

BUSINESS

Axcelead Chief Says Small-Molecule Drug Targets More than Doubled in Last Few Years; IPO Eyed in 4-5 Years

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Axcelead President Yoshinori Ikeura

Advances in drug discovery technology have made it possible for small-molecule compounds to target proteins that they could not target before, Yoshinori Ikeura, president of Axcelead Drug Discovery Partners, tells Jiho. While many industry observers say targets for small-molecule drugs have all but dried up, the number of such targets has “more than doubled in the last few years,” he stresses.

Technological Innovation

Human genes produce around 20,000 different proteins. Some 3,000 of these proteins are disease-related, while around 3,000 proteins have “pockets” that small molecules can bind to. Up to now, drug makers have targeted the 600-1,500 proteins that share both these characteristics. When people say

targets for small-molecule drugs have dried up, they're referring to these 600-1,500 proteins, Dr Ikeura explained in a recent interview.

In addition to these, however, there are many disease-related proteins whose functions have not been easy to regulate by small molecules because they lack the appropriate pockets. Thanks to technological innovations in recent years, however, these proteins can now be targeted as well, Dr Ikeura said.

One new technology mentioned by Dr Ikeura is "gluing" that uses PROTAC (proteolysis-targeting chimeras) and molecular glues. The idea is to use small molecules to create glues that fasten to both disease-related proteins and molecules in the body that degrade proteins. This makes it possible to draw these molecules to and degrade disease-related proteins that couldn't be targeted before.

Meanwhile, a completely new target for drug discovery has appeared. Around 80% of human DNA is transcribed to RNA, and most of it functions as non-translated RNA. In recent years, mRNA and non-translated RNA have been found to play a variety of roles in the body. In drug discovery research, a process called "splicing" in which only mRNA is cut out has come to the fore and some small-molecular compounds have been found to regulate its function. As a result, RNA is now recognized as an important target for drug discovery alongside protein, which will broaden opportunities for small-molecule drugs, Dr Ikeura said.

"Thanks to a variety of new approaches, the number of proteins we can target has increased significantly. If you include RNA, the number that can be targeted by small molecules has more than doubled in the last few years," Dr Ikeura said. "It might seem like a modality shift from small molecules to antibodies and middle molecules is taking place, but it isn't. Modalities are expanding, and in the years ahead companies will select the best ones for their targets. Small molecules remain important. In fact, their opportunities are expanding," he said.

2nd Annual Profit in a Row; 10 Billion Yen Sales in FY2022

Axcelead was established in 2017 through a spinout of Takeda Pharmaceutical's drug discovery platform business. In April this year, a holding company was created and Axcelead was placed under its umbrella. In the interview, Dr Ikeura revealed that the holding company aims to go public within the next four or five years.

Axcelead posted sales of around 6.5 billion yen and net profits of 388.5 million yen for the fiscal year ended March 2020. Despite being spun out of Takeda only a few years ago, the company has made a profit the last two years in a row. In FY2022, it hopes to see its sales top 10 billion yen.

To date, Axcelead has done business with 88 corporations. Of them, 30% have been drug makers, 24% startups, 23% academia, and 23% other. The company, which has already done projects with customers in the US and Asia, is looking to tap further into overseas markets through agents.

Sixty percent of the company's sales comes from "integrated drug discovery" (IDD), a package of drug discovery research services. The remaining 40% comes from fees for services, a contract service

provided for each clinical study. The company aims to increase sales on the strength of its IDD business in particular.

Rather than depend on its hereditary drug discovery technologies from Takeda, Axcelead will also work to introduce new innovative technologies through strategic partnerships with other companies. The company has already entered partnership agreements in the areas of AI and iPS cell drug discovery, crystallization technology, and transdermal delivery technology. It will enhance its IDD services by combining its own technologies with technologies from a variety of companies.

Efficient Discovery Research

“We need to make drug discovery research, including small-molecule drugs, more efficient in Japan. A lot of companies have redundant drug discovery infrastructures, which is a waste of resources,” Dr Ikeura said. “Companies have prioritized the modalities they’ll focus on. Within the next five years, I predict they’ll work to boost efficiency by increasingly outsourcing their drug discovery research and focusing on their remaining resources internally,” he said.

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