# A NEW HOME FOR GENE THERAPY EXPLORATION

MITSUBISHI TANABE PHARMA CORPORATION has invested in cutting-edge gene therapy projects for critical limb ischaemia and haemophilia B, as well as a bold new research facility.

**Mitsubishi Tanabe Pharma Corporation** isn't backing away from the complex field of gene therapies — it's investing in new cutting-edge projects on critical limb ischaemia and haemophilia B, as well as new facilities.

**Critical limb ischaemia**

"Gene therapies provide an entirely new option for patients, particularly for those with very rare and serious diseases," says Rikako Yamauchi, who leads gene therapy research at Mitsubishi Tanabe Pharma.

The company recently partnered with biomed start-up AnGes on the sales and marketing for a gene therapy to treat critical limb ischaemia, a disease that involves the severe blockage of peripheral arteries. These blockages lead to cell death and lower limb amputation in roughly 30 per cent of patients within the first year.

In 1995, Japanese researchers found that Hepatocyte Growth Factor (HGF), a protein that helps liver cells grow, also promotes blood vessel growth. The new gene therapy can deliver genes encoding HGF to regenerate blood vessels in limbs with blocked arteries.

There is now the possibility of acquiring conditional, time-limited approval for regenerative medicines such as this in Japan. Introduced in 2014, a special regime allows cell and gene therapies aiming to treat serious diseases to go to market. Official approval is granted if the drug's efficacy can be verified within five years.

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**Open innovation hub**

Mitsubishi Tanabe Pharma has already pioneered new treatments in four priority areas: autoimmune diseases, central nervous system diseases, diabetes, and vaccines. Now it's committing time and resources to expand into regenerative medicine.

To accelerate its in-house research on gene therapies, the company recently decided to shut down a research centre at Toda, Saitama, and unveiled a brand-new, collaborative research facility at Shonan Health Innovation Park (iPark) in Kanagawa. The hub houses various start-ups, pharmaceutical companies, academic groups, hospitals, medical device companies, IT companies, government entities, venture capital funding providers, and genomics and data researchers. It's hoped this set-up will help catalyze the free exchange of ideas.

"Our San Diego facilities have already run innovative projects in an open environment. In a way, we're replicating that here in Japan," says Yoshiharu Hayashi, an executive officer who oversees drug research and development in the company.

"Some colleagues from other pharmaceutical companies do tell me that this was a bold, and perhaps even risky, move," he adds. "But therapeutic methods are diversifying and becoming more complex. We thought that we needed to throw ourselves into a buzzing environment like Shonan iPark, and to stay at the forefront of rapidly evolving technologies."

**Treating haemophilia B**

Major projects are already underway at Shonan iPark. "Our main targets right now are gene therapies for the many diseases caused by a single gene," says Yamauchi.

One goal is to develop a gene therapy for haemophilia B, the minor subtype of the two major types of haemophilia. Haemophilia B is an inherited disorder that results from genetic mutations in the gene that provides instructions for producing blood clotting factor IX. Low levels or faulty versions of this protein mean that patients aren't able to stop bleeding from their injuries. Research into gene therapy options to treat this disease focus on using the adeno-associated virus (AAV) as a vehicle to carry genes producing blood coagulation factors.

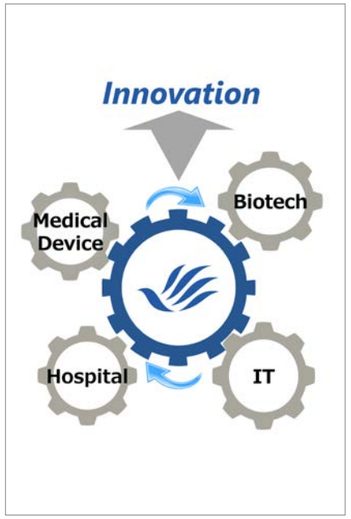
Yamauchi explains that this gene therapy could free patients from the burden of regular intravenous injections of a coagulation factor concentrate, which is the current conventional treatment. "When gene therapy for haemophilia B becomes available, a single administration should be enough to treat the disease. Some patients will also find that other therapies are not suitable for them due to various physical factors; we're planning to make tweaks to the AAV vectors so that the drug works for those patients as well."

Much has been made of the fact that gene therapies can sometimes cost huge amounts due to high manufacturing costs, and Yamauchi does acknowledge that the cost is a potential business risk. "However, when taking into account the lifetime cost, it will mostly likely be a good investment, both for drug developers and patients, whose treatment costs would have been continual," she explains.

The company aims to start clinical trials in collaboration with Jichi Medical University within the next few years. In the longer term, they "plan to apply insights from this study to other gene therapies, especially for neurological diseases in which the company has a wealth of experience," says Hayashi.

"It's definitely not easy achieving results with research. But this is about bringing solid solutions to patients with rare diseases," says Hayashi. "I think that the research in Shonan iPark is going to be extremely rewarding." ■

A new facility at Kanagawa's Shonan Health Innovation Park will drive innovation.




Head of gene therapy research, Rikako Yamauchi (not pictured), says an open research environment produces robust results.

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