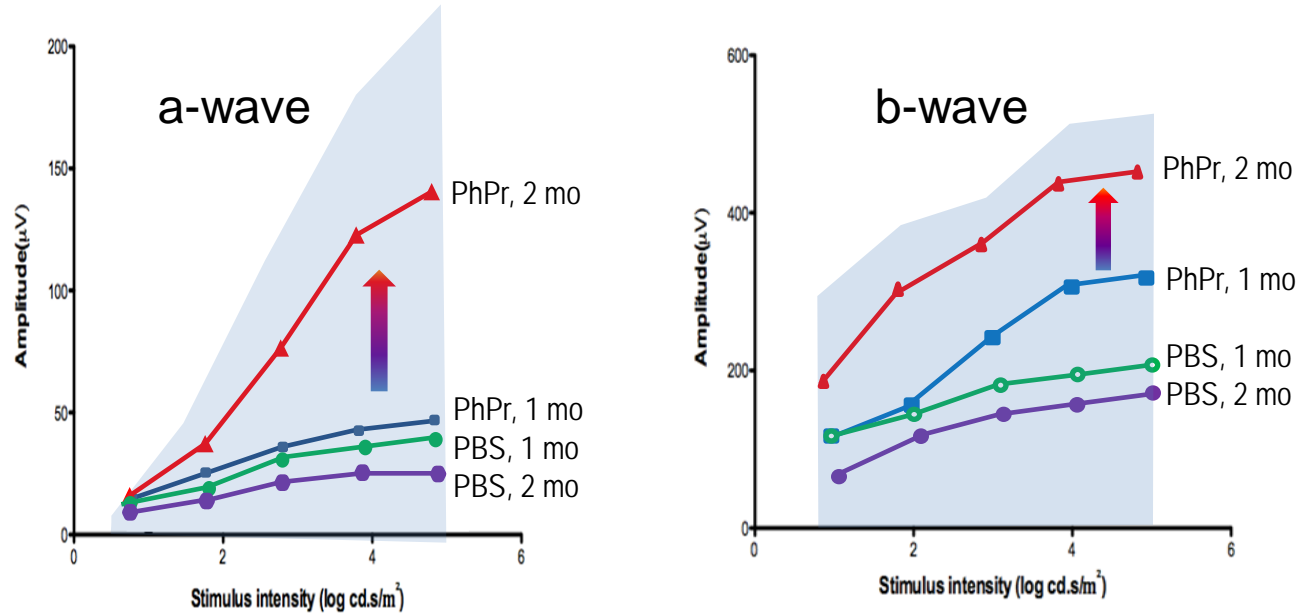


Photoreceptor Progenitor Cells

In addition, **systemic injection** of progenitor cells had the **surprising** result of providing **neuroprotective** activity

Preliminary data suggests cells secrete soluble and potent neuroprotective agent(s)

Evidence for Secreted Neuroprotective Agent

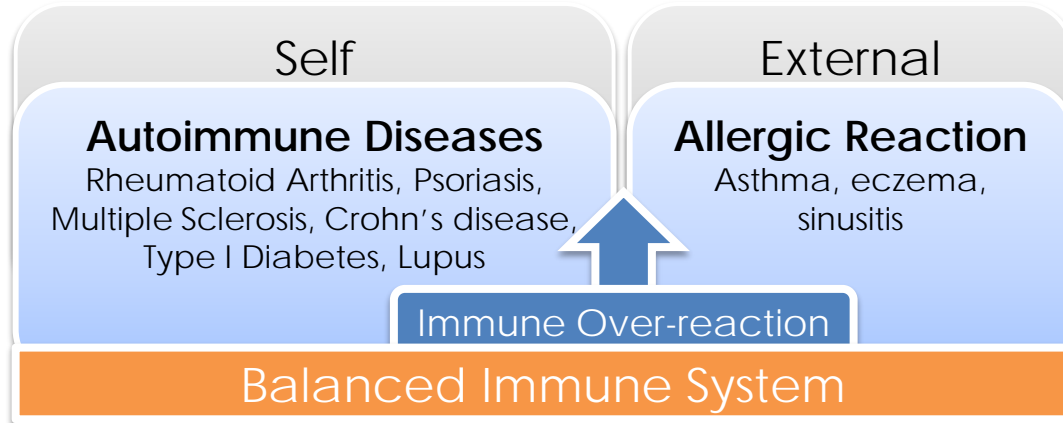


Systemically delivered Photoreceptor Progenitor cells reversed the progression of photoreceptor degeneration – and promoted regeneration of both Rods and Cones



