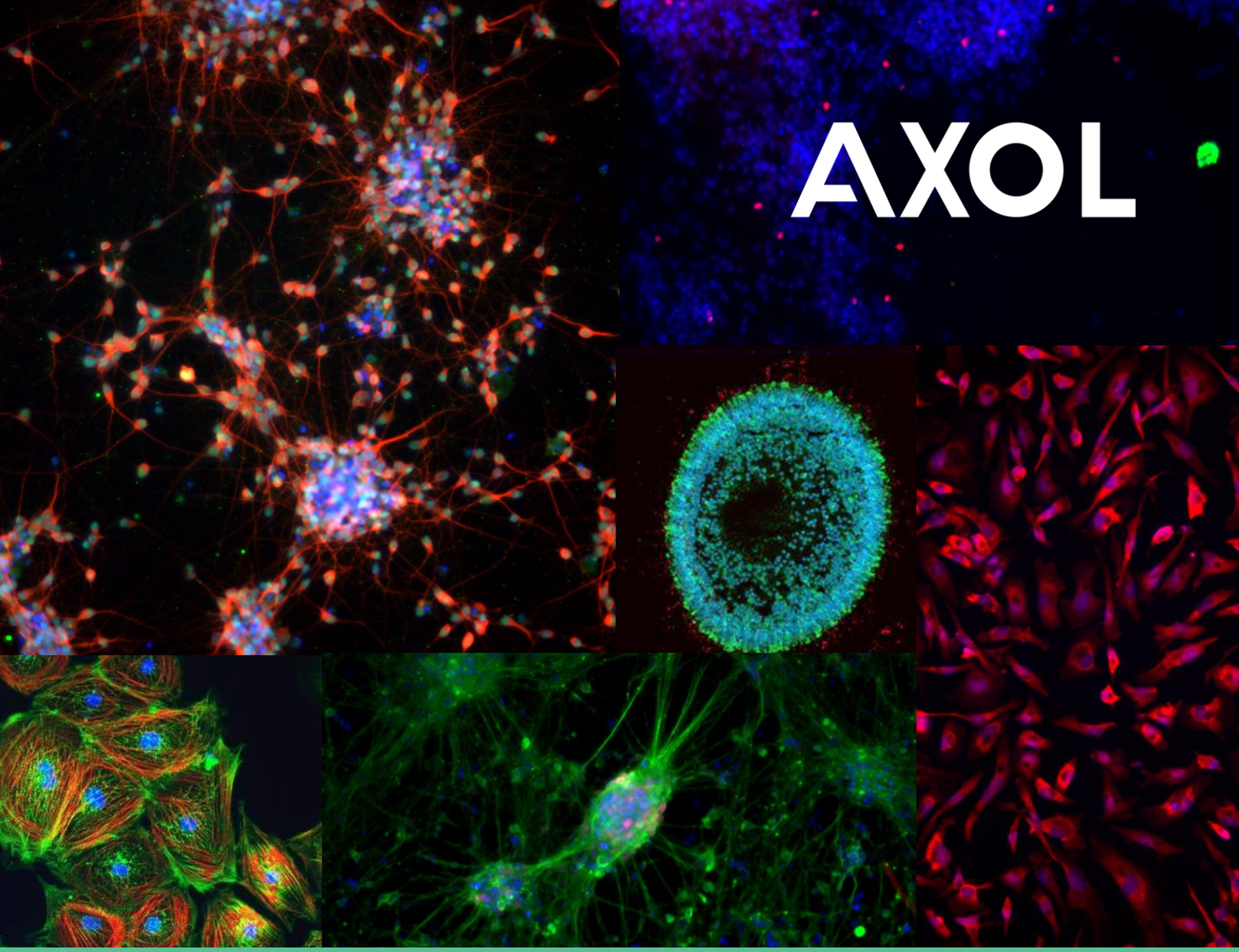


Physiologically relevant human iPSC-derived cell models and specialist services

For drug discovery and translational research

Human iPSC-derived *in vitro* models

Cell provision and specialist services

- Predictive, highly validated disease and safety models
- Scalable differentiation and manufacture of cryopreserved iPSC-derived cells, shipped globally
- Comprehensive discovery and preclinical specialist services, including small molecule, ASO and AAV testing
- Model development, pilot and assay development expertise, including complex co-culture systems
- iPSC production and quality control, reprogramming and gene editing

