

# Pfizer Expands Rare Disease Research with Establishment of Gene Therapy Platform

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Signs Collaboration with Spark Therapeutics to Advance Phase 1/2 Adeno-Associated Virus (AAV) Vector Program in Hemophilia B Launches Gene Therapy Unit Headed by Michael Linden, Ph.D., Leading Researcher from King's College London

Pfizer Inc. (NYSE:PFE) announced today two strategic decisions to expand the company's rare disease research and development activities through the establishment of a gene therapy platform to investigate potential treatments for patients. First is an agreement with Spark Therapeutics to develop SPK-FIX, a program incorporating a bio-engineered AAV vector for the potential treatment of Hemophilia B expected to enter Phase 1/2 clinical trials in the first half of 2015. Additionally, Pfizer has appointed Michael Linden, Ph.D., Professor at King's College London and Director of the University College London Gene Therapy Consortium, who will be with the company for a two-year secondment to lead gene therapy research in the rare disease area.

"The fundamental understanding of the biology of hereditary rare diseases, coupled with advances in the technology to harness disarmed viruses as gene delivery vehicles, provide a ripe opportunity to investigate the next wave of potential life-changing therapies for patients," said Mikael Dolsten, M.D., Ph.D., president of Worldwide Research and Development at Pfizer. "By establishing our gene therapy capabilities, we hope to gain a deeper understanding of the mechanisms that could potentially bring true disease modification for those suffering from devastating hematologic and neuromuscular diseases."

## *Agreement with Spark Therapeutics for Hemophilia Research*

Philadelphia-based Spark Therapeutics and Pfizer will collaborate to progress the clinical program for SPK-FIX, a program incorporating a bio-engineered AAV vector for the potential treatment of hemophilia B. Pfizer has a long-standing commitment to the hemophilia community and has been providing hemophilia products to patients for more than 17 years.

"Pfizer strives to provide meaningful enhancements to the lives of patients with hemophilia, and the agreement with Spark Therapeutics offers an important expansion of Pfizer's commitment to the bleeding disorder community and builds on our leading hemophilia portfolio," said Geno Germano, group president, Global Innovative Pharma Business at Pfizer. "We believe the SPK-FIX program could add to our existing portfolio of hemophilia products and could pioneer a potential new treatment technology for patients with bleeding disorders."

Under the terms of the agreement, Spark will maintain responsibility for clinical development through Phase 1/2 studies. Pfizer will assume responsibility for pivotal studies, any regulatory approvals and potential global commercialization of the product.

## *Establishment of Gene Therapy Research in Pfizer Rare Disease*

Effective December 1, 2014, Professor Michael Linden has joined Pfizer from his current position at King's College London, for a two-year secondment to lead gene therapy research within the company's rare disease research area.

"The establishment of a gene therapy group under the leadership of Professor Linden will help Pfizer explore the potential of this important technology that could possibly benefit patients living with serious diseases," said Kevin Lee, Ph.D., senior vice president and chief scientific officer of Pfizer's Rare Disease Research Unit. "Professor Linden brings to Pfizer his extensive expertise in AAV technology obtained from over 20 years working in the field."

## **Pfizer and Rare Diseases**

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than a decade of experience and a global portfolio of 22 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

## **Pfizer Inc.: Working together for a healthier world®**

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care products. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at [www.pfizer.com](http://www.pfizer.com)

**DISCLOSURE NOTICE:** *The information contained in this release is as of December 3, 2014. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.*

*This release contains forward-looking information about an agreement between Pfizer and Spark Therapeutics to jointly develop SPK-FIX that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Such risks and uncertainties include, among other things, the uncertainties inherent in research and development, including the ability to meet anticipated clinical study commencement and completion dates as well as the possibility of unfavorable study results; whether and when drug applications may be filed in any jurisdictions for any potential product candidates or combination therapies; whether and when any such applications may be approved by regulatory authorities, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of any of such product candidates or combination therapies; and competitive developments.*

*A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2013 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information That May Affect Future Results", as well*

*as in its subsequent reports on Form 8-K, all of which are filed with the SEC and available at [www.sec.gov](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).*

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