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.

**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**

---

[^1]: [World Health Organization](https://www.who.int/news-room/fact-sheets/detail/blindness-and-visual-handicaps)
[^2]: [International Agency for the Prevention of Blindness](https://www.ialp.org/en/vision-loss)
Pipeline
We are exploring innovative modalities to protect and/or restore degenerating cells important to visual functions.

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.

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

<table>
<thead>
<tr>
<th>Program</th>
<th>Modality/Mechanism</th>
<th>Indication</th>
<th>Current phase</th>
<th>Origin/Partner</th>
</tr>
</thead>
<tbody>
<tr>
<td>ASP7317</td>
<td>RPE cell</td>
<td>Geographic atrophy secondary to AMD, Stargardt disease</td>
<td>Phase 1</td>
<td>Astellas Pharma</td>
</tr>
<tr>
<td>ASP1015</td>
<td>Gene therapy (AAV)</td>
<td>Glaucoma</td>
<td>Predclinical</td>
<td>quethera</td>
</tr>
<tr>
<td>ASP1819</td>
<td>Photoreceptor rescue cell</td>
<td>Retinitis pigmentosa</td>
<td>Predclinical</td>
<td>Astellas Pharma</td>
</tr>
<tr>
<td>ASP2020</td>
<td>Universal donor cell (UDC)</td>
<td>Dry AMD, Other macular degeneration</td>
<td>Predclinical</td>
<td>Universal Cells</td>
</tr>
<tr>
<td>Not disclosed</td>
<td>Ganglion rescue cell</td>
<td>Glaucoma, Optic neuropathy</td>
<td>Discovery</td>
<td>Astellas Pharma</td>
</tr>
<tr>
<td>Not disclosed</td>
<td>Corneal endothelial cell</td>
<td>Corneal dystrophy</td>
<td>Discovery</td>
<td>Astellas Pharma</td>
</tr>
<tr>
<td>Not disclosed</td>
<td>Vascular progenitor cell</td>
<td>Vascular disease</td>
<td>Discovery</td>
<td>Astellas Pharma</td>
</tr>
<tr>
<td>Not disclosed</td>
<td>Gene therapy (AAV)</td>
<td>Dry AMD, Other macular degeneration</td>
<td>Discovery</td>
<td>University of Pittsburgh</td>
</tr>
</tbody>
</table>

† Accurate as of Aug 2022. * Acquired (current programs classified as ‘in-house’)
RPE: Retinal pigment epithelial, AMD: Age-related macular degeneration, AAV: Adeno-associated virus, IND: Investigational New Drug application


In this material, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas Pharma. These statements are based on management’s current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties. Information about pharmaceutical products (including products currently in development) which is included in this material is not intended to constitute an advertisement or medical advice.