Extensive patient and unaffected donor iPSC line portfolio

- Library of >100 iPSC lines from unaffected and patient donors representing over twenty indications with particular focus on neurodegenerative disorders and ophthalmology
- Collaboration with patient advocacy groups and PRISM ALS consortia
- Extensive quality control documentation and full commercial use ethical consents

Supporting NAMs (New Approach Methodologies)

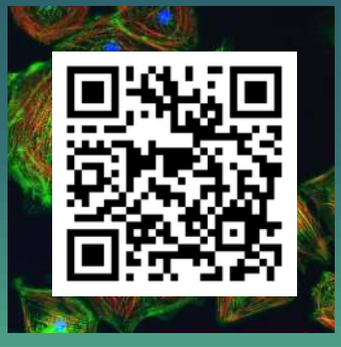
- iPSC-derived cells validated on multiple MPS and advanced *in vitro* analysis platforms
- Support for tech transfer to CROs and in-house facilities

Scientific area focus

Neuroscience

Ophthalmology

Cardiovascular



Dermatology

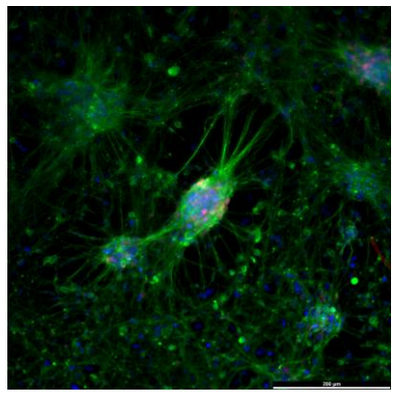
For information, please contact operations@axolbio.com

Neuroscience

Human iPSC-derived models for neurodegeneration, neuroinflammation & preclinical drug discovery

Huntington's disease

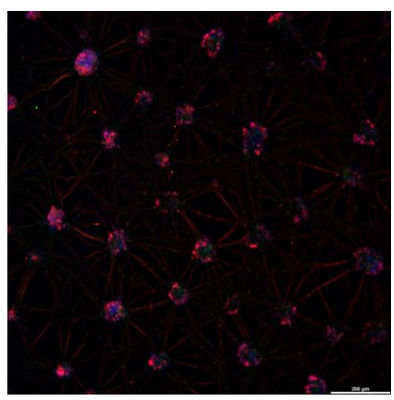
iPSC lines from five HD patients and one asymptomatic carrier. CENSOi019-B line (*HTT*: 14/127 CAG, now CAG143) displays instability in culture and is associated with accelerated disease onset. Striatal neurons derived from this iPSC line provide a model for longitudinal studies of repeat expansion and neurodegeneration and recapitulate key HD phenotypes: CAG instability, neurite pathology, and functional deficits.



Striatal neurons

ALS/MND

Mono- and co-culture models encompassing motor neurons and microglia from multiple ALS genotypes, including C9orf72, SOD1, and TDP-43, consistently recapitulate key disease phenotypes such as neuronal hyperexcitability, altered firing, morphological changes and impaired microglial phagocytosis.
The PRISM ALS consortia is extending access to iPSC-derived cells from 14 sporadic disease lines to accelerate preclinical discovery.



Motor neurons

Alzheimer's disease

A portfolio of sporadic Alzheimer's disease iPSC lines with *APOE* genotypes (E2/E3, E3/E3, E3/E4, E4/E4) differentiated into cortical neurons, astrocytes and microglia for complex cell model development and patient-stratification studies.

