



TAIWAN'S SCIENTIFIC SAVIORS

PHARMAESSENTIA – The Formula for Success

PharmaEssentia Corp. announced in early June that the U.S. Food and Drug Administration had accepted its application for the approval of Ropeginterferon alfa-2b to treat polycythemia vera (PV). If all goes well, it should be approved for marketing early next year.

The announcement came as the COVID-19 virus was continuing its spread in the U.S., so the domestic and international media were spoilt for choice of medical stories -- and this one largely slipped under the radar. But the prospect of a drug to treat PV is very big news indeed for the approximately 160,000 American PV sufferers, because, for them, the condition can be deadly. Initially caused by an over-production of blood cells, the disease can eventually lead to stroke, heart attack and deep vein thrombosis.

Although there are a relatively small number of people in the U.S. afflicted with PV, it is estimated to affect two in every 100,000 of the world's population. PharmaEssentia's dedication to this particular early cause of cancer has the potential to alleviate a considerable amount of suffering and significantly reduce the economic burden imposed by the condition. It also puts PharmaEssentia among an elite band of pharmaceutical companies that have made it their mission to help eliminate the multitude of rare diseases that affect millions of people around the world.

"Our focus is on stunting these rare malignancies, preserving patient well-being and slowing the progression into more aggressive and deadly cancers," says Meredith Manning, PharmaEssentia's U.S. general manager.

Speaking after the FDA's acceptance of the company's Biologics License Application, Manning went on to say,

"WE BELIEVE ROPEGINTERFERON ALFA-2B COULD BECOME AN IMPORTANT NEW THERAPEUTIC TOOL AND LOOK FORWARD TO ENGAGING WITH THE REGULATORS IN OUR EFFORTS TO INTRODUCE THIS OPTION TO THE UNDERSERVED PV COMMUNITY IN THE U.S."

Gaining access to the U.S. market is just the latest step in a journey which will eventually see PharmaEssentia provide relief to PV sufferers worldwide. Early last year, the European Commission gave the company the green light to start marketing Ropeginterferon alfa-2b under the Besremi™ trademark. Developed in conjunction with AOP Orphan Pharmaceuticals, Besremi™ remains the only monotherapy available to PV sufferers in most of Europe.

In addition to conducting pioneering research into the treatment of rare diseases, PharmaEssentia is also one of a select group of companies that have helped establish Taiwan as an engine room of the global biotechnology

industry. From a standing start in 1984, the number of biotechnology companies listed on the Taiwan Stock Exchange has grown to over 100. PharmaEssentia joined their ranks four years ago through an IPO that raised more than \$100 million. It says a great deal about the standing in which PharmaEssentia is now held by the investment community -- and Taiwan as whole -- that the company has raised a further \$50 million since the COVID-19 pandemic swept around an unsuspecting world.

"The response to our private placement has been absolutely unbelievable, considering we are in the middle of a pandemic," says Dr. Ko-Chung Lin, CEO of PharmaEssentia. "And for me, what is even more remarkable is the fact that over half of the funding is coming from foreign investors."

It certainly vindicates his decision to look to Taiwan for the financial backing he needed when he set up PharmaEssentia in 2003. Once he had secured funding from the Taiwan government's National Development Fund, half a dozen private investors soon followed. "Unlike some other sectors like manufacturing where investors can expect to see a return in three to five years, in biotech it can take between 10 to 15 years, at least," he says. "I reasoned that Taiwan would probably be the best place in the world to raise money for such a long-term venture, and I was right. And it clearly still is."

Over the ensuing 17 years, PharmaEssentia has grown into one of the world's top 50 biotechnology companies. Ko-Chung and his colleagues are now looking to raise at least the same amount of funding again from another round of financing.

PharmaEssentia's recent success in raising capital may actually have been helped by the COVID-19 pandemic -- or, more precisely, by Taiwan's notably successful efforts in containing the virus's spread. The fact that such a densely populated island has recorded a mere seven deaths that have been officially attributed to coronavirus

is nothing short of remarkable. It reflects how very much to heart its people took the lessons of the SARS crisis of 2003, when 73 residents lost their lives. The island's success in clamping down on the coronavirus likely boosted investor confidence in the Taiwan business community, subconsciously or not.

