



Roche signs agreement with PTC Therapeutics to advance treatment for Spinal Muscular Atrophy (SMA)

Collaboration offers new hope for a potential treatment for the leading genetic cause of death in infants and toddlers

Roche (SIX: RO, ROG; OTCQX: RHHBY), PTC Therapeutics, Inc. (PTC) and the SMA Foundation, announced today a licensing agreement for PTC's Spinal Muscular Atrophy (SMA) programme. SMA is a genetic neuromuscular disorder that causes muscle weakness. One in every 10,000 children born is affected with the disorder, which currently has no effective treatment.

PTC Therapeutics' programme has been developed in partnership with the SMA Foundation, which will remain active in the collaboration. The SMA Foundation was established in 2003 by Loren Eng and Dinakar Singh to accelerate the development of a treatment for SMA.

SMA is caused by a missing or defective SMN1 gene, which results in reduced levels of the survival motor neuron (SMN) protein. The compounds in PTC's research treat the underlying cause of the disorder and demonstrate increases in SMN levels in nervous system, muscles and other tissues in SMA models. SMA is a rare disorder and could be eligible for orphan status by regulatory authorities, thereby potentially reducing the time needed for a drug to reach patients.

Under the terms of the agreement, Roche gains an exclusive worldwide license to PTC's SMA programme, which includes three compounds currently in preclinical development, as well as potential back-up compounds. PTC receives USD30 million as an upfront payment, up to USD460 million upon successful completion of certain development and commercialization milestones, and up to double-digit royalties on commercial sales. Development will be overseen by a joint steering committee comprised of members from Roche, PTC and the SMA Foundation.

"This collaboration brings us one step closer to developing a treatment for a condition that has a profound effect on the lives of many thousands of children and their parents worldwide," said Dinakar Singh, Chairman of the SMA Foundation. "We are very optimistic that, by building on the pioneering efforts of PTC Therapeutics, Roche can help us realise what we have been working so hard to achieve."

Luca Santarelli, Global Head of Roche Neuroscience, said: "We found the science behind this programme very compelling, with the potential to help treat a currently incurable condition. This is the essence of Roche's entire strategy, focused on solid science and high unmet clinical need, and these compounds bolster our rich pipeline in Central Nervous System diseases. As an established partner of Roche, we already have experience with PTC's scientific approach. Together with the involvement of the SMA Foundation, we now have the opportunity to make a significant impact in the treatment of SMA."

"Having been a partner with Roche for several years, we have every confidence that the combination of our own expertise and Roche's considerable capabilities in clinical development, biomarkers and diagnostics will help us maximize the potential for this programme," commented Stuart W. Peltz, Ph.D., President and CEO of PTC. "We are delighted that the SMA Foundation continues to be an active participant in the collaboration as we share a strong commitment to advancing this innovative potential treatment as rapidly as possible."

Roche and PTC announced their first collaboration in September of 2009 for the development of orally bioavailable small molecules utilizing PTC's technology called Gene Expression Modulation by Small-molecules (GEMS™). The SMA programme has been developed by PTC utilizing a different scientific approach than GEMS, called alternative splicing.

About the SMA Foundation

Founded in 2003, the Spinal Muscular Atrophy Foundation is a nonprofit organization dedicated to accelerating progress towards a treatment for Spinal Muscular Atrophy through targeted funding of clinical research and novel drug development efforts. Since its inception, the Foundation has awarded over \$100 million for SMA research. In addition, the Foundation is committed to raising awareness and generating support for increased research efforts in SMA among the leaders of industry and government. For more information, visit the SMA Foundation website at www.smafoundation.org.

About PTC Therapeutics, Inc.

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare genetic disorders, oncology and infectious diseases. PTC has developed

proprietary technologies that it applies in its drug discovery activities and that have served as the basis for collaborations with leading biopharmaceutical companies such as AstraZeneca, Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's website at www.ptcbio.com.

About Roche

Headquartered in Basel, Switzerland, Roche is a leader in research-focused healthcare with combined strengths in pharmaceuticals and diagnostics. Roche is the world's largest biotech company with truly differentiated medicines in oncology, virology, inflammation, metabolism and CNS. Roche is also the world leader in in-vitro diagnostics, tissue-based cancer diagnostics and a pioneer in diabetes management. Roche's personalised healthcare strategy aims at providing medicines and diagnostic tools that enable tangible improvements in the health, quality of life and survival of patients. In 2010, Roche had over 80,000 employees worldwide and invested over 9 billion Swiss francs in R&D. The Group posted sales of 47.5 billion Swiss francs. Genentech, United States, is a wholly owned member of the Roche Group. Roche has a majority stake in Chugai Pharmaceutical, Japan. For more information: www.roche.com

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