Candidate drug testing across multiple iPSC models and neurotoxicity assays

Drug candidate, viral delivery vectors and novel mechanism drug toxicity and activity screening using multi-electrode array (MEA), flow cytometry, cellular imaging, plate-based assays and 'omics.

iPSC lines from patients and unaffected donors

Library of fully quality-controlled iPSC lines from unaffected and patient donors, including ALS, Alzheimer's disease, Huntington's disease, Parkinson's disease, frontotemporal dementia, Friedreich's ataxia, spinocerebellar ataxia, Nasu-Hakola disease and more.

iPSC-derived neurons and neuroinflammatory cells for advanced *in vitro* modeling

Cryopreserved functional cortical excitatory neurons, striatal neurons, cortical inhibitory interneurons, microglia, astrocytes and motor neurons shipped globally. Disease-relevant phenotypes and mono-culture, co-culture and complex model formats.

Specialist services

Services include reprogramming from patient cohort material and gene-editing. Custom differentiation of our iPSC lines, or client lines to multiple neuronal and glial cell types. Preclinical drug discovery services, assay development, target validation, mode-of-action and safety.

Ophthalmology

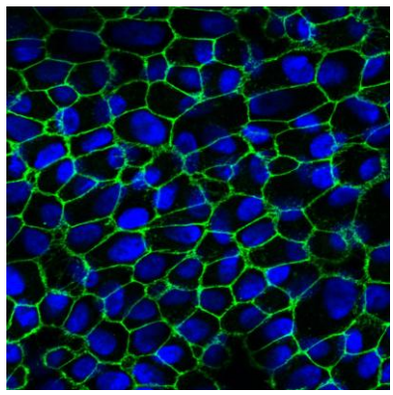
Retinal disease modeling & safety assessment

Dry age-related macular degeneration (AMD)

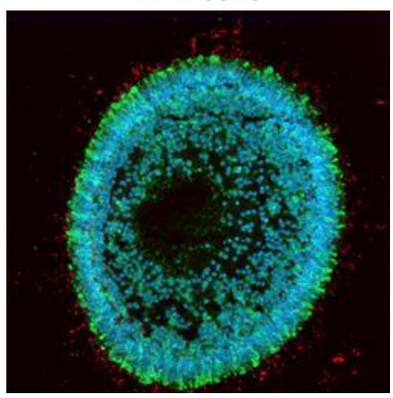
Dry AMD model using iPSC-derived RPE cells exposed to chronic low-dose N-retinylidene-N-retinylethanolamine (A2E) and blue light to simulate lipofuscin photoactivation. The model recapitulates key features of dry AMD pathology, including RPE atrophy, oxidative stress, complement activation, and inflammatory signaling.

Comprehensive high content screening services for assessment of RPE health and function, including growth factor secretion (VEGF, PEDF), barrier integrity (TEER), phagocytosis, pigmentation and maturity markers, oxidative stress, complement activation, cell viability and apoptosis.

Automated 384-well dry AMD screening platform enabling robust and reproducible screening of 6,000 compounds per run for preclinical drug discovery.



RPE cells



Retinal organoid

RPE-microglia co-culture model

iPSC-derived RPE cells and iPSC-derived microglia co-culture for modeling retinal inflammation and the impact of AMD-related stressors. RPE cells displayed increased sensitivity to A2E- and blue light–induced stress when co-cultured with microglia compared to monoculture conditions including microglial proliferation and elevated secretion of inflammatory cytokines.

Retinal organoids for discovery and retinal toxicity

Compound efficacy and toxicity testing using human iPSC-derived retinal organoids, enabling assessment of therapeutic response, mechanism of action. Optimised retinal organoid-based screening systems for retinal toxicity with cell viability (ATP-based assays), morphological assessment, and short- and long-term treatment regimes.

iPSC lines from patients and unaffected donors

Extensive human iPSC line library including multiple retinopathies, Leber congenital amaurosis and Ushers syndrome type 1.

Human iPSC-derived retinal-related cell models for advanced *in vitro* modeling

Functional and well characterized retinal pigment epithelium (RPE) cells, retinal organoids (d60, d150, d180 and custom maturation) and microglia. Provision of scalable custom manufacturing from iPSC library, client lines or post-gene edit.

