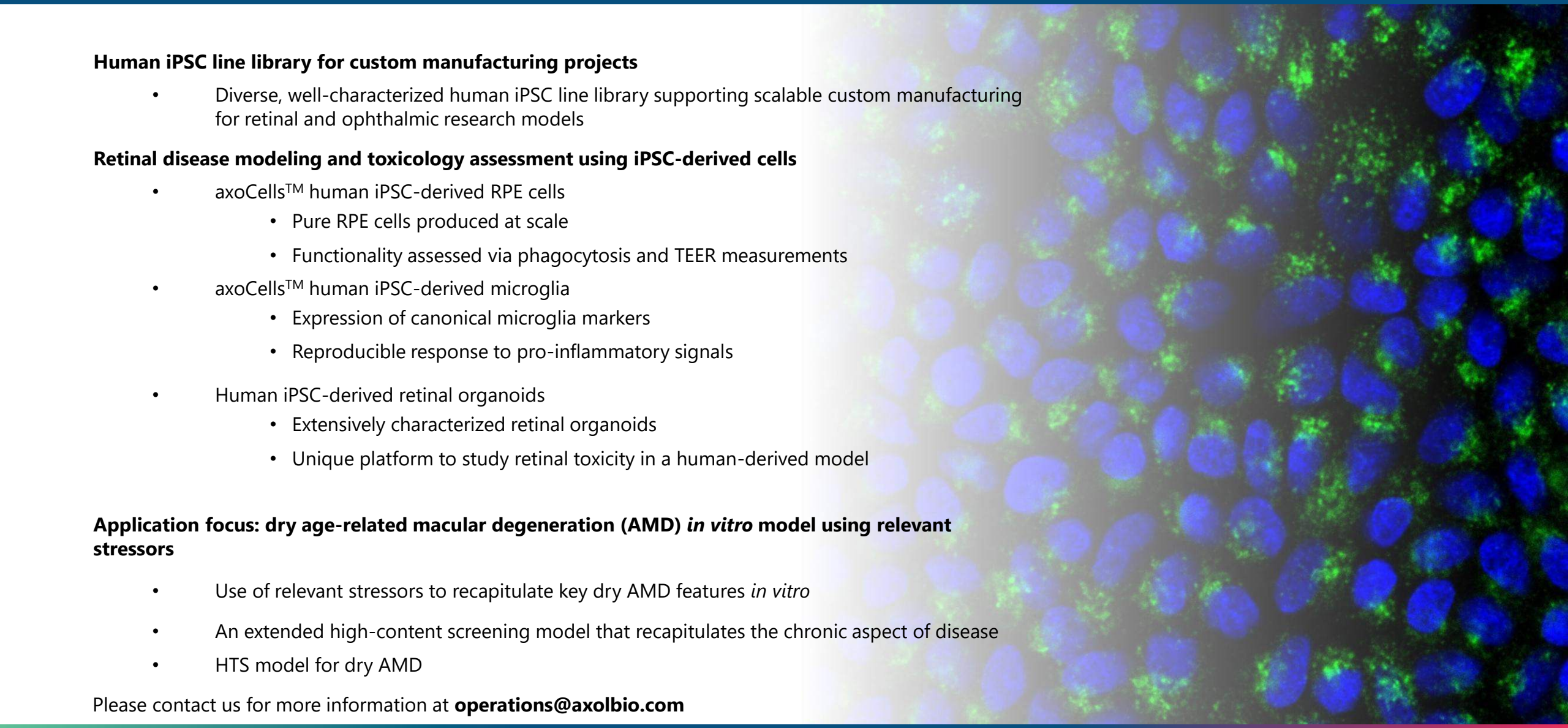
AXOL

Physiologically relevant *in vitro* retinal models for ophthalmology drug discovery and safety testing

Human iPSC-derived products and specialist services

eBook

Retinal drug discovery, research and safety using iPSC technology



Human iPSC line library for custom manufacturing projects

- Diverse, well-characterized human iPSC line library supporting scalable custom manufacturing for retinal and ophthalmic research models

Retinal disease modeling and toxicology assessment using iPSC-derived cells

- axoCells™ human iPSC-derived RPE cells
  - Pure RPE cells produced at scale
  - Functionality assessed via phagocytosis and TEER measurements
- axoCells™ human iPSC-derived microglia
  - Expression of canonical microglia markers
  - Reproducible response to pro-inflammatory signals
- Human iPSC-derived retinal organoids
  - Extensively characterized retinal organoids
  - Unique platform to study retinal toxicity in a human-derived model

Application focus: dry age-related macular degeneration (AMD) *in vitro* model using relevant stressors

- Use of relevant stressors to recapitulate key dry AMD features *in vitro*
- An extended high-content screening model that recapitulates the chronic aspect of disease
- HTS model for dry AMD

Please contact us for more information at **operations@axolbio.com**

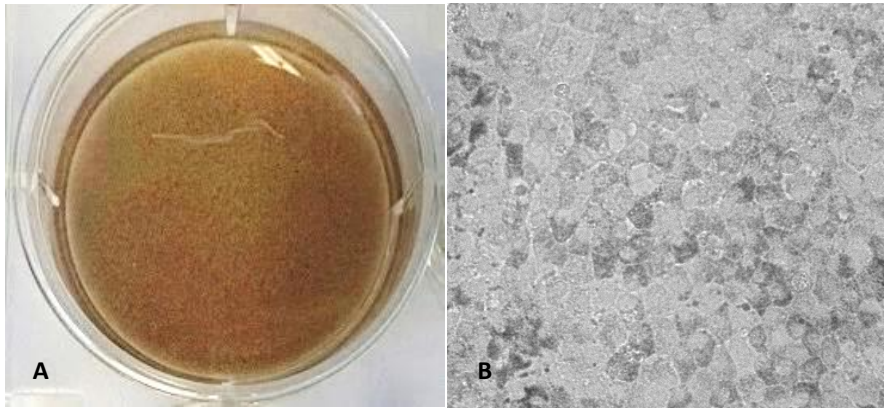
iPSC line library for custom manufacturing projects

				Relevant Genotypes					
iPSC line name	**Gender**	**Age at sampling**	**Disease status**	**CFH (rs1061170) - p.Y402H**	**ARMS2 (rs10490924) p.A69S**	**HTRA1 (rs11200638)**	**C3 (rs2230199) - p.R102G**	**APoE**	**Notes**
PCi-CAU2	M	30	Unaffected	p. Y402H +/+	p.A69S +/+	rs11200638 +/+	Unknown	Unknown	RPEs available as catalog product
CENSO-4E	M	45	Unaffected	p. Y402H +/+	p.A69S +/+	rs11200638 +/+	Unknown	Unknown	RPEs available in service projects*
PC093	F	70	AMD	p. Y402H -/-	p.A69S -/-	Unknown	p. R102G +/-	E2/E3	RPEs available in service projects*
PC101	M	73	AMD	p. Y402 +/+	p.A69S -/-	Unknown	p. R102G +/-	E2/E3	Custom manufacturing
PC102	F	83	AMD	p. Y402H -/-	p.A69S +/-	Unknown	p. R102G +/-	E3/E4	Custom manufacturing
PC105	F	91	AMD	p. Y402 +/+	p.A69S -/-	Unknown	p. R102 +/+	E3/E3	Custom manufacturing
PC115	F	85	AMD	p. Y402 +/+	p.A69 +/+	Unknown	p. R102G +/-	E3/E3	RPEs available in service projects*
PC207	F	71	AMD	p. Y402 +/+	p.A69 +/+	rs11200638 +/+	Unknown	Unknown	Custom manufacturing
PC208	F	87	AMD	p. Y402 +/+	p.A69 +/+	rs11200638 +/+	Unknown	Unknown	Custom manufacturing
PC209	M	70	AMD	p. Y402H -/-	p.A69S -/-	rs11200638 -/-	Unknown	Unknown	RPEs available in service projects*
PC214	F	67	AMD	p. Y402 +/+	p.A69 +/+	rs11200638 +/+	Unknown	Unknown	Custom manufacturing
PC215	F	72	AMD	p. Y402H -/-	p.A69S -/-	rs11200638 -/-	Unknown	Unknown	Custom manufacturing
PC216	F	88	AMD	p. Y402H +/-	p.A69S +/-	rs11200638 +/-	Unknown	Unknown	Custom manufacturing
PC217	F	75	AMD	p. Y402H -/-	p.A69S -/-	rs11200638 -/-	Unknown	Unknown	Custom manufacturing
PC218	F	91	AMD	p. Y402H -/-	p.A69S -/-	rs11200638 -/-	Unknown	Unknown	Custom manufacturing
PC219	F	88	AMD	p. Y402H +/-	p.A69S +/-	rs11200638 +/-	Unknown	Unknown	Custom manufacturing
PC220	F	89	AMD	p. Y402 +/+	p.A69 +/+	rs11200638 +/+	Unknown	Unknown	RPEs available in service projects*
CENSOi079-A	M	87	AMD	p. Y402H -/-	Unknown	Unknown	Unknown	Unknown	Custom manufacturing
CENSOi080-A	F	84	Unaffected	p. Y402 +/+	Unknown	Unknown	Unknown	Unknown	Custom manufacturing
CENSOi085-A	M	69	Retinopathy	BEST1 +/- [+/c.887A>G; (p.Asn296Ser)]					Available by end of 2026
CENSOi086-A	M	20	Retinopathy	BEST1 +/- [+/c.691A.C; (p.Ser231Arg)]					Available by end of 2026
CENSOi093-A	F	50	Retinopathy	BEST1 (TBC)					Available by end of 2026
CENSOi090-A/B	F	39	Micropthalmia	NM_0012026 c.-7-192G>A					Custom manufacturing
CENSOi091-A/B	F	4	Leber congenital amaurosis	CRB1 +/- [c.2401A>T; (p.Lys801Ter) / c.2688T>A; (p.Cys896Ter)]					Custom manufacturing
CENSOi092-A/B	F	4	Leber congenital amaurosis	CRB1 +/- [c.1339dupC; (p.His447ProfsTer7)/c.2401A>T; (p.Lys801Ter)]					Custom manufacturing
CENSOi082-A	F	58	Retinopathy	EFEMP1 +/- [+/c.1033C>T; (p.Arg345Trp)]					Available by end of 2026
CENSOi083-A	M	64	Retinopathy	EFEMP1 +/- [+/c.1033C>T; (p.Arg345Trp)]					Available by end of 2026
CENSOi094-A	M	76	Retinopathy	EFEMP1 +/- [+/c.1033C>T; (p.Arg345Trp)]					Available by end of 2026
CENSOi084-A	M	60	Ushers Syndrome Type1	MYO7A +/- (c.3719G>A; (p.Arg1240Gin)/ c.3764del; (p.Lys1255ArgfsTer8)]					Available by end of 2026
CENSOi095-A	F	TBC	Ushers Syndrome Type1	MYO7A +/- [c.223G>C; (p.Asp75His)/ c.6070C>T; (p.Arg2024Ter)]					Available by end of 2026
CENSOi081-A	F	24	Retinopathy	MERTK +/- [c.1868T>A; (p.Leu623Ter) / c.2167G>A; (p.Asp723Asn)]					Available by end of 2026
CENSOi087-A	M	21	Retinopathy	MERTK +/- [+/c.2164C>T; (p.Arg722Ter)/ multi-exon deletion]					Available by end of 2026
CENSOi088-A	F	18	Retinopathy	MERTK -/- [c.2163T>A; (p.His721Gln)]					Available by end of 2026
CENSOi089-A	F	51	Unaffected	MERTK +/- [c.2163T>A; (p.His721Gln)]					Available by end of 2026