The outbreak of SARS actually coincided with Ko-Chung's decision to join a team of high-flying Taiwanese-American business executives and leading biotech and pharmaceuticals enterprises after 20 years working with a number of blue-chip companies, including Biogen. He and his colleagues cut their entrepreneurial teeth in the midst of that earlier crisis. This undoubtedly inspired their confident can-do response to the lockdown that followed the outbreak of the current pandemic, which threatened to deprive the company of its lifeblood -- the development pipeline.

Partly because of the length of time it takes to bring a drug to market and partly driven by the passion and determination of Ko-Chung and his colleagues, research and development has been at the core of PharmaEssentia's operations from day one. Today its R&D center is organized around five centers of excellence, each focused on a different aspect of the product development cycle: analytical science, chemistry, cell culture and engineering, pharmaceutical science and process engineering.

Thanks to the strength and depth of this resource, PharmaEssentia's pipeline will at any one time contain several products in various stages of development, from the pre-investigative (pre-IND) to marketing.



Dr. Ko-Chung Lin CEO, Dr. Ching-Leou Teng Chairperson and CSO, and Jack Hwang General Manager

ITS PRINCIPAL SPECIALIST THERAPEUTIC AREAS COVER DERMATOLOGY, HEMATOLOGY AND INFECTIOUS DISEASES, AS WELL AS ONCOLOGY. EARLIER THIS YEAR, A TEAM OF RESEARCHERS ALSO BEGAN EXPLORING POSSIBLE TREATMENTS FOR COVID-19 SUFFERERS.

Its world-class cGMP biologics facilities in Taichung are engaged full time in this urgent effort, as are its regional headquarters in Tokyo, Beijing, Seoul and Boston, Massachusetts.

With so much at stake, the PharmaEssentia management team was not about to let the minor matter of a worldwide pandemic stop them in their tracks. "We've managed surprisingly well in minimizing the impact to our business," says Ko-Chung, "especially since we have a clinical trial being conducted in China. As the gravity of the situation unfolded, the hospitals where the trials were being conducted were closed to non-essential use and the subjects of those trials were confined to their homes. It became a real challenge to retrieve blood samples and to collect the data," he recalls.

With the help of the Taiwan Center for Drug Evaluation (CDE), PharmaEssentia was able to amend the protocols to conduct the Hepatitis C Genotype 2 Phase 3 trial, and hit deadlines that had been agreed upon long before the word 'coronavirus' made it into daily conversation. "COVID-19 seemed to bring out the best in people and the level of cooperation has

been immense," says Ko-Chung. "Can you believe that we managed to get CDE's consent without having a face-to-face meeting?"

The company also plans to expand ropeginterferon's label to Essential Thrombocythemia (ET) and have already started a Phase 3 clinical trial to investigate the efficacy and safety of ropeginterferon in ET subjects. Ko-Chung now expects development and roll-out to take another two to three years, giving it access to a segment of the healthcare market which he estimates could net the company between \$5 billion and \$10 billion.

Approximately 40% of revenues will be generated in the U.S., prompting PharmaEssentia to put a considerable amount of time and effort into consolidating its position there. Meanwhile, though, it is also actively looking to expand its sphere of operations elsewhere, with both Southeast Asia and South America high on the agenda. The number of partnership proposals the company has received from Latin America is, Ko-Chung says, "in the hundreds." Current circumstances have compelled him to put further exploration on hold until travel restrictions are lifted.

Pursuing a growth strategy that involves both product development and geographical expansion requires financial capital, which PharmaEssentia appears to have no problem raising. It also requires another type of capital, and that is human talent.

"To attract top people in this industry you need to have a good product and one which corresponds to a medical need," says Ko-Chung. PharmaEssentia has both, which has enabled it to recruit from the crème de la crème of the biotech industry, both in its home market and abroad. Committed to the values of corporate social responsibility, the company has shown determination in avoiding layoffs during the pandemic. This policy has won unswerving loyalty from many of its staff, who believe their long-term future lies with PharmaEssentia.

If Ko-Chung is right, then the future is bright indeed and the pandemic just a bump in the road. "We believe that everything will get back to normal in the next couple of years," he says. He and his team will be ready to take PharmaEssentia to the next stage in its development.

PharmaEssentia