Specialist services

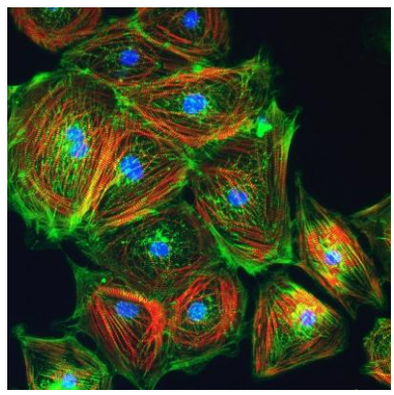
Services include reprogramming from patient cohort material and gene-editing. Custom differentiation of our iPSC lines, or client lines for retinal and ophthalmic research models, including retinal organoid generation. Preclinical drug discovery services, assay development, target validation, mode of action and safety.

Cardiovascular

Cardiovascular disease modeling and cardiac safety assessment

Cardiac safety and disease-relevant models

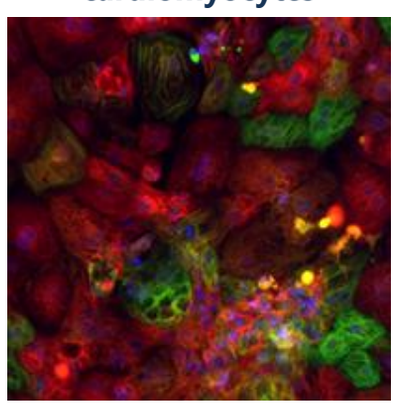
Extensively characterised iPSC-derived ventricular and atrial cardiomyocyte models capture key cardiac functional endpoints with consistent readouts, including action potential dynamics, conduction velocity, contractility, and chamber-specific pharmacology. Ventricular cardiomyocytes are CiPA-validated models supporting consistent assessment of pro-arrhythmic risk and regulatory-relevant cardiac safety profiling. These systems support NAMs (New Approach Methodologies) for more predictive, physiologically relevant testing.



Ventricular cardiomyocytes

Cardiac model optimisation and maturation

For applications requiring enhanced maturity, we provide MyoMax™ cardiac maturation media, which improves functional and structural characteristics of human iPSC-derived ventricular cardiomyocytes, including reduced spontaneous beat rate, improved conduction velocity, shortened action potential duration, increased expression of maturity-associated cardiac genes, and improved sarcomere alignment and expression of cardiac maturity markers.



Atrial cardiomyocytes

In vitro cardiovascular model & assay development with platform integration

Services include complex model development of advanced cardiac models, custom protocol design and optimization, model characterization, phenotypic analysis, treatment titration and endpoint assays, integration with MPS and other platforms and collaborative scale-up for transfer to higher throughput platforms and CRO, or in-house workflows.

Cardiotoxicology and candidate drug testing in human iPSC-derived models

Candidate drug testing and cardiac safety assessment using human iPSC-derived cardiomyocyte models. Services and capabilities include evaluation of drug effects on cardiac electrophysiology, contractility, ion channel function, and chamber-specific pharmacological responses.

iPSC lines from patients and unaffected donors

Fully quality-controlled iPSC lines from male and female donors and we are actively building diversity and additional disease-relevance into the collection.

Chamber- and sex-specific iPSC-derived cardiomyocytes for advanced *in vitro* modeling

Functionally and phenotypically characterised iPSC-derived ventricular, left ventricular, and atrial cardiomyocytes exhibiting distinct chamber-specific ion-channel expression, contractile behaviour, and pharmacological responses. Models support mono-culture formats and scalable assay systems for cardiac safety and efficacy screening.

Specialist services

Services include manufacturing-scale differentiation of iPSCs to cardiac cells, quality control, cryopreservation, media optimisation, and custom workflows using our, client-supplied, or third-party iPSC lines.

Dermatology

Human iPSC-derived skin-relevant models for active compound testing and research

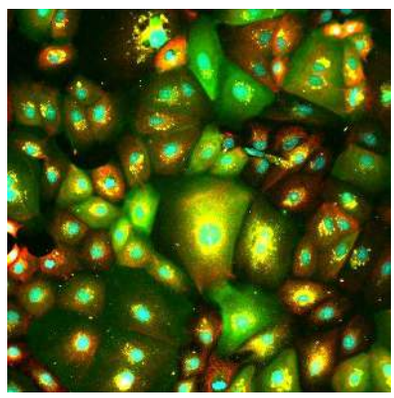
Sensory neurons
Utilized in models of pain and sensation including co-culture, microfluidics devices, organ-on-chip platforms and for *in vitro* disease modeling research. Exhibit functional relevance across multiple assays.

