Primary Focus Candidate: Immune Homeostasis

Developing safe and potentially curative therapies for patients with immune-related diseases

Our Mission

Our mission for Primary Focus Candidate Immune Homeostasis is to deliver safe and potentially curative therapies for patients with immune-related diseases by specifically targeting autoreactive immune cells without impacting the body’s overall immune system. Our aim is to develop innovative therapeutics to restore immune system equilibrium in patients whose immune system has become dysregulated due to autoimmune disease, and potentially offer treatment or a cure for a wider range of autoimmune diseases.

Background

Current treatments for autoimmune diseases include general immunosuppressants and do not specifically target autoreactive immune cells. This broad approach to immune suppression can lead to debilitating and sometimes life-threatening side effects and increase susceptibility to infection. By specifically suppressing disease-related immune response, we have the potential to create new, highly targeted and safer treatment options.

Strategic Approach

We are establishing competitive and innovative modalities that can restore immune homeostasis by leveraging our Immunology R&D experience and cell therapy capabilities:

FOCUS

Focusing on the development of human hemangioblast-derived mesenchymal stem cells which have the potential to be recruited to the site of inflammation and stop the autoreactive inflammatory cascade.

ENRICH

Leveraging our regenerative medicine and gene-editing expertise at AIRM and Universal Cells to develop novel immunoregulatory cell therapies.

EXPAND

Engaging continually with the scientific community to expand our pipeline through partnering, collaboration and acquisition, e.g. collaboration with Pandion.

Our versatile platform technologies include:

Gene editing technology to enhance the immunomodulatory activity and increase disease specificity

Pluripotent stem cell derived immunoregulatory cell therapy for autoimmune diseases

Innovative technology that can induce endogenous immunoregulatory cells, e.g. tissue-specific immune regulation by targeted immunotherapy

R&D: Research & Development, AIRM: Astellas Institute for Regenerative Medicine
Pipeline

We are exploring innovative technologies and modalities that can eliminate disease-specific immune response and induce immune tolerance.

Spotlight: Pandion Therapeutics, Inc.

Astellas collaborates with Pandion Therapeutics, Inc. to investigate potential immunomodulators for the treatment of type 1 diabetes and other autoimmune diseases of the pancreas.

Pandion focuses on developing modular biologics that activate regulatory pathways of the immune system to suppress uncontrolled immune responses. This, combined with tissue-selective ‘tethers’ to focus their effects on target organs, has the potential to directly address abnormal immune responses and modify the disease at the site of immune attack.

Pandion’s Therapeutic Autoimmune reguLatOry proteiN (TALON) drug design platform is devised to directly address a dysregulated immune response and modify the disease where that response occurs. The platform has the potential to be a next-generation autoimmune modality and may also be versatile with application in various other autoimmune diseases.

Current Status†

Through strategic external collaborations and acquisitions, we aim to establish a robust and competitive pipeline:

<table>
<thead>
<tr>
<th>Compound</th>
<th>Modality/mecchanism</th>
<th>Origin/partner</th>
<th>Target indication</th>
<th>Current stage</th>
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</thead>
<tbody>
<tr>
<td>(Not disclosed)</td>
<td>Hemangioblast-derived MSCs</td>
<td>* OCACA therapeutics*</td>
<td>(Not disclosed yet)</td>
<td>Start P1 FY22</td>
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<tr>
<td>(Not disclosed)</td>
<td>Immunoregulatory cell therapy</td>
<td>* Universal Cells</td>
<td>(Not disclosed yet)</td>
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<tr>
<td>(Not disclosed)</td>
<td>Targeted immunotherapy</td>
<td>** Pandion</td>
<td>Type 1 diabetes</td>
<td></td>
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</tbody>
</table>

MSC: Mesenchymal stem cell, P1: Phase 1.
† Accurate as of December 2020, * Acquired (current programs classified as ‘in-house’), ** Programs developed under joint research.

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