Phenotypic characterization of iPSC-derived RPE cells

Phenotypic characterization: morphology

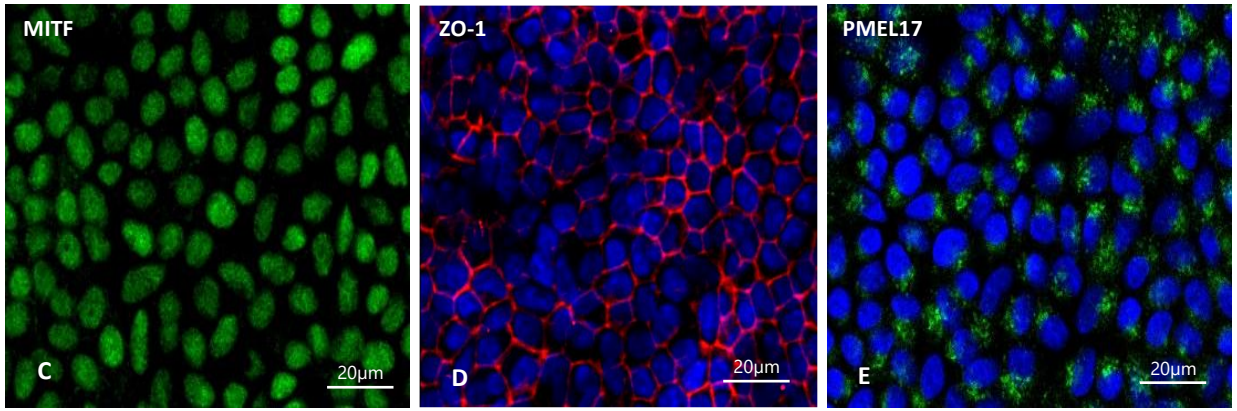
Human iPSC-derived RPE cells demonstrate typical cobblestone morphology and pigmentation on microscopy.



Morphology of mature RPE cells in both macroscopic and microscopic views

Phenotypic characterization: expression of RPE markers

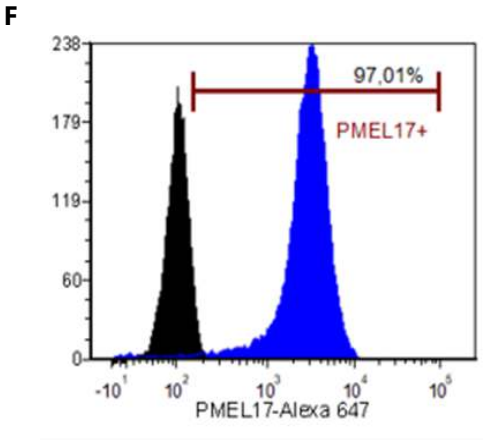
RPE cells express key markers MITF, ZO-1 and PMEL17



ICC images demonstrating expression of key markers MITF, ZO-1 (tight junction) and PMEL17 (melanosome)

Phenotypic relevance: flow cytometry

RPE cells demonstrated >95% PMEL17 expression against isotype control.



Flow cytometry demonstrating >95% PMEL17 expression (blue) against isotype control (black).

Functional characterization of iPSC-derived RPE cells

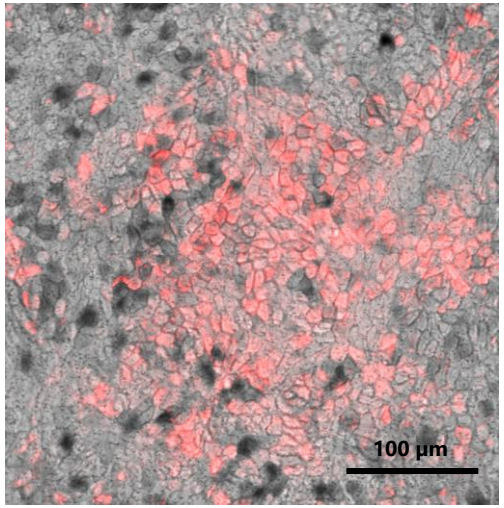
Functional characterization: phagocytosis and TEER measurement

(A) RPE cells incubated with pHrodo labelled photoreceptor outer segments (POS) for 4h. Demonstrates iPSC-derived RPE cells phagocytosis of POS, an essential physiological function *in vivo*.

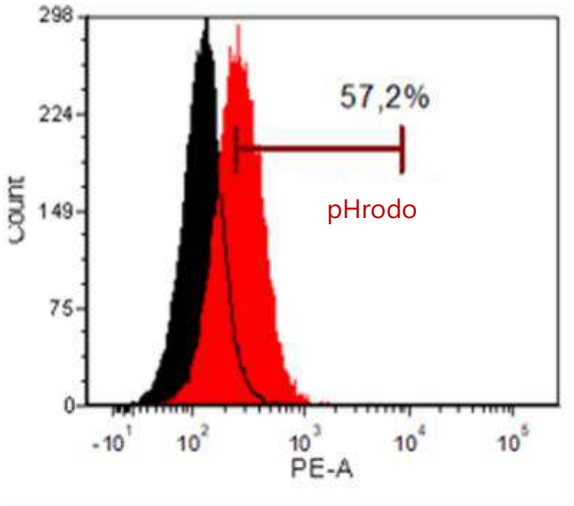
(B) Quantification of the phagocytotic potential of iPSC-derived RPE cells by flow cytometry, demonstrating phagocytotic activity in 57.2% of the RPE cells.

(C) TEER increases during RPE maturation, reflecting the development of a functional epithelial barrier.