Acne and hyperseborrhea
Human iPSC-derived sebocyte models recapitulate key sebocyte functions, including lipid synthesis, androgen responsiveness, inflammation, and response to C. acnes, arachidonic acid, and cannabinoids. Available in 2D and reconstructed 3D sebaceous gland formats.

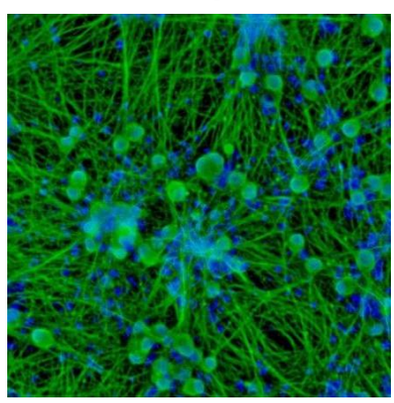
Pigmentation, tanning, and lightening
Human iPSC-derived melanocytes from multiple donor phototypes, support assays for melanogenesis, depigmentation, tanning, toxicity, and proliferation, with demonstrated melanosome production and transfer.

Candidate compound testing
Outsourced efficacy and toxicity testing using sebocyte, melanocyte, and sensory neuron models, with flexible assay design and physiologically relevant endpoints.

High-content screening
Multiparametric readouts including lipid synthesis, pigmentation, cytokine release, receptor activation, neurite outgrowth, cell viability, and morphology across 96-well and scalable screening formats.



Sebocytes



Sensory neurons

iPSC lines from patients and unaffected donors
A diverse, well-characterized human iPSC line library enabling scalable, reproducible manufacturing of dermatology-relevant cell types. Lines support research across cosmetics, dermatology, pigmentation, acne, and neuro-cosmetics.

iPSC-derived skin models for advanced *in vitro* modeling
Functionally validated sebocytes, melanocytes, and sensory neurons, suitable for monolayer, 3D, co-culture, and microfluidic model formats.

Specialist services
Services include manufacturing-scale differentiation of iPSCs, quality control, cryopreservation, media optimisation, and custom workflows using our, client-supplied, or third-party iPSC lines. Automated, reproducible high-throughput screening platforms for dermatology and cosmetic ingredient testing, supporting multi-condition and multi-endpoint studies with consistent physiologically relevant biology.

iPSC line collection

A library of high-quality iPSC-derived lines from unaffected and patient donors, the portfolio is strengthened through strategic collaborations including with StrataStem to deliver stratified sporadic Alzheimer's disease models, the PRISM ALS consortia with LifeArc and ALS TDI to democratise access to diverse, patient-derived ALS models for researchers worldwide and other academic research institutes and patient advocacy groups.

	Mutation	Sex	Age
ALS	C9orf72 >100 G4C2 +/-	F	44
ALS	SOD1 p.D109Y +/-	F	61
ALS	C9orf72 >145 G4C2 +/-	F	64
ALS	C9orf72 >145 G4C2 +/-	M	62
ALS/FTD	TARDBP p.A382T +/-	F	62
AD	APOE E3/E3	F	59
AD	APOE E3/E4	M	60
AD	APOE E3/E4	F	52
AD	APOE E3/E3	M	83
AD	APOE E4/E4	F	69
AD	APOE E3/E3	M	80
AD	APOE E3/E4	M	74
AD	APOE E4/E4	F	76
AD	APOE E3/E4	M	82
AD	APOE E3/E4	F	72
AD	APOE E3/E3	F	79
AD	APOE E3/E3	M	70
AD	APOE E3/E3	M	81
AD	APOE E2/E3	M	73
AD	APOE E3/E4	M	70
AD	APOE E3/E4	M	77
AD	APOE E3/E4	M	72
AD	APOE E2/E3	M	77
AD	APOE E3/E4	F	52
AMD	CFH p.Y402H -/-; ARMS2 p.A69S -/-; C3 p. R102G +/-; APOE E2/E3	F	70
AMD	CFH p. Y402H -/-	M	87
bvFTD	MAPT c.1920+16C>T +/-	M	68
bvFTD	MAPT c.1920+16C>T +/-	M	59
bFTD	MAPT c.1920+16C>T +/-	F	54
bFTD	MAPT c.1920+16C>T +/-	F	42
bFTD	MAPT c.1920+16C>T +/-	M	35
CMT4J	FIG4 pI41T +/-	M	27
DRPLA	ATN1 CAG 13/66 +/-	M	16
DRPLA	ATN1 CAG 17/57 +/-	M	51
Friedreich's ataxia	FXN >75 GAA +/-	M	21
Friedreich's ataxia	FXN 8 GAA +/-	M	44
Friedreich's ataxia	FXN >75 GAA +/-	F	17
Friedreich's ataxia	FXN >66 GAA +/-	M	23
Friedreich's ataxia	FXN >75 GAA +/-	M	34
FTD	PRGN p.R493* +/-	F	31
FTD	PRGN p.C31FS +/-	M	65

