RIKEN Program for Drug Discovery and Medical Technology Platforms

Message

The aim of the RIKEN Program for Drug Discovery and Medical Technology Platforms (DMP) is to contribute to the identification of new treatments for cancer and other diseases by promoting collaboration within RIKEN for the development of innovative new pharmaceuticals and medical technologies. The program is involved in all phases of development, from the discovery of promising targets to the identification of potential lead compounds such as small molecules and antibodies, and the acquisition of intellectual property rights to drugs and technologies that can then be brought to the development phase.

Toshio Goto Program Director (D.Agr.):

Facts

DMP has adopted 8 projects and 40 themes from approximately total 200 research subjects (both internal and external of RIKEN) based on original guidelines since DMP’s foundation in 2010. We are promoting 7 projects and 27 themes as of the end of fiscal 2015.

DMP’s efforts

In drug discovery, we identify/analyze the drug target such as disease-causing proteins, create the seeds through several screenings, optimize the lead through medicinal chemistry, and proceed to preclinical studies. This process cannot be completed by a single research laboratory alone. We need to promote drug discovery research strategically by directing a wide range of technology platforms. DMP has restructured drug discovery research functions in Riken life science research centers to set up several drug discovery technology platforms. We are boosting drug discovery themes/projects by the smooth collaboration of these platforms. We have also identified three research/development areas that enables drug discovery and medical technology, promoted maintenance and systemization of them, and taken actions for the collaboration with external institutions.

Business Development Office

This program is aiming to transfer drug discovery and medical technology themes/projects to companies or medical institutions eventually. We are exploring the outstanding seeds of drug discovery and medical technology nurtured by basic research at RIKEN or collaborating universities and research institutes, and conducting seed identification and lead optimization utilizing drug discovery platforms at RIKEN and other external platforms network. We take charge of making alliances such as joint research or licensing with companies, academic or other institutions for our themes/projects.

We are looking for partners for the drug discovery and medical technology themes and projects at any time. If you are interested in any of these themes and projects, we will introduce the non-confidential information first, followed by the disclosure of confidential information upon completion of a confidentiality agreement.

Several types of alliance are acceptable such as license agreement where we grant a license and relevant information, joint research/agreement where we conduct research and development together with partner, and option license agreement where we grant first refusal right to partner so that partner can evaluate the results of research/development progress before obtaining full license.

Organization (Matrix Management)

Our organization is composed of the matrix management system as shown in the right figure. Program Director and Vice Program Director lead Portfolio Managers (mid-level researchers with experience of research and development in the pharmaceutical industry), Regulatory Manager, Clinical Development Managers and Business Development Office. The team promotes and supports research and development with strong leadership on the mission to realize drug discovery and medical technologies. Together with the Portfolio Managers, the Theme leaders or Project leaders organize strategic teams, including appropriate platform members inside/outside of RIKEN. In this way, we have focused on structuring the management team which enables steady advancement of research and development. This two-top system facilitates optimum planning and implementation.

We suggest following 3 types of exit strategy.

Exit 1 (License / Joint research):
MTA or joint research alliance of a specific target protein or disease area or collaboration at the identification of drug target (seed compound) stage.

Exit 2 (License / Joint research):
Alliance with company at patent application with strong claims. Transfer at the patent application stage which includes development candidates.

Exit 3 (License / Joint research):
Transfer to the pre-clinical or clinical development product stage.

Since DMP’s foundation in 2010, 4 projects exited from among adopted 8 projects. Public information of some themes and projects we are seeking for partners are mentioned on our website.

<table>
<thead>
<tr>
<th>Project</th>
<th>Leader</th>
<th>Disease</th>
<th>Category</th>
<th>Exit Type</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug for Alzheimer</td>
<td>Hachiro Sagimoto</td>
<td>Alzheimer</td>
<td>Low Molecular Compound</td>
<td>EXIT2 (FY2011, Investment by INCJ)</td>
</tr>
<tr>
<td>Antithrombotic Drug without Hemorrhagic Side-effects</td>
<td>Toshiyuki Niyama</td>
<td>Thrombosis</td>
<td>Low Molecular Compound</td>
<td>EXIT3 (FY2012, Expiry of the term of the Joint Research)</td>
</tr>
<tr>
<td>Cancer Treatment by NKT Cell</td>
<td>Masayuki Taniguchi</td>
<td>Lung Cancer</td>
<td>Regenerative Medicine or Cell Therapy</td>
<td>EXIT3 (FY2012, Transfer to medical institution)</td>
</tr>
<tr>
<td>Regenerative Medicine for Retinal Degenerative Disease</td>
<td>Masayuki Nakanishi</td>
<td>ARMD</td>
<td>Regenerative Medicine or Cell Therapy</td>
<td>EXIT3 (FY2013, Transfer to RIKEN venture)</td>
</tr>
</tbody>
</table>