A



B



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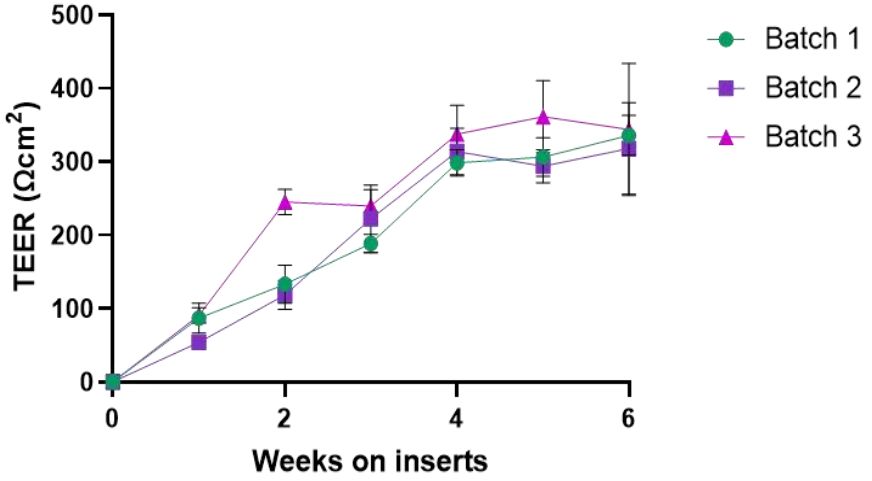
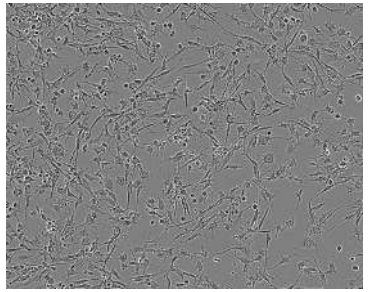


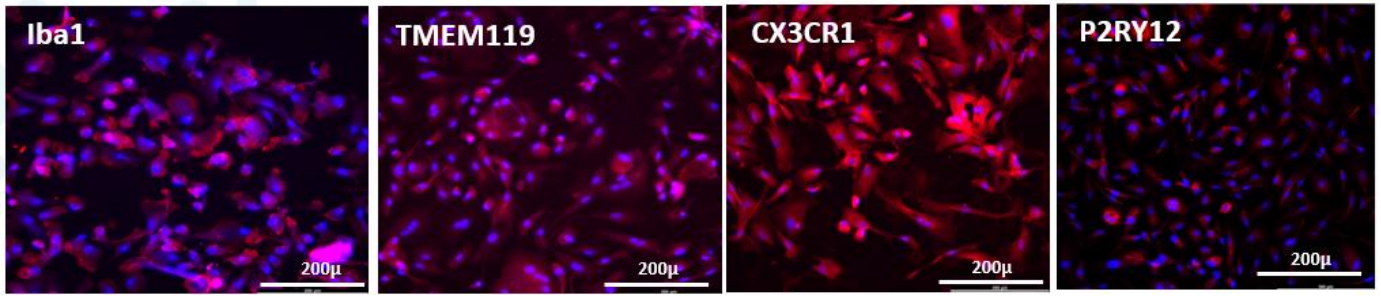
Characterization of iPSC-derived microglia

Phenotypic characterization: morphology and expression of microglia markers

Microglia show expected elongated and ramified morphology, and expression of microglia canonical markers



iPSC-derived microglia were thawed and matured for 7 days on Surebond XF and concanavalin A

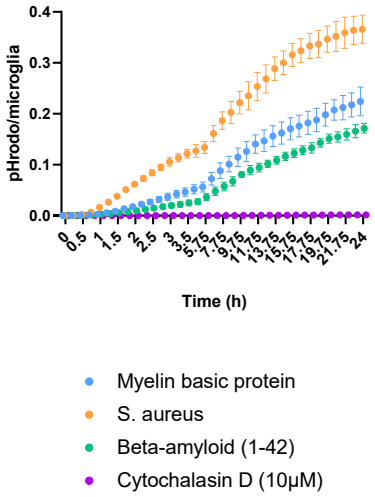


iPSC-derived microglia express key microglia markers Iba1, TMEM119, CX3CR1 and P2RY12. Red: Marker of interest, Blue: DAPI

Functional characterization:

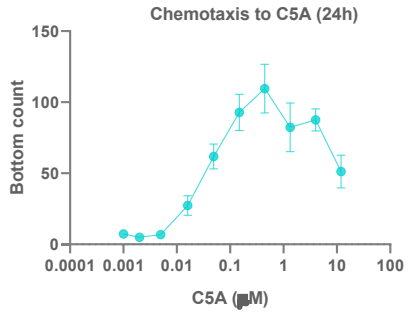
Phagocytosis

Microglia were fed with pHrodo labelled bait. Phagocytosis monitored over 24h . Cytochalasin D (10uM) was used as a negative control and showed complete inhibition of phagocytosis.



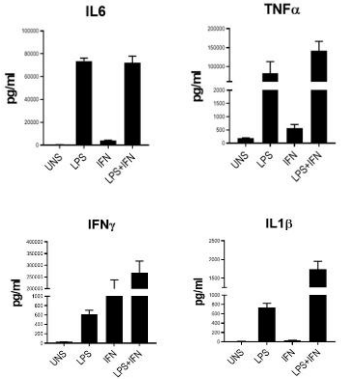
Chemotaxis

Chemotaxis assay toward C5a on iPSC-derived microglia. Microglia were plated in chemotaxis chambers. Cell migration toward different C5a concentrations was measured after 24 h using live imaging. Data are n=4 ± SEM.



Cytokine release

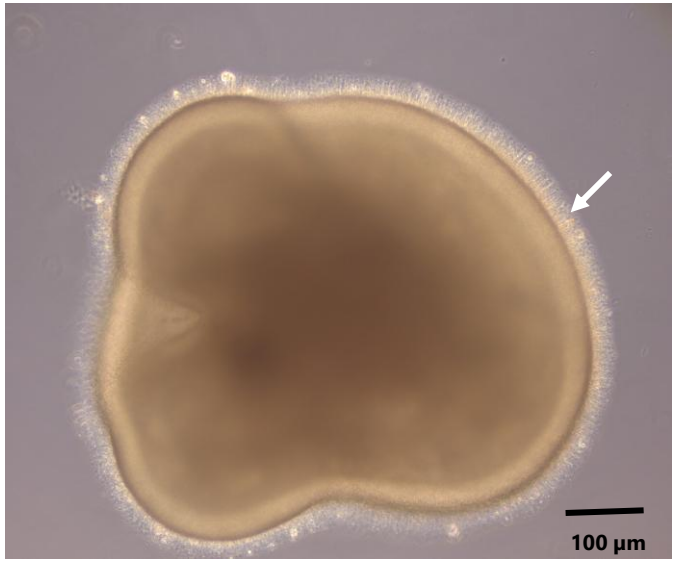
Cytokine release from fresh microglia following 24 h stimulation with LPS, INFγ or both (n=3). UNS= unstimulated. The expected pattern of cytokine release demonstrated functional relevance.



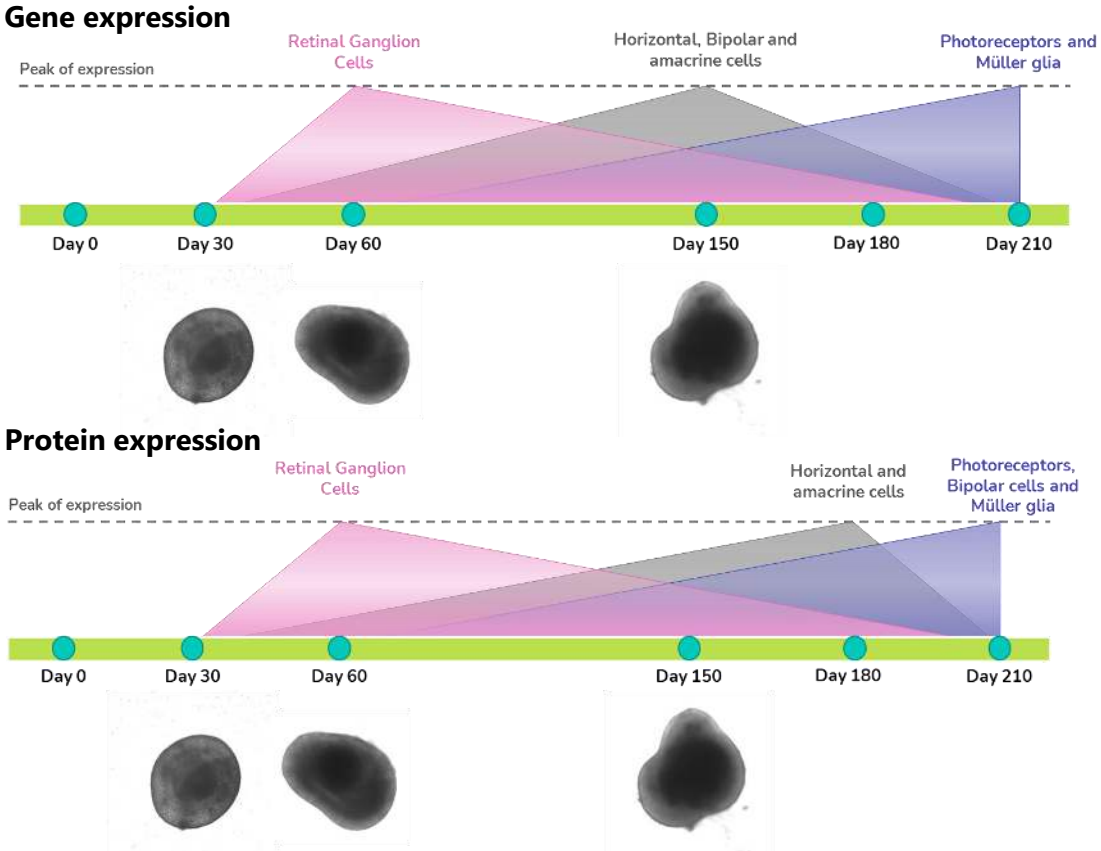
Retinal organoids characterization: temporal gene and protein expression

Retinal organoid generation follows the developmental timeline of retinogenesis *in vivo*

- Retinal organoids are three-dimensional structures derived from iPSCs that mimic the cellular composition and multi-layer architecture of the human retina
- Retinal organoids represent a physiologically relevant tool to study retinal development, disease mechanisms, toxicity, and potential therapeutic interventions in a highly controlled environment and with high translatability to human



Mature retinal organoids exhibit characteristic phase-bright outer rim and elongated outer segment-like structures (white arrow).



