Ultragenyx Enters into Strategic Partnership with Daiichi Sankyo for Gene Therapy Manufacturing Technology

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Daiichi Sankyo granted non-exclusive license to Ultragenyx HeLa manufacturing platform

Ultragenyx to receive $200 million upfront, including $125 million in cash and $75 million via equity investment

NOVATO, Calif., March 31, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare diseases, today announced a strategic partnership and non-exclusive license and technology access agreement with Daiichi Sankyo Company, Limited for Ultragenyx's proprietary AAV-based gene therapy manufacturing technologies. Ultragenyx's HeLa producer cell line (PCL) platform enables large commercial-scale AAV-based gene therapy product manufacturing that is intended to be highly reproducible, more consistent, and less expensive than other gene therapy manufacturing platforms. In addition, Ultragenyx has developed a proprietary HEK293 transient transfection system for AAV manufacture which is also a subject of the collaboration.

Under the license and technology access agreement, Ultragenyx granted Daiichi Sankyo a non-exclusive license to intellectual property, including know-how and patent applications, with respect to its HeLa PCL and HEK293 transient transfection manufacturing technology platforms for AAV-based gene therapy products. The parties will collaborate closely as part of a technology transfer plan to enable Daiichi Sankyo to use the technologies for its internal gene therapy programs. Ultragenyx retains the exclusive right to use its manufacturing technology for its current target indications and additional indications identified now and in the future.

"This new partnership with Daiichi Sankyo provides further validation of the value of Ultragenyx's gene therapy-related technologies, especially our HeLa producer cell line platform that we believe is the most scalable mammalian cell AAV manufacturing system," said Emil D. Kakkis, MD, PhD, Chief Executive Officer and President of Ultragenyx. "We are encouraged that our proprietary technologies continue to enable the development of numerous programs, both internally and for our partners, to treat patients with serious unmet medical needs."

"We are currently doing discovery research for gene therapy drugs using AAV vectors as one of our focused modalities toward sustained growth beyond achievement of our 2025 vision," said Masayuki Yabuta, PhD, Executive Officer, Head of Biologics Division, Daiichi Sankyo. "In order to provide these drugs to patients in the future, manufacturing technology must be established early. Ultragenyx's proprietary technology is particularly excellent in terms of stable quality, high production efficiency, and ability to accommodate mass production."

Daiichi Sankyo will be responsible for the manufacture, development, and commercialization of products manufactured with the Ultragenyx technology, except that Ultragenyx receives an option to co-develop and co-commercialize rare disease products at the IND stage. Ultragenyx will also provide strategic consultation to Daiichi Sankyo on the development of both AAV-based gene therapy products and other products for rare diseases.

Under the terms of the agreements, Daiichi Sankyo will make an upfront payment of $125 million and will purchase $75 million of Ultragenyx common stock at a price of approximately $60 per share. Daiichi Sankyo will pay an additional $25 million upon completion of the technology transfer of the HeLa PCL and HEK293 platforms as well as single-digit royalties on net sales of products manufactured in either system. Daiichi Sankyo will reimburse Ultragenyx for all costs associated with the transfer of the manufacturing technology.

About Ultragenyx Pharmaceutical Inc.

Ultragenyx is a biopharmaceutical company committed to bringing patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics.

Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the Company's website at www.ultragenyx.com.

About Daiichi Sankyo

Daiichi Sankyo Group is dedicated to the creation and supply of innovative pharmaceutical therapies to improve standards of care and address diversified, unmet medical needs of people globally by leveraging our world-class science and technology. With more than 100 years of scientific expertise and a presence in more than 20 countries, Daiichi Sankyo and its 15,000 employees around the world draw upon a rich legacy of innovation and a robust pipeline of promising new medicines to help people. In addition to a strong portfolio of medicines for cardiovascular diseases, under the Group's 2025 Vision to become a "Global Pharma Innovator with Competitive Advantage in Oncology," Daiichi Sankyo is primarily focused on providing novel therapies in oncology, as well as other research areas centered around rare diseases and immune disorders. For more information, please visit: www.daiichisankyo.com.

Forward-Looking Statements

Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations regarding plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Annual Report filed on Form 10-K with the Securities and Exchange Commission on February 14, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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