Improving the lives of patients with rare diseases.

Looking Beyond Today's Treatments to Patients' Futures.

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Since only a small number of patients have rare diseases, it is difficult to research and develop therapeutic drugs, and there is little social awareness of the issue.
To deliver drugs for rare diseases (orphan drugs) to patients in Japan, CMIC Group established OrphanPacific in 2012. Megumi Hara, the president and representative director of OrphanPacific, explains CMIC Group's efforts regarding rare diseases.

6,000-8,000 Rare Diseases Worldwide: Impacting 350 Million Lives, Creating Challenges in Drug Development with Limited Patients per Disease.

Rare diseases are conditions that affect a very small number of patients, and many of them are difficult to treat, which is why they are also referred to as rare and intractable diseases. It is estimated that there are between 6,000 to 8,000 types of rare diseases worldwide, affecting 350 million patients, making it a significant societal challenge.

To be designated as an orphan drug in Japan, the number of patients has to be approximately less than 50,000, which is why diseases with 50,000 or fewer patients are referred to as rare diseases. The number of patients per disease can range from just a few individuals to more than 10,000, but the small number of patients and limited information make it difficult to invest in drug development, thereby hindering research into diagnosis and treatment.

Moreover, even when a diagnosis is confirmed, there is a lack of accurate and easy-to-understand information about these diseases available to the general public, which also presents a challenge.

Criteria for Designating Orphan Drugs

(1) The number of patients who may use the drug should be less than 50,000 in Japan. (in the U.S., less than 200,000).
(2) The drug should be indicated for the treatment of serious diseases, including difficult-to-treat diseases.
(3) There is a high medical need.
(4) There is no appropriate alternative drug or treatment.
(5) High efficacy or safety is expected compared with existing products.
(6) The drug has a high possibility of development.
(7) The development costs are not expected to be recovered from sales (in the U.S.).

(Referenced from the Japan Pharmaceutical Manufacturers Association website, translated by CMIC.)

OrphanPacific established to address “Drug Loss and Drug Lag” in rare diseases in Japan.

In 2010, CMIC Group began its work on orphan drugs. The Ministry of Health, Labour and Welfare, responding to the issue of drugs approved in Europe and the U.S. not yet being approved in Japan, held a conference to discuss unapproved and off-label drugs with high medical needs, which led to requests for addressing the 'drug loss/drug lag' issue.

Leveraging its expertise in developing drugs, CMIC Group conducted clinical trials for rare diseases such as urea cycle disorders and acute porphyrias, obtained regulatory approval, and established OrphanPacific in 2012 to deliver these medications to patients in Japan. These activities have strengthened CMIC Group's unique business model, the Pharmaceutical Value Creator (PVC), which provides total support to the value chain of pharmaceutical companies.

In 2023, the Ministry of Health, Labour and Welfare revealed that 56% of drug loss items that have not yet been developed in Japan originate from overseas startup companies, and 47% of those drugs were orphan drugs.*1

When overseas ventures consider establishing operations in Japan, they face many uncertainties regarding future business viability, including the limited development pipeline and the unpredictability of post-marketing regulatory trends, such as the drug pricing system. CMIC Group uses the PVC model to consistently provide support in Japan, from development to post-marketing, enabling foreign drug companies to launch orphan drugs in Japan without establishing a Japanese subsidiary.

By taking on the responsibility of manufacturing and sales, OrphanPacific aims to deliver orphan drugs to patients in Japan as quickly as possible and reduce drug loss.

*1: Ministry of Health, Labour and Welfare’s website: “The Report of the Expert Review Committee on Comprehensive Measures for Achieving a Rapid and Stable Supply of Pharmaceuticals”

Solving Patients' Challenges: Continuously Seeking Solutions.

In order to continuously deliver medications to patients with extremely rare diseases, following the conventional methods of typical pharmaceutical companies makes sustaining the business challenging. This is why at OrphanPacific, we have constantly sought ways to deliver drugs to patients in need while ensuring business sustainability by building new business models. Previously, we advanced our business with the dual focus on long-listed products and orphan drugs. Looking ahead, we are not only handling long-listed products but also focusing on products that contribute to the company's growth, aiming to deliver medications to even more patients.

Supporting the use of drugs in Japan that are already being used in Europe and the U.S.

On the other hand, the challenges patients face is not limited to treatment availability.

One of the challenges is that there is not enough easily understandable information about rare diseases available to the public.

Unlike more common diseases that are widely recognized, rare diseases often suffer from a lack of understanding, making it challenging for patients to receive support from those around them. This can lead to difficulties in working while undergoing treatment and a general sense of unease in their daily lives. To address these difficulties in society, we participate in initiatives such as Rare Disease Day and conduct awareness activities both internally and externally.

Additionally, starting this fiscal year, we have initiated activities to listen to the voices of patients and their families to better understand their needs and incorporate these insights into our business.

We began by conducting internal workshops where all employees considered what we could do to address patients’ unmet needs. Moving forward, we plan to hold regular meetings with patient advocacy groups to continuously explore ways to support patients and their families as they navigate living and working with their conditions.