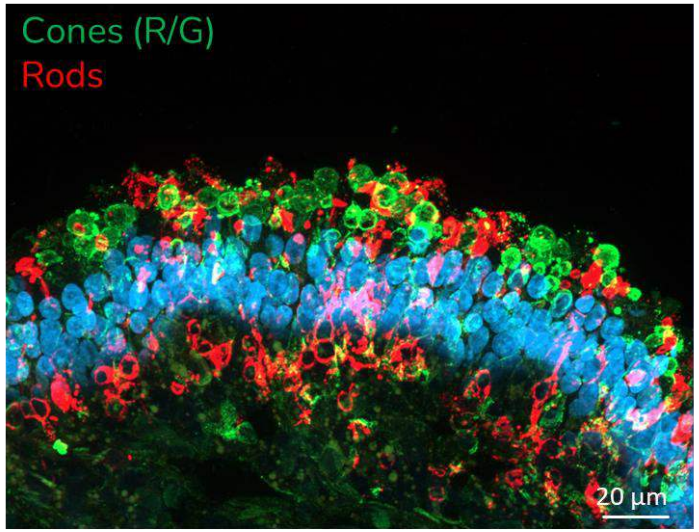
Depending on the cell type of interest, the organoids can be used at different stages of development

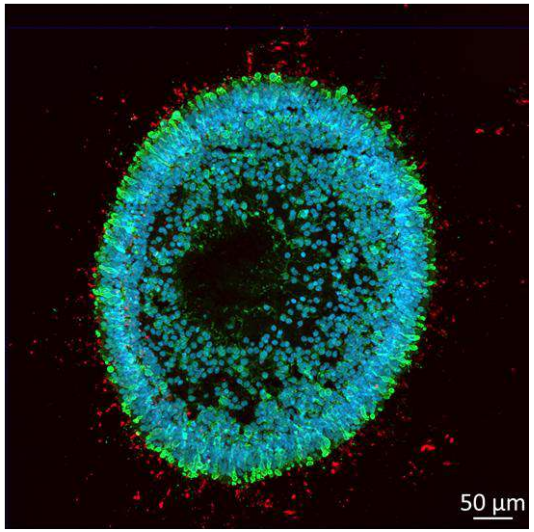
- Retinal ganglion cells are more abundant at day 60
- At day 150 the organoids contain major retinal cell types organized in a laminated manner
- Bipolar cells, cone and rod photoreceptors being more prevalent from day 180

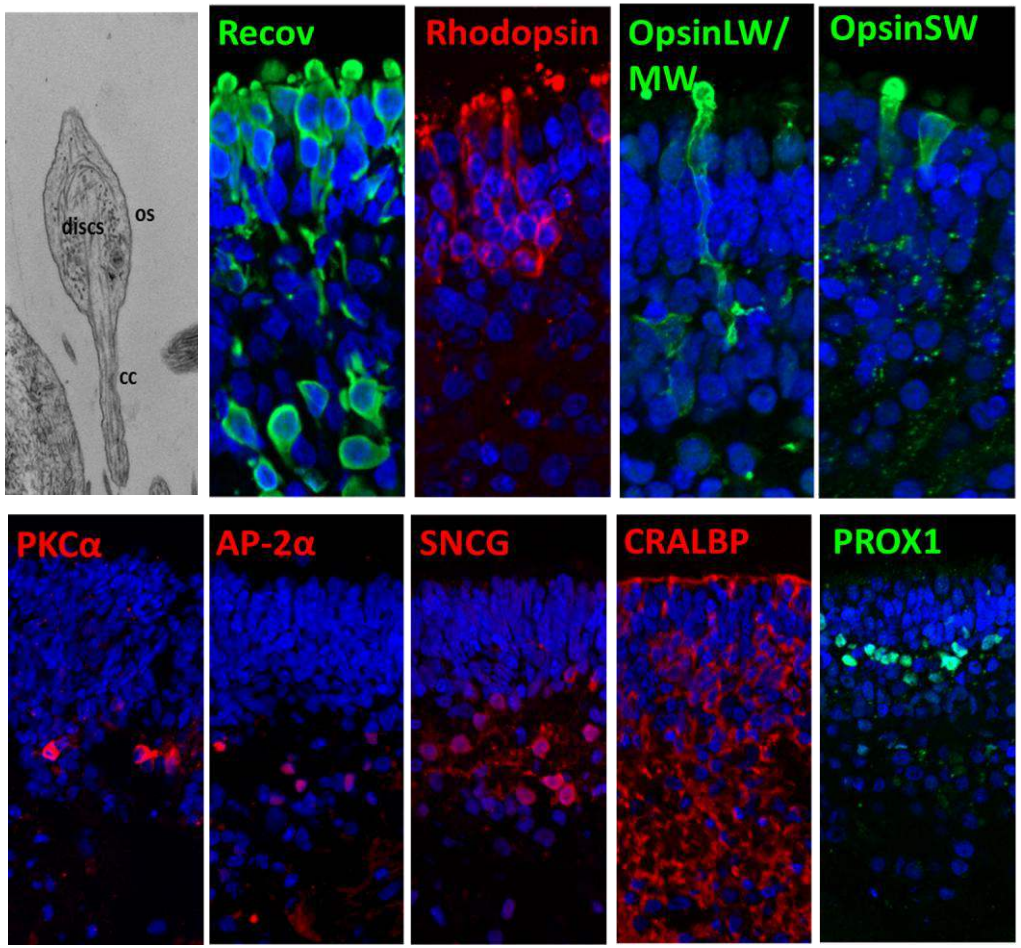
Retinal organoid characterization: expression of key retinal markers

Phenotypic characterization: expression of retinal organoid markers

iPSC-derived retinal organoids contain the major retinal cell types organized in a laminar structure, as shown by the expression of Recoverin (photoreceptors), Rhodopsin (rods), Opsin LW/MW and SW (cones), PKC-α (bipolar cells), AP-2α (amacrine cells), SNCG (ganglion cells), CRALBP (Müller glial cells), and PROX1 (horizontal cells).





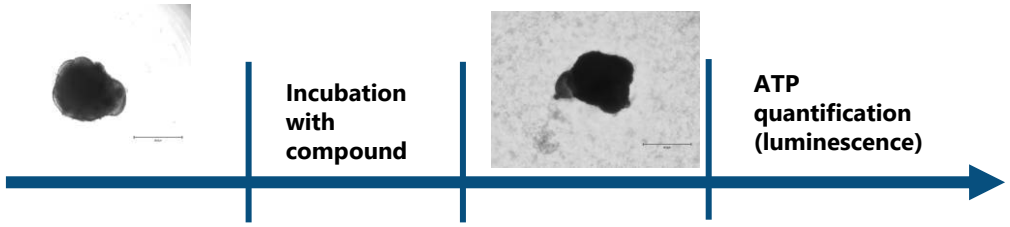


Chichagova et al. *Stem Cells*. 2019

Retinal toxicology assessment using iPSC-derived retinal organoids

Retinal organoids respond to compounds known to induce retinal toxicity in a dose-response manner

Assay overview



Drug treatment effect on retinal cell viability

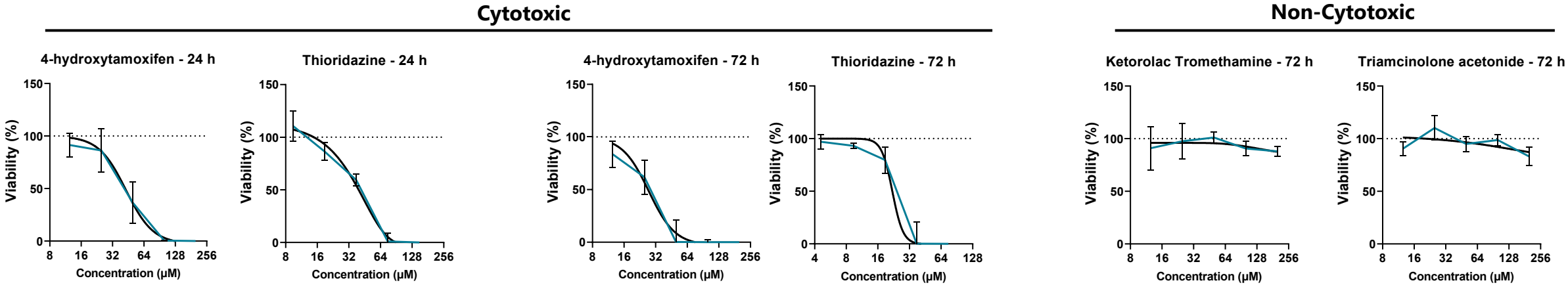
Dose-response curve demonstrating the dose-dependent effect of known cytotoxic drug to the retina, on retinal organoids.