Mesenchymal Stem Cell Program

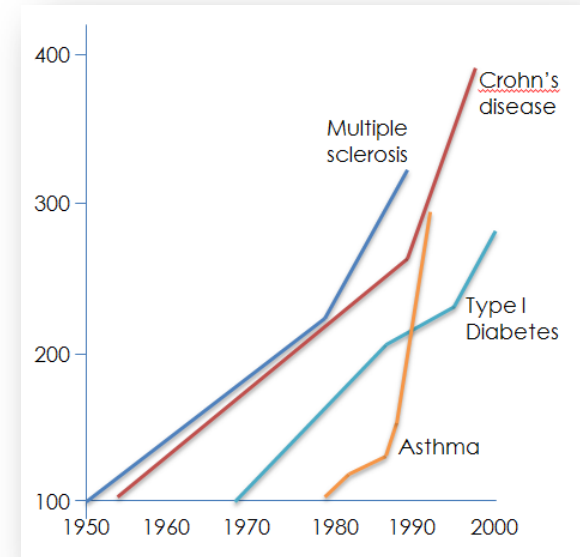
Mesenchymal Stem Cells in Therapy



Autoimmune Disease Prevalence

- At least 80 disease affecting every organ system
- Americans spend over **\$100B** each year in total healthcare costs associated with autoimmune disease
- In the U.S., **14.7-23.5M people (5%-8%)**
(for comparison: heart disease (22M), Cancer (9M))

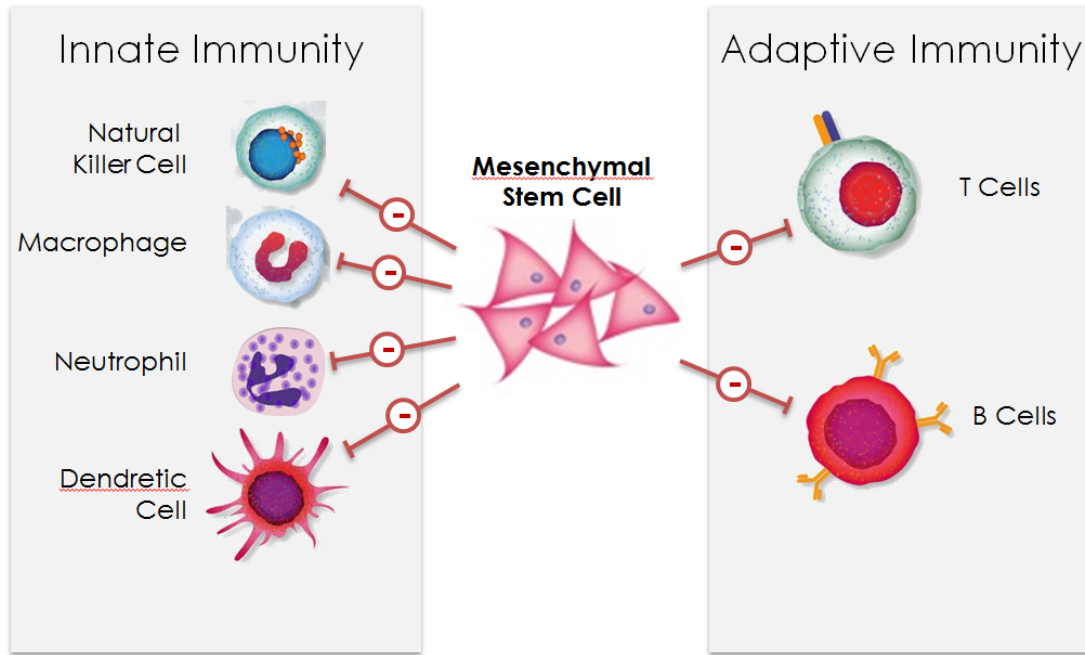
A rapidly growing health issue (% growth)



Suppressing Immune Responses gives rise to Therapy

Mesenchymal stem cells (MSCs)

suppress disease-causing immune responses



Promising **therapeutic potential** for treating autoimmune and inflammatory diseases.

However, **adult-derived MSCs** are **limited** by **replicative capacity**

ACT's Breakthrough – Inexhaustible Supply of Very Potent MSC's

ACT Proprietary Process

- Manufacture MSC's from hES and iPS Cell Banks
 - Virtually **inexhaustible source** of starting material
 - Use Single Master Cell Bank
 - Less labor-intensive

A further differentiating feature...

Our MSC's are substantially **more potent** than current sources of cells

An Experienced and Dedicated Management Team

Gary Rabin –CEO

Edward Myles – CFO and EVP of Corp Development

Dr. Matthew Vincent, Ph.D. – Dir., Business Development

Dr. Robert Lanza, MD – Chief Scientific Officer

Dr. Irina Klimanskaya, Ph.D. – Dir., Stem Cell Biology

Dr. Shi-Jiang (John) Lu, Ph.D. – Senior Director of Research

Eddy Anglade, M.D. – EVP, Clinical Development

Dr. Roger Gay, Ph.D. - Senior Director of Manufacturing

Proven business leaders who can develop and implement corporate strategy and monetize assets to maximize shareholder value

World-renowned scientific thought leaders pushing the cutting edge of science to develop important therapies

Deep experience in clinical development programs for ophthalmology drug products from early through late-and post-marketing stages

GMP manufacturing to ensure the highest quality products are delivered to our patients

A World-Class Board of Directors

Michael Heffernan	CEO – Collegium Pharmaceuticals
Robert S. Langer, Sc.D.	Institute Professor, MIT
Zohar Loshitzer	CEO – Presbia, Inc., & Principal in Orchard Capital
Greg Perry	EVP & CFO – InVivo Therapeutics
Alan C. Shapiro	Finance Professor and Chairman of the Department of Finance and Business Economics (retired) – University of Southern California
Gary Rabin	CEO – Advanced Cell Technology



Thank you
For more information, visit www.advancedcell.com

