Moderna Launches Third Venture Company Elpidera for Rare Diseases

May 12, 2015

Greg Licholai, M.D., hired as president to lead the venture to advance novel messenger RNA therapies

CAMBRIDGE, Mass., May 12, 2015 — Moderna Therapeutics, a pioneer in the development of messenger RNA (mRNA) Therapeutics™, today announced the launch of Elpidera LLC, a new Moderna venture focused exclusively on the advancement of mRNA-based medicines for the treatment of rare diseases. The third in a series of venture companies created by Moderna, Elpidera will be led by Dr. Greg Licholai, an industry leader with vast experience in drug development, rare disease and R&D innovation. Elpidera, which is derived from the Greek word *elpida* meaning “hope,” will leverage Moderna’s mRNA platform to create novel therapies to address diseases in small patient populations with severe unmet medical needs.

Moderna signed a strategic agreement with Alexion in January 2014 allowing Alexion to have 10 product options in rare diseases. Elpidera will advance programs that are independent from the Alexion programs and proprietary to Moderna, while also supporting Alexion in its efforts to leverage the Moderna technology platform through the existing agreement.

“The creation of new venture teams is critical to advancing our decentralized drug development business strategy at Moderna. We are pleased to announce Elpidera as our second venture company launched in 2015 after launching Valera, our venture company focused in infectious diseases,” said Stéphane Bancel, chief executive officer of Moderna. “Ventures are the other leg of our strategy to add to the efforts of our pharma and biotech partners. In total, we are driving more than 50 preclinical mRNA programs across cardiovascular, infectious diseases, oncology, and rare diseases.”

Dr. Greg Licholai will serve as president of Elpidera. He has more than 25 years of experience in biotechnology leadership, in both consulting and entrepreneurial capacities. Prior to Moderna, he was senior vice president for real-world and late-stage research at Quintiles. Before that he was a partner-level consultant at McKinsey & Company, where he led a business line devoted to big data analytics for new drug launches. Dr. Licholai also has extensive experience as an executive at multiple biotechnology companies. He was chief operating officer of Proteostasis, a private biotechnology company, and earlier, was one of the first senior executives at rare disease biotech Amicus Therapeutics, helping put three products into human clinical trials for orphan indications and take the company public in 2007. He was also co-founder of Immunome, a biotech firm focused on a novel antibody platform. Dr. Licholai received his M.D. from Yale Medical School and his M.B.A from Harvard Business School.

“I am excited to join Elpidera and leverage Moderna’s mRNA platform to discover new treatments for patients living with rare, genetic diseases,” said Dr. Licholai. “As it has demonstrated in other therapeutic areas, mRNA Therapeutics hold great potential to serve rare patient populations in a way never before seen. Our approach offers hope in a novel and powerful platform to potentially affect the many thousands of underserved patients suffering from hundreds of diseases with extraordinarily high unmet medical needs. I am pleased to bring my broad array of experience leading innovative biotech companies and background in rare diseases to the team, and am committed to the company’s unique vision to make a real difference – for patients and the industry.”

Mr. Bancel added, “Greg is a superb leader with extensive biotechnology and orphan disease experience, and we are happy to have him on board to generate new medicines and advance the pipeline.”

Moderna is creating first-in-class *in vivo* medicines called mRNA Therapeutics™ designed to directly utilize the body’s natural processes to enable the *in vivo* production of therapeutic proteins. The company has demonstrated the ability to direct the production of both intracellular proteins, which remain within the cells, and secreted proteins, which are released into the bloodstream and act to restore function elsewhere in the body. The range of potential applications make mRNA Therapeutics a powerful tool to address the more than 7,000 rare diseases identified to date, only a small fraction of which are adequately addressed by existing therapies.

In addition to strategic agreements with industry collaborators AstraZeneca (cardiovascular/oncology), Alexion Pharmaceuticals (rare diseases) and Merck (infectious diseases), Moderna is creating a series of venture teams focused in distinct therapeutic areas, enabling the rapid advancement of new mRNA medicines across different modalities and therapeutic areas at an unprecedented pace and scale. This model has enabled Moderna to simultaneously scale up to more than 50 drug development programs across multiple therapeutic areas.

For more information on Moderna, its venture companies and disruptive business model, please visit www.modernatx.com or follow on Twitter @moderna_tx.

About Elpidera LLC, a Moderna Venture

Elpidera LLC, the third venture company formed by Moderna, is focused exclusively on the advancement of messenger RNA (mRNA) Therapeutics™ for the treatment of rare diseases. Elpidera, which is derived from the Greek word *elpida* meaning "hope," is leveraging Moderna’s mRNA platform, an entirely new *in vivo* drug technology that produces human proteins, antibodies and entirely novel protein constructs inside patient cells, which are in turn secreted or active intracellularly. For more information please visit www.modernatx.com/ventures.

About Messenger RNA Therapeutics™

Moderna is creating first-in-class *in vivo* medicines called mRNA Therapeutics™. mRNA Therapeutics™ are designed to directly utilize the body’s natural processes to enable the *in vivo* production of both intracellular proteins, which remain within the cells, and secreted proteins, which are released into the bloodstream and act to restore function elsewhere in the body. This is a quantum change in the way protein therapeutics are traditionally produced and used, and has the potential to transform the treatment of a broad range of diseases.