(A) Organoid health was monitored under the brightfield microscope which showed darker organoids with rougher edges following Thioridazine drug treatment. (B) Cell viability was measured as the percentage of ATP released in treated organoids relative to untreated organoids across increasing drug concentrations. The datapoints represent the average from two experimental repeats(n=2 independent experiments) conducted with 5 biological replicates per dose (n=5 organoids per dose). Day 230 organoids were treated for 24 or 72 hours with the indicated range of compounds concentrations.

A

	Vehicle	Drug (IC50)
0 hrs		
72 hrs		

B



Overview of cell types in dry AMD

AMD is an ophthalmologic disorder that causes irreversible central vision loss or, in the worst cases, blindness[1]. AMD is the predominant cause of vision loss after the age of 50 years; prevalence is about 25% for people older than 75. Currently affecting 200 million people worldwide, it is estimated that AMD will reach nearly 300 million people worldwide in 2040[2].

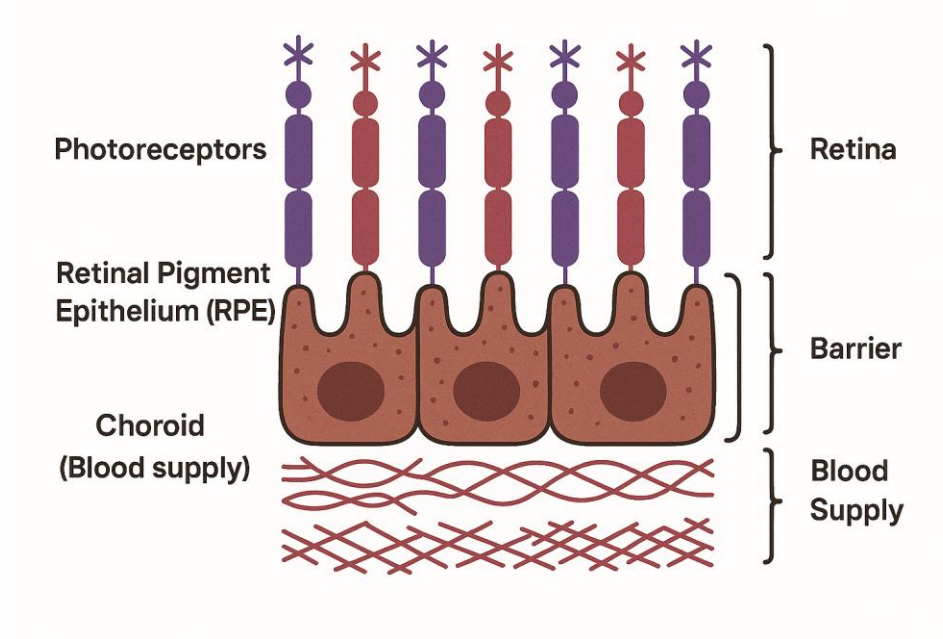
There are two types of AMD

- Dry, that results from degeneration of retinal cells
- Wet (or exudative) that results from abnormal growth of blood vessels in the macula area

While treatments exist for the exudative form, mainly anti-VEGF antibodies (ranibizumab and bevacizumab), the dry form, which affects about 80-90% of individuals with AMD, does not have any satisfactory therapeutic options to prevent vision loss.

In the retina, the photoreceptor cells are in direct contact with the RPE monolayer. The RPE plays an essential role in the maintenance and survival of photoreceptors, including the phagocytosis of cellular debris produced daily by photoreceptors. The cell type primarily affected in AMD is the retinal pigment epithelium (RPE) cell.

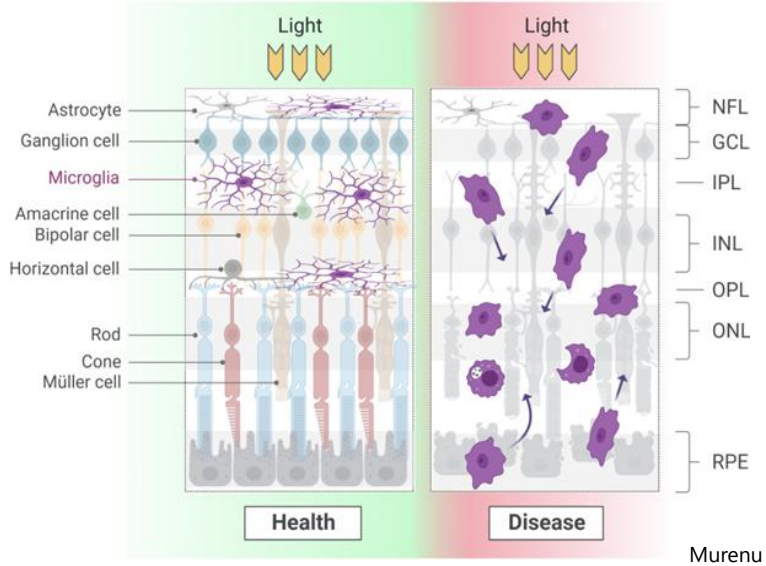
Accumulation of lipofuscin, a by-product of the visual cycle is a hallmark of RPE aging. N-retinylidene-N-retinylethanolamine (A2E) is one of the main component of lipofuscin granules. When this molecule undergoes photoexcitation, it results in the production of reactive oxygen species and cellular damage. This damage in combination with other pathological events, such as complement activation, participate to the death of RPE cells and, therefore, of the photoreceptors that depend on them. Overtime, the progressive atrophy and death of photoreceptors in the macula of the retina can lead to the development of AMD and central vision loss.



The role of microglia in dry AMD degeneration

Microglia, the resident immune cells of the retina, also play a crucial role in the pathogenesis of dry AMD. During AMD progression, microglia become activated in response to oxidative stress, release of pro-inflammatory cytokines, and drusen deposits in the retinal pigment epithelium (RPE).

The role of microglia activation in AMD remains unclear. While it may be initially be protective by contributing to debris clearance, chronic activation leads to a proinflammatory state characterized by the release of proinflammatory cytokines and complement factors, which could contribute to photoreceptor degeneration and geographic atrophy, a hallmark of late-stage dry AMD. Understanding microglial involvement in dry AMD offers novel potential therapeutic targets to modulate their activity and reduce neuroinflammation-associated retinal degeneration.



Murenu et al., 2022

A major challenge in developing effective treatments is the absence of reliable *in vitro* AMD models, which has slowed drug discovery efforts. While primary and immortalized RPE lines have provided valuable insights into RPE functions under normal and pathological conditions, they exhibit limitations for drug discovery, including low amplification potential in primary RPE cells and a lack of maturation in immortalized RPE lines[3].

Induced pluripotent stem cell (iPSC) technology has enabled the generation of unlimited quantities of mature, functional RPE cells and microglia for research. Derivation of iPSC from cell lines from multiple AMD patients is also facilitating the study of how genetic background influences disease progression and treatment efficacy.

To establish a highly relevant *in vitro* AMD model, we have developed a protocol for the large-scale differentiation of multiple iPSC lines into RPE cells. These cells are treated with chronic low doses of N-retinylidene-N-retinylethanolamine (A2E) and exposed to blue light, simulating lipofuscin accumulation, an aging mechanism implicated in AMD[4].

Our results demonstrated that this combination of stressors induces AMD hallmarks in iPSC-derived RPE cells, including increased oxidative stress, complement pathway activation, increased pro-inflammatory cytokine secretion, and RPE atrophy. To further reinforce complement activation, a major pathological mechanism in AMD, A2E and blue light stress can be combined with human serum treatment.

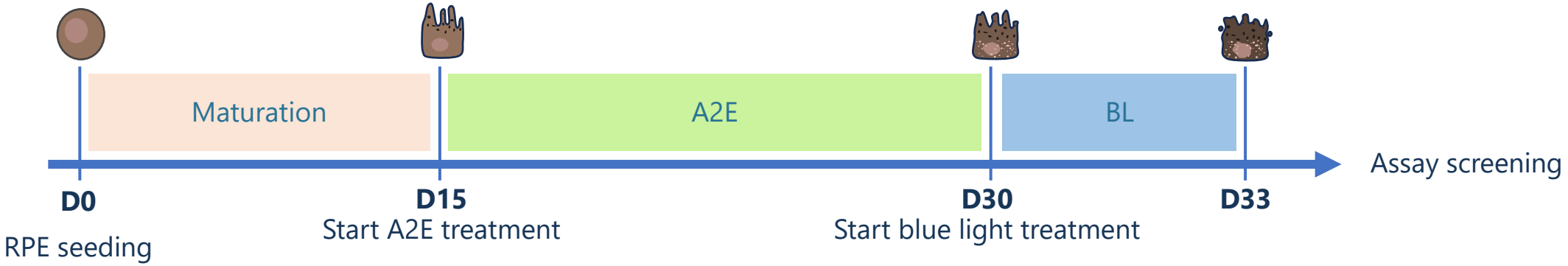
High-content and a high-throughput model for dry AMD

The model focuses on creating a controlled environment that mimics chronic stress that RPE cells experience in AMD. A major factor in this process is the accumulation of lipofuscin, a byproduct of the visual cycle, which increases oxidative stress when exposed to light, especially blue light. This oxidative stress triggers inflammatory responses and activates mechanisms that ultimately participate to RPE cell death and atrophy.

To replicate this stress in the lab, we treat RPE cells with a chronic low dose of A2E followed by photoactivation using a custom-made blue illuminator. This combination of treatments induces the key pathological features of dry AMD, including oxidative stress, inflammation, and cell death.

