SMALL PHARMA TAKES A RARE APPROACH TO TREAT NEGLECTED DISEASES

Nobelpharma is a niche Japanese pharmaceutical company with **A MISSION TO DELIVER TREATMENTS FOR RARE DISEASES**.

Pharmaceutical research and development activities are hugely expensive, and many drug candidates (even some shown to be effective) don't make it to market because their sales will be too small – often because they treat a neglected disease. Japanese pharmaceutical company, Nobelpharma, bases its business on finding ways to deliver these much-needed treatments.

"Nobelpharma's core mission is about making a positive contribution to society by producing essential medicines and medical devices that have been neglected and which other companies do not pursue," says Arata Tabata, the company's chief operating officer and head of its vaccine business division.

The company's activities range from late-stage development, obtaining approvals and delivering 'orphan drugs' — medicines that would be unprofitable to develop without government assistance — to treat rare diseases, to bringing paediatric and intractable disease drugs to the Japanese market.

RARE DISEASES AND ORPHAN DRUGS

The World Health Organization has estimated that around one in 15 people worldwide could be affected by a rare disease. More than 7,000 rare diseases have been identified, and while more than 70% of these have a genetic origin, the category also includes cancers, some autoimmune diseases and rare infections such as bacterial Legionnaire's disease, 'black fungus' (Mucormycosis), and viral Dengue fever.

DESPITE ITS SMALL CONSUMER BASE, THE COMPANY HAS GROWN STEADILY

Numerous governments have created initiatives that provide incentives to pharmaceutical companies to develop orphan drugs. Examples of these initiatives include the 1983 US Orphan Drug Act, the European Union Regulation No 141/2000 on orphan medicinal products, and Japan's 1993 Orphan Product Development Support Programme, designed to encourage product development.

"In the early days, our founder Jin Shiomura saw opportunities to develop products that were available in some markets outside Japan, but not approved in Japan for rare diseases," Tabata says.

An example of this is Nobelpharma's first successful product launched in 2008, a zinc acetate formulation approved as a treatment for Wilson's disease, a rare genetic disorder that causes a build-up of copper in the body. With just 3,000 people with Wilson's disease in Japan, Nobelpharma relied on patient organisations to recruit subjects for clinical trials to gain approval for this drug, already successfully in use in the US. It was the first of 11 Nobelpharma orphan designated products.

Plasmodium falciparum malaria, kills an estimated 750 children each day in developing countries. Nobelpharma, partnering with Osaka University and an international consortium, is about to complete a second clinical study in Burkina Faso for a malaria vaccine, NPC-SE36, targeted at young children in endemic areas, such as sub-Saharan Africa with support from the Global Health Technology Fund.

RUNNING A TIGHT SHIP

Nobelpharma was founded in 2003 by former Mitsubishi Chemical pharmaceutical executive and current chief executive, Shiomura. Despite its often small potential consumer base, the company has grown steadily since its establishment. Its first three drugs were approved in 2008, and by 2020 the company had achieved 15 new drug approvals by the Ministry of Health, Labour and Welfare (MHLW) and 16.9 billion yen in annual sales.

Tabata says that with around 350 staff, the company runs a tight ship with low fixed costs. He adds that its success comes partly from strategic decisions about product selection, efficient management of clinical trials and regulatory compliance, and then outsourcing certain aspects of the business. "Nobelpharma doesn't have laboratories, or manufacture products," he explains. "It's our focus on later-phase development that speeds up our ability to get to the approval stage," Tabata says.

Today, the core business for the company involves working with academia and securing rights to drugs that have passed early-stage development, then undergoing later-phase development (often including clinical trials) and approvals for patients in Japan or in other markets.

The company's clinical development division is staffed by small teams of highly experienced senior researchers, many recruited from large pharmaceutical companies and most aged over 50. The small teams evaluate early-phase drug trials from around the world to target the drugs to develop for patients in Japan.

Nobelpharma then subcontracts bulk drug supplies and formulation manufacture across multiple trusted contractors in Europe, East Asia, Japan and the United States. The company has a policy of sticking to selling products by itself rather than licensing them out, which has contributed to keeping a healthy bottom line needed to make the business sustainable. Life cycle management is also an important part of its strategy: additional approval for new indications with a large number of patients can lead to higher profitability.

The company has four branches, in the USA, Germany, England and China, in order to make products available to patients who need them. ■

Hayato Orui from Future Code, a non-profit organization, and Toshihiro Horii from Osaka University, visiting Sapone village in Burkina Faso, with Sodiomon B Sirima from the Groupe de Recherche Action en Santé.

Governments have created initiatives to support the development of orphan drugs.

Mucor mould (pictured), also known as black fungus, can cause the rare disease, Mucormycosis.

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