Primary Focus: Blindness and Regeneration

Developing transformative therapies to restore sight and free patients from the fear of deteriorating vision

Our Mission

Our mission for Primary Focus Blindness and Regeneration is to **identify, develop** and **deliver next generation treatments** to restore sight for patients with eye diseases. Utilizing our in-house ophthalmology expertise and regenerative medicine capabilities, we are targeting **transformational changes** in the management of multiple devastating eye diseases.

160+ million worldwide

Background

Vision loss caused by diseases of the eye affects over 160 million people globally[1,2] and can have a devastating long-term impact on quality of life. Many of these diseases have few, or no, effective treatment options. Through **cell** and **gene therapies**, we aim to restore and preserve the critical vision-supporting cells in the eye, offering the potential to **protect against declining vision** and even **restore lost sight**.

Strategic Approach

We are combining innovative cell and gene modalities with a deep understanding of disease biology to establish a robust platform of regenerative medicine:

FOCUS

Delivery of novel therapeutic options for patients suffering from ocular diseases.

ENRICH

Leveraging cell and gene therapies with our ophthalmology R&D and manufacturing capabilities to target key cells central to the pathophysiology of eye diseases. Building a broad, differentiated pipeline across multiple eye diseases.

EXPAND

Through exploration and collaboration with inspiring innovation partners, we are seeking ways to expand our capability to deliver value for patients in diseases of the eye and in other organs.

Our differentiated platform technologies include:

A strong foundation in pluripotent stem cell (PSC)-derived cell therapies in all aspects of the value chain, including development, manufacturing and access capabilities

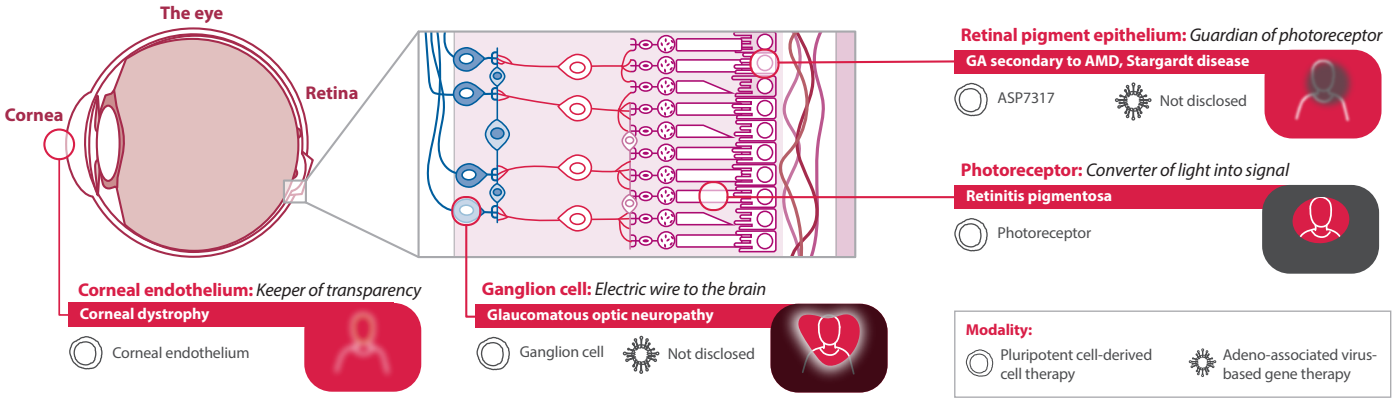
Expertise in AAV-based gene delivery technology

Ophthalmology research and development capability for a variety of modalities

R&D: Research & Development, AAV: Adeno-associated virus

Pipeline

We are exploring innovative modalities to protect and/or restore degenerating cells important to visual functions.



AAV: Adeno-associated virus, GA: Geographic atrophy, AMD: Age-related macular degeneration

Spotlight: Pluripotent stem cell-derived cell therapy

Human PSCs are ideal for regenerative medicine applications as they have the capability to differentiate into a huge number of different types of human cells. Our unique cell therapy platform for 'off the shelf' human PSC-derived allogeneic cell therapies aims to deliver safe and highly-efficacious treatments. This novel approach can create fully-differentiated cells that can then be transplanted into the body to replace damaged, lost or diseased tissue, offering the potential for improved outcomes or even cure.

The lead program, ASP7317, retinal pigment epithelial allogeneic cell transplantation is currently in phase 1 clinical development for geographic atrophy secondary to age-related macular degeneration and Stargardt disease.

Astellas has established an efficient and unique protocol for the production and delivery of high quality ophthalmic cell therapy programs. Through the application of universal donor cell technology, we are pursuing an immune-rejection free allogeneic cell therapy approach that can be used by any patient, with the potential of overcoming the need for immunosuppressive therapies.

PSC: Pluripotent stem cell

Current Status†

Robust and competitive pipeline based on partnership with top-notch entrepreneurial venture and academia:

Compound	Modality/Mechanism	Indication	Current phase	Origin/Partner
ASP7317	RPE cell	Geographic atrophy secondary to AMD, Stargardt disease	Phase 1	OCATA THERAPEUTICS (AIRM) *
Not disclosed	Gene therapy (AAV)	Glaucoma	Preclinical (IND planned in 2023)	Quethera *
Not disclosed	Photoreceptor rescue cell	Retinitis pigmentosa	Discovery	OCATA THERAPEUTICS (AIRM) *
Not disclosed	Ganglion rescue cell	Glaucoma, Optic neuropathy	Discovery	OCATA THERAPEUTICS (AIRM) *
Not disclosed	Corneal endothelial cell	Corneal dystrophy	Discovery	OCATA THERAPEUTICS (AIRM) *
Not disclosed	Universal donor cell (UDC) RPE	Dry AMD, Other macular degeneration	Discovery	Universal Cells *
Not disclosed	Gene therapy (AAV)	Dry AMD, Other macular degeneration	Discovery	University of Pittsburgh

† Accurate as of June 2021. * Acquired (current programs classified as 'in-house')
RPE: Retinal pigment epithelial, AIRM: Astellas Institute for Regenerative Medicine, AMD: Age-related macular degeneration, AAV: Adeno-associated virus, IND: Investigational New Drug application

REFERENCES: 1. Data from the WHO Blindness and Visual Impairment fact sheet. Version 8 Oct 2020. 2. Data from the Foundation Fighting Blindness 2019 Annual Report.

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