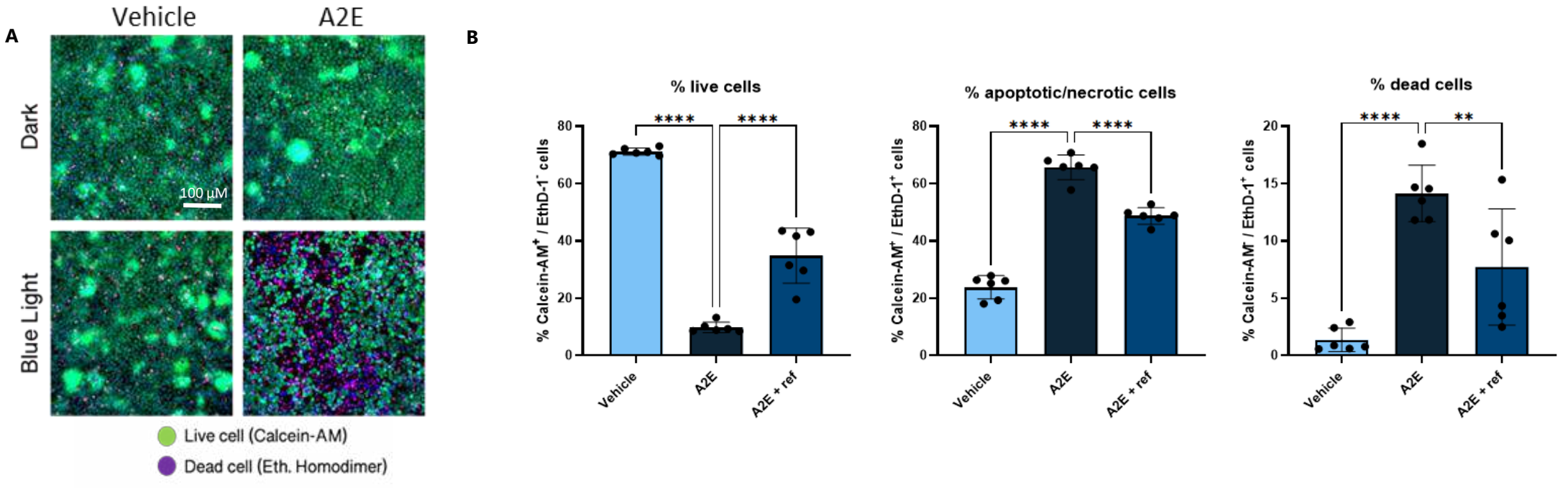
A typical timeline of our model spans 33 days. For the first two weeks, the RPE cells undergo maturation, a crucial step to ensure they develop the proper characteristics and functionality of native RPE cells. During this maturation step, the RPE form tight junctions and display the typical polygonal RPE morphology. After maturation, the cells are exposed to chronic oxidative stress induced by low levels of A2E for two weeks. Then, for three days, blue light is used to activate the accumulated A2E, triggering further cellular damage. We track the effects by assessing cell viability, oxidative stress markers, and inflammatory responses.

We also developed a shorter version of our model for high-throughput screening (HTS). In this version, cell culture is miniaturized and automated using liquid handling systems.



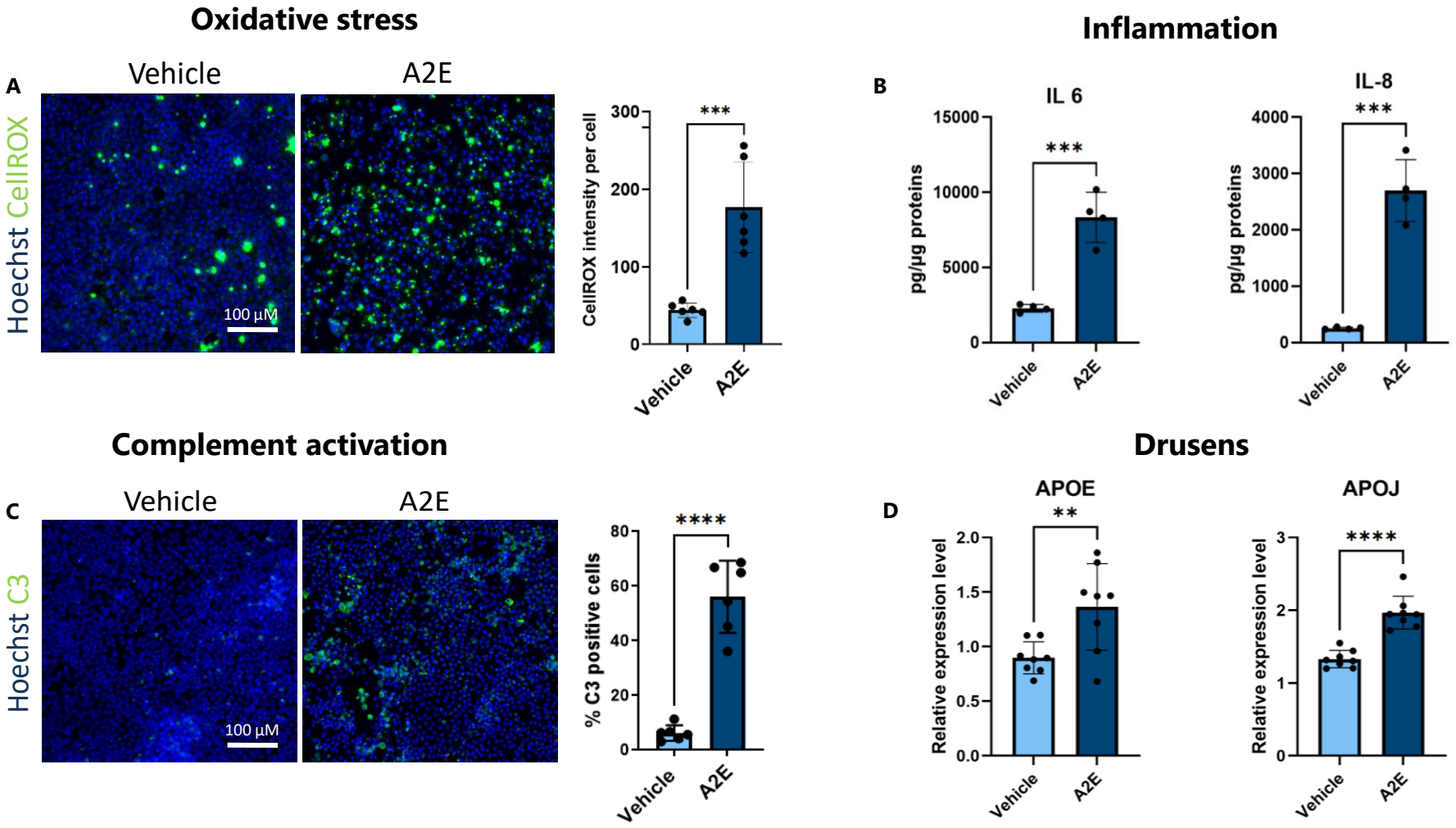
An automated liquid handling system is used for high-throughput screening. During HTS, the Tecan Freedom EVO® platform is used for cell seeding, medium changes, treatments, and cell viability assays.

An *in vitro* model that faithfully recapitulates the main hallmarks of AMD pathophysiology



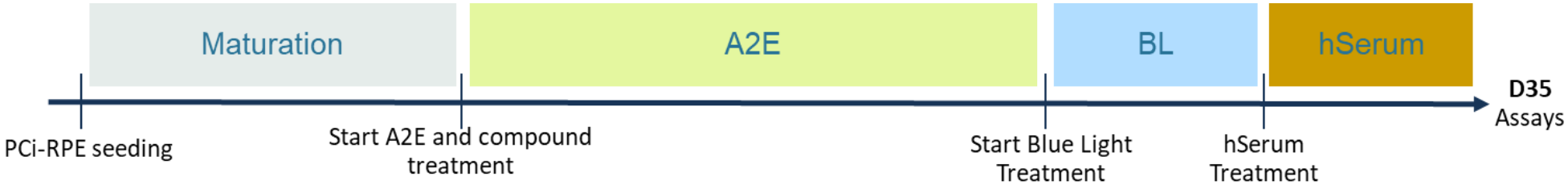
A2E treatment combined with blue light can reproduce RPE atrophy. (A) Images demonstrating enhanced cell death with combination treatment (A2E and blue light) versus vehicle control and blue light. (B) Live/Dead assay demonstrating decreased cell viability in RPE cells treated with A2E and exposed to blue light. Live cells are stained in green using calcein-AM, while dead cells are stained in red using ethidium homodimer (EthD-1). Cells labelled by both calcein-AM and EthD-1 are considered apoptotic or necrotic. A reference compound is included in all our assays to ensure that the stress applied to RPE cells is in a reversible range. Ns: mean +/- SD, $P > 0.05$, $*P \leq 0.05$. $** P \leq 0.01$, $*** P \leq 0.001$, $**** P \leq 0.0001$.