	Mutation	Sex	Age
FTDP	VCP p.R155C +/-	F	43
FTDP	VCP p.R191Q +/-	M	42
HD	HTT CAG 18/39 +/-	F	40-50
HD	HTT CAG 17/42 +/-	F	51
HD	HTT CAG 14/127 +/-	F	6
HD	HTT CAG 14/127 +/-	F	7
HD	HTT CAG 28/66 +/-	M	16
HD	HTT CAG 17/38 +/-	M	64
LCA	CRB1 p.K801* +/-; p.C896* +/-	F	4
LCA	CRB1 p.H447Pfs*7 +/-; p.K801* +/-	F	4
Microphthalmia	NM_0012026 c.-7-192G>A	F	39
ML4	MCOLN1 p.F262S +/-	F	36
NHD SCA3	TREM2 p.W50C -/-	F	41
NHD SCA3	TREM2 p.W50C +/-	M	68
NHD SCA3	ATXN3 CAG 14/69 +/-	M	50
NHD SCA3	ATXN3 CAG 26/70 +/-	F	53
PD	PINK1 p.W90Lfsx12 -/-; PRKN pR275W +/-	F	48
PD	PINK1 p.W90Lfsx12 +/-; PRKN p.R275W +/-	M	75
PD	PINK1 p.W9OLfsx12 -/-	F	52
Retinopathy	BEST1 p.N296S +/-	M	69
Retinopathy	BEST1 p.S231R +/-	M	20
Retinopathy	BEST1 p.A243V +/-	F	50
Retinopathy	EFEMP1 p.R345W +/-	F	58
Retinopathy	EFEMP1 p.R345W +/-	M	64
Retinopathy	EFEMP1 p.R345W +/-	M	76
Retinopathy	MERTK p.L623* +/-; p.D723N +/-	F	24
Retinopathy	MERTK p.R722* / multi-exon deletion +/-	M	21
Retinopathy	MERTK p.H721Q -/-	F	18
SBMA	AR >47 CAG	M	66
SCA2	ATXN2 CAG 22/38 +/-	M	41
SCA2	ATXN2 CAG 22/36 +/-	M	58
SCA2	ATXN2 CAG 22/39 +/-	F	N/A
SCA2	ATXN3 CAG 14/75 +/-	F	22
SCA2	CACNA1A CAG 23 +/-	F	63
SCA2	ATXN7 CAG 10/39 +/-	F	61
USH1	MYO7A p.R1240Q +/-; p.K1255RfsTer8 +/-	M	60
USH1	MYO7A p.D75H +/-; p.R2024* +/-	F	N/A