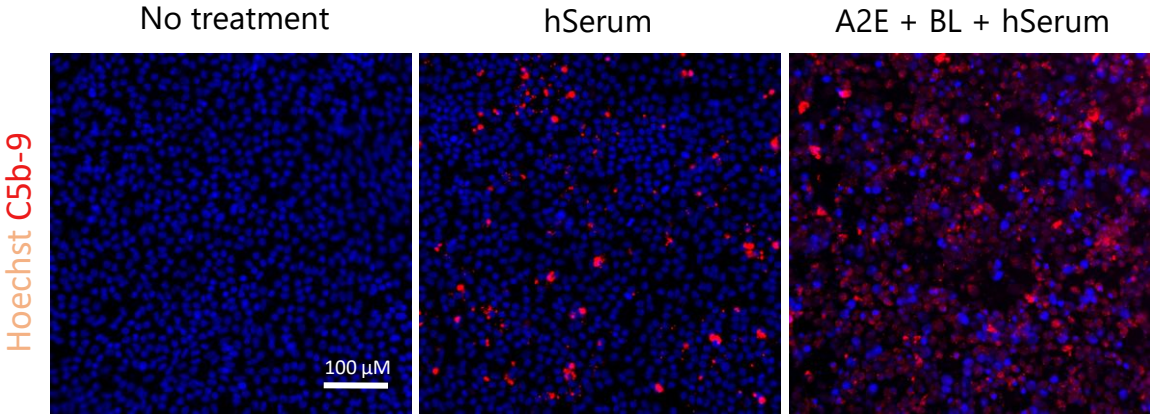
Evolution of AMD markers after A2E and blue light exposure

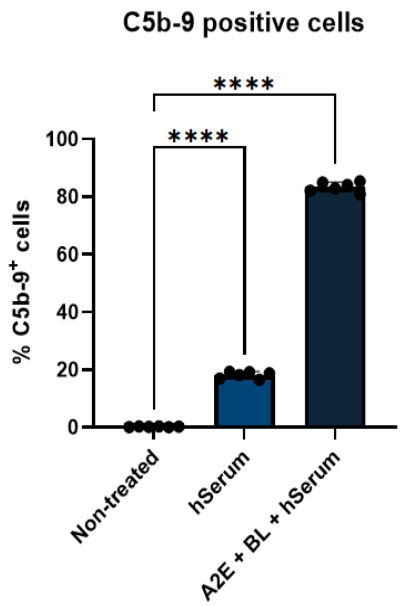


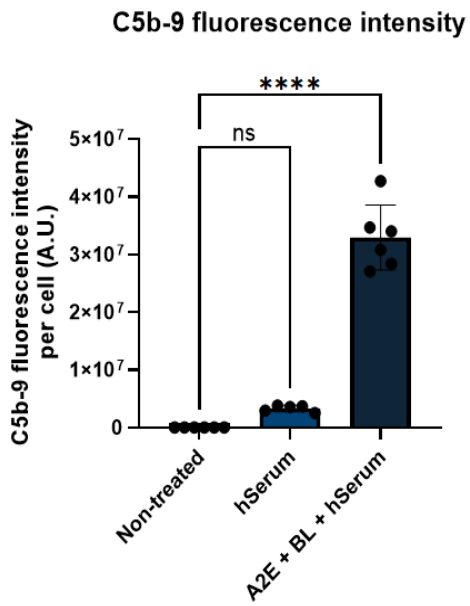
Evolution of important AMD markers in RPE cells treated with A2E and blue light. (A) Intracellular reactive oxygen species levels measured using the CellROX probe. (B) Concentration of IL-6 and IL-8 cytokines in cell culture supernatants quantified by ELISA. (C) Immunostaining for C3 protein. (D) Quantification of APOE and APOJ expression measured by RT-qPCR.

Human serum induces activation of the complement cascade









Complement activation in RPE cells treated with human serum alone or a combination of A2E, blue light and human serum. C5b-9 (Membrane attack complex, MAC) accumulation in RPE cells quantified by immunostaining

High content AMD model for compound testing and endpoint evaluation

High-content screening services offered for dry AMD research assess the health, function, and response of iPSC-derived RPE cells (table 1).

These tools support evaluation of the mechanisms of dry AMD and therapeutic discovery and evaluation workflow. We support clients by streamlining the research process, experimental setup and data analysis.

Each assay in this table measures a specific aspect of RPE cell behaviour or health such as

- VEGF secretion assay (RPE-VEGF)
- Assessment of pigmentation (TYRP1, PMEL17, and Melanin) to provide insights into maturity and functionality
- Cell viability and apoptosis using RPE-LCC (cell viability) and RPE-TUN (apoptosis) assays

Utilising these high-content screening assays provides comprehensive insights into the health, function, and viability of RPE cells, enabling researchers to better understand dry AMD and evaluate new therapeutic strategies.

Area	Name	Method	Endpoint	Reference
Retinal disorders	VEGF (growth factor) secretion	OD reading	VEGF concentration	RPE-VEGF
Retinal disorders	PEDF (growth factor) secretion	OD reading	PEDF concentration	RPE-PEDF
Retinal disorders	Outer retinal barrier resistance	Volt/Ohm meter	Resistance in Ohm/cm^2	RPE-ORB
Retinal disorders	Phagocytosis assay	Bioparticles labeling	Normalized fluorescence	RPE-PHAG
Retinal disorders	mRNA expression of characterized markers	RT-qPCR	Relative expression level	RPE-RNA
Pigmentation	TYRP1 expression	Immunolabeling	Normalized fluorescence	RPE-TYRP1
Pigmentation	Melanin content	OD reading	Melanin concentration	RPE-MEL
Pigmentation	PMEL17 expression	Immunolabeling	Normalized fluorescence	RPE-PMEL17
Oxidative stress	Cellular oxidative stress	CellROX™ staining	Normalized fluorescence	RPE-CRX
Oxidative stress	Mitochondrial oxidative stress	Immunolabeling	Normalized fluorescence	RPE-MSX
Identification	ZO1 expression	Immunolabeling	Cell population purity	RPE-ZO1
Identification	MITF expression	Immunolabeling	Cell population purity	RPE-MITF
Cell viability	Cell viability / survival	Calcein AM – EthD1	Living cells / dead cells	RPE-LCC
Apoptosis	RPE apoptosis	TUNEL staining	Normalized fluorescence	RPE-TUN

Table 1. High-content screening services for dry AMD

High-throughput dry AMD model using iPSC-derived cells

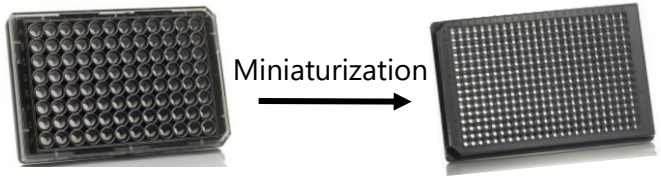
Our HTS model for dry AMD is designed to provide a highly automated and reproducible platform for testing potential therapeutic compounds. Here, our automated liquid handling system is used for every step of the process including cell seeding, medium changes, treatments, and cell viability assays.

In this HTS model cell culture is miniaturized and performed in 384-well plate format. This enable us to work with smaller volumes, enhancing throughput and reducing costs.

The system achieves a Z-factor greater than 0.5, indicating a strong signal-to-noise ratio and robust assay performance. This is complemented by good reproducibility between plates, with a coefficient of variation (CV) of less 10%. This ensures that the data generated from each experiment is reliable and can be confidently used to assess the effects of treatments.

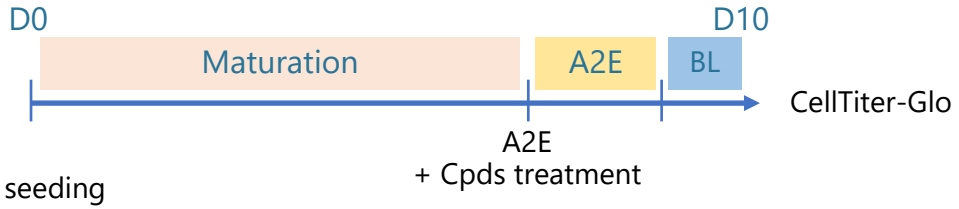
Our reference compound has been shown to efficiently protect RPE cells against stress induced by A2E, a key factor in the progression of dry AMD, further validating the model's ability to identify positive hits with potential therapeutic benefits.

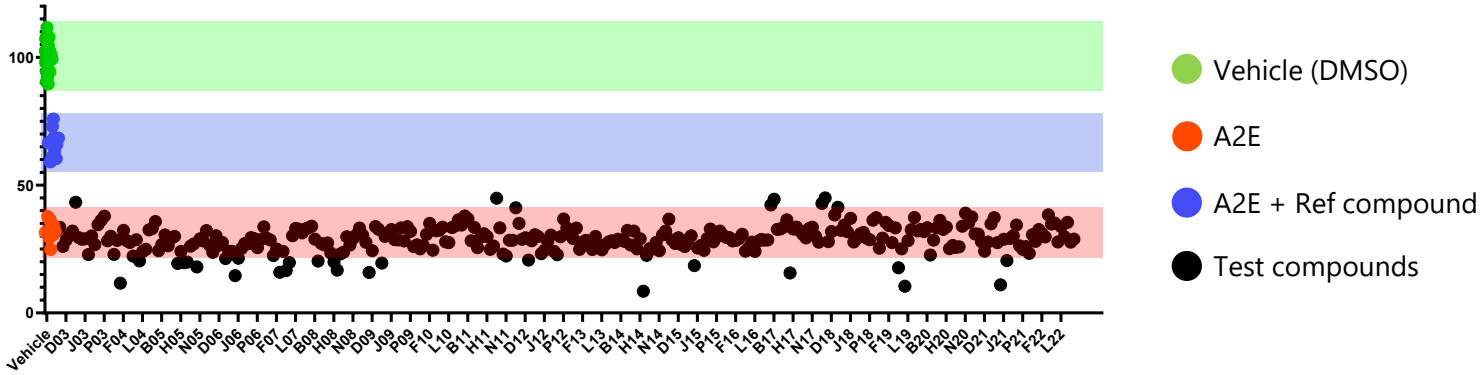
With the capacity to screen up to 6000 compounds per run, our HTS platform offers a scalable solution for testing a wide variety of therapeutic candidates. This high-throughput capability allows for rapid and efficient evaluation, accelerating the discovery of novel treatments for dry AMD.



Miniaturization

RPE seeding



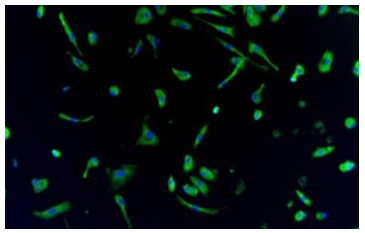


Tecan Freedom EVO® platform

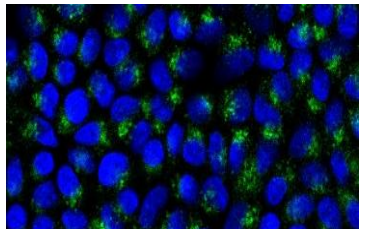
Case study: HALT-AMD project

The future of dry AMD research is moving toward advanced co-culture models that better replicate the complex cellular environment of the human retina. The integration of iPSC-derived RPE cells, microglia, and photoreceptors into 3D *in vitro* disease models is a key step forward. These co-culture systems will enable more accurate modeling of the intricate interactions that occur in the retina, providing researchers with tools to study disease mechanisms in a physiologically relevant environment.

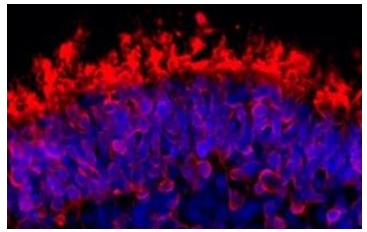
Such models are especially valuable as they can mimic the cellular interactions and microenvironment seen in real-world conditions. For example, by integrating RPE cells, the model replicates the supportive role these cells play in the retina's health. The addition of microglia allows for the study of immune responses and inflammatory processes, while photoreceptors enable researchers to better understand the mechanisms behind vision loss in dry AMD.



Microglia



RPE cells



Photoreceptor

A significant advancement in this area is the ongoing collaboration between Phenocell (Axol Bioscience) and Amarna Therapeutics, which has recently been awarded a prestigious Eurostars grant. This partnership focuses on validating Amarna's Nimvec™ gene therapy platform for dry AMD using Phenocell's cutting-edge 3D co-culture models. The project will leverage iPSC-derived retinal cells to replicate the disease environment and assess the potential of Amarna's gene therapy to inhibit inflammation and promote tissue regeneration in the retina.

This collaboration highlights the growing role of co-culture models in advancing research, offering a glimpse into the potential for more effective therapies for dry AMD. The ability to test gene therapies in these models with greater accuracy and relevance could pave the way for innovative treatments, helping to address one of the leading causes of vision loss globally.

By advancing these models, researchers and biotech companies are making strides toward understanding cellular interactions in dry AMD, accelerating drug discovery, and ultimately moving toward better treatments of this disease.

Human iPSC-derived products for use in advanced *in vitro* models

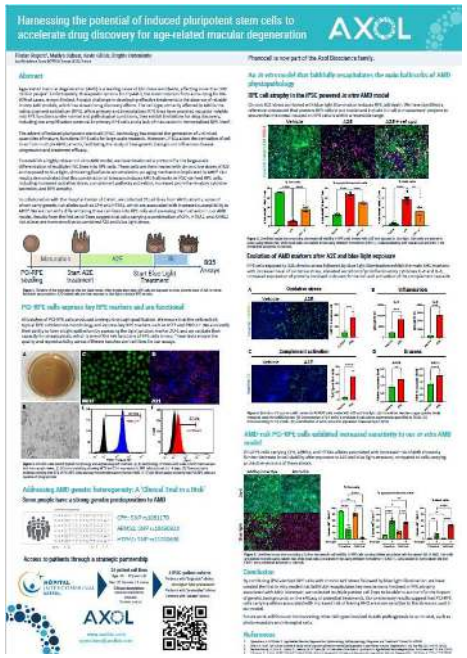
Extensively characterised iPSC-derived RPE cells, microglia and retinal organoids available. We also supply complete media solutions for ease of adoption and extensive user guides. Our scientific team are always available to support new users and workflow development.

Product name	Cells (kit)
axoCells™ human iPSC-derived RPE cells, male donor, aged 30, ≥2 million cells	ax8002 (ax8003)
axoCells™ human iPSC-derived microglia, male donor, aged 40-50, ≥1 million cells	ax0664 (ax0679)
Retinal organoids (d50)	ax8006 (provided as a kit)
Retinal organoids (d150)	ax8007 (provided as a kit)

Media & supplements

Product name	Product code
axoCells™ RPE culture media, 100 ml / 500 ml	ax8000
axoCells™ microglia maintenance medium kit	ax0660
axoCells™ microglia maintenance medium kit	ax0660
axoCells™ SureBond-XF coating, 1 ml	ax0053
axoCells™ SureBond-XF coating, 1 ml	ax0053

Additional resources



Harnessing the potential of induced pluripotent stem cells to accelerate drug discovery for age-related macular degeneration

By combining iPSC-derived RPE cells with chronic A2E stress followed by blue light illumination, we have created the first in vitro model that faithfully recapitulates key mechanisms involved in RPE atrophy associated with AMD. Moreover, we collected multiple patient cell lines to be able to account for the impact of genetic backgrounds on the efficacy of potential treatments. Our preliminary results suggest that PCi-RPE cells carrying alleles associated with increased risk of having AMD are more sensitive to the stressors used in our model.

Future work will focus on incorporating other cell types involved in AMD pathogenesis in our model, such as photoreceptors and microglial cells.

References and further reading

1. Vyawahare, H. & Shinde, P. Age-Related Macular Degeneration: Epidemiology, Pathophysiology, Diagnosis, and Treatment. Cureus 14, e29583.
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3. Bharti, K. et al. Cell culture models to study retinal pigment epithelium-related pathogenesis in age-related macular degeneration. Exp Eye Res 222, 109170 (2022).
4. Moreno-García, A., Kun, A., Calero, O., Medina, M. & Calero, M. An Overview of the Role of Lipofuscin in Age-Related Neurodegeneration. Front Neurosci 12, 464 (2018).
5. Cameron, D. J. et al. HTRA1 Variant Confers Similar Risks to Geographic Atrophy and Neovascular Age-related Macular

Ophthalmology services delivery and scientific team

Collaboration is at the heart of everything we do, ensuring that we bring the best minds together to drive meaningful progress in supporting the industry with physiologically relevant models for retinal diseases. Whether you're seeking advice, services, or support, our team is here ready to help you.

Our team is a dedicated group of experts that range across our sites, in Cambridge, Roslin, Newcastle and Grasse. We bring an innovative approach to advancing research and drug development for retinal diseases, leveraging the diverse expertise and approaches across these sites. Many members of our team are recognised as scientific leaders in their fields. Our customers can leverage their experience to accelerate their programs of work.

When you collaborate with Axol Bioscience, you gain access to a tailored approach that supports your research every step of the way. From the start, we facilitate open communication with our iPSC experts, ensuring that your project is guided by decades of experience.

We offer flexibility in experimental design, leveraging our in-house expertise to customize assays to meet your specific needs. Throughout the project, we maintain rigorous data management and ensure full transparency with detailed reporting and data-sharing meetings.

Our commitment doesn't end at project completion, we continue to offer ongoing support, helping you integrate cells into your systems and maintain an open channel for future collaborations.

Dr Florian Régent
Head of Ophthalmology, Axol Bioscience

AXOL

If you would like to discuss the adoption of iPSC-derived cells or organoids into your workflow, or our specialist services provision, please contact us.

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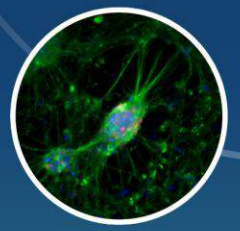

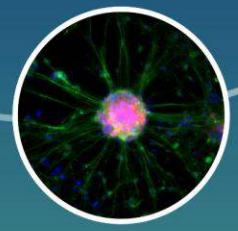

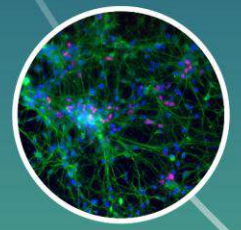

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