Unaffected	CFH p.Y402H +/+; ARMS2 p.A69S +/+; HTRA1 rs1120063 +/+	M	30
Unaffected	CFH p. Y402H +/+; ARMS2; p.A69S +/+; HTRA1 rs11200638 +/+	M	45
Unaffected	CFH p.Y402 +/+	F	84
Unaffected	MERTK +/- p.H721Q	F	51
Unaffected	ATN1 CAG 12/13 +/+	F	45

axoCells™: physiologically relevant human iPSC-derived cells and kits

	Product name	Cells	Kit
Neuroscience	Human iPSC-derived neural stem cells (unaffected), male donor, aged 40-50, ≥1.5M cells	ax0004	ax5004/ ax3104
	Human iPSC-derived neural stem cells (unaffected), male donor, newborn, ≥1.5M cells	ax0015	ax5115/ ax3115
	Human iPSC-derived motor neurons (unaffected, C9orf72 carrier), male donor, aged 62, ≥2M cells	ax0073	ax0183
	Human iPSC-derived motor neurons (sALS, C9orf72), female donor, aged 64, ≥2M cells	ax0074	ax0184
	Human iPSC-derived motor neurons (unaffected), male donor, aged 40-50, ≥2M cells	ax0076	ax0186
	Human iPSC-derived motor neurons (sALS, TDP-43), female donor, aged 62, ≥2M cells	ax0079	ax0189
	Human iPSC-derived neural stem cells (sAD, ApoE4/E4), female donor, aged 69, ≥1.5M cells	ax0108	ax5108
	Human iPSC-derived neural stem cells (sAD, ApoE3/E3), male donor, aged 80, ≥1.5M cells	ax0109	ax5109
	Human iPSC-derived neural stem cells (sAD, ApoE4/E4), female donor, aged 76, ≥1.5M cells	ax0110	ax5110
	Human iPSC-derived neural stem cells (sAD, ApoE3/E3), female donor, aged 59, ≥1.5M cells	ax0170	ax5170
	Human iPSC-derived neural stem cells (sAD, ApoE3/E4), male donor, aged 60, ≥1.5M cells	ax0174	ax5174
	Human iPSC-derived neural stem cells (sAD, ApoE3/E4), female donor, aged 52, ≥1.5M cells	ax0177	ax5177
	Human iPSC-derived neural stem cells (HD, CAG125), female donor, aged 7, ≥1.5M cells	ax0219	ax3219
	Human iPSC-derived cortical inhibitory neurons (unaffected), male donor, aged 40-5, ≥1M cells	ax0662	
	Human iPSC-derived microglia (unaffected), male donor, aged 40-50, ≥1M cells	ax0664	ax0679
	Human iPSC-derived astrocytes (unaffected), male donor, aged 40-50, ≥1M cells	ax0704	
	Human iPSC-derived motor neurons (sALS, SOD1), female donor, aged 61, ≥2M cells	ax0735	ax01835
Cardiovascular	Human iPSC-derived ventricular cardiomyocytes (unaffected), male donor, aged 74, ≥1M cells	ax2508	ax2500
	Human iPSC-derived atrial cardiomyocytes (unaffected), male donor, aged 74, ≥1M cells	ax2518	ax2510
	Human iPSC-derived ventricular cardiomyocytes (unaffected), female donor, aged 45, ≥1M cells	ax5808	ax5800
	Human iPSC-derived atrial cardiomyocytes (unaffected), female donor, aged 45, ≥1M cells	ax5818	ax5810
Ophthalmology	Human iPSC-derived retinal pigment epithelial cells (unaffected), male donor, aged 30, ≥2M cells, PCi-RPE	ax8002	ax8003
	Human iPSC-derived retinal organoid (unaffected, day 60), media, plate and pipette kit, 20/50/100 organoids		ax8006
	Human iPSC-derived retinal organoid (unaffected, day 150), media, plate and pipette kit, 20/50/100 organoids		ax8007
	Human iPSC-derived retinal organoid (unaffected, day 180), media, plate and pipette kit, 20/50/100 organoids		ax8010
Dermatology	Human iPSC-derived sensory neurons (unaffected), male donor, newborn, >1M cells	ax0055	ax0157/ ax4055
	Human iPSC-derived sensory neurons (unaffected), male donor, newborn, ≥3.2M cells	ax0555	ax4555
	Human iPSC-derived sebocytes (unaffected), male donor, aged 30, ≥2M cells, PCi-SEB_CAU	ax6616	ax6618

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

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