Licensing agreement for Spinal Muscular Atrophy (SMA) programme

Roche
PTC Therapeutics

Nov 29 2011
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**Companies:**

- **Roche**
- **PTC Therapeutics**

**Announcement date:** Nov 29 2011

**Deal value, US$m:** 490.0 : sum of upfront and milestone payments

#### Details

- **Announcement date:** Nov 29 2011
- **Industry sectors:** Bigpharma, Bigbiotech, Pharmaceutical, Biotech
- **Therapy areas:** Central Nervous System » Spinal cord, Central Nervous System » Weakness, Musculoskeletal » Spinal muscular atrophy, Biological compounds
- **Technology types:** Small molecules
- **Deal components:** Licensing
- **Stages of development:** Preclinical
- **Geographic focus:** Worldwide

#### Financials

- **Deal value, US$m:** 490.0 : sum of upfront and milestone payments
- **Upfront, US$m:** 30.0 : upfront payment
- **Milestones, US$m:** 460.0 : development and commercialization milestones
- **Royalty rates, %:** n/d : double-digit royalties on commercial sales

#### Press Release

Licensing agreement for PTC's Spinal Muscular Atrophy (SMA) programme.

SMA is a genetic neuromuscular disorder that causes muscle weakness.

PTC Therapeutics' programme has been developed in partnership with the SMA Foundation, which will remain active in the collaboration.

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.
PTC Therapeutics, Inc. Announces Achievement Of Major Milestone In SMA Collaboration

SOUTH PLAINFIELD, N.J., Aug. 8, 2013- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the selection of a development candidate in its spinal muscular atrophy (SMA) collaboration with Roche and the SMA Foundation. The achievement of the milestone triggers a $10 million payment to PTC from Roche.

(Logo: http://photos.prnewswire.com/prnh/20010919/PTCLOGO)

"We are excited about this important achievement in our SMA program", stated Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "This program exemplifies PTC's technology platform and its chemistry and biology expertise applied in an area of great unmet medical need. We are grateful for the exceptional commitment and dedication from our PTC team, as well as from our partners, Roche and the SMA Foundation. The discovery and advancement of a potential new treatment for SMA is a significant milestone for SMA patients and their families."

SMA is caused by a missing or defective SMN1 gene, which results in reduced levels of the survival motor neuron (SMN) protein. It is a genetic neuromuscular disease responsible for the early death of motor neuron cells within the spinal cord leading to muscle atrophy and eventually death in the most severe form of the disease. The oral small molecule compounds in the program target the underlying cause of the disorder by increasing SMN protein levels in the nervous system, muscles, and other tissues. It is estimated that SMA affects approximately 10,000 to 25,000 children and adults in the United States, and that between one in 6,000 and one in 10,000 children are born with this rare disorder.

Luca Santarelli, Head of Neuroscience and Small Molecule Research at Roche commented: "The compelling science behind this project and the highly synergistic alliance between Roche, PTC Therapeutics and the SMA Foundation are the groundwork for this potential therapeutic program. We consider every step towards a treatment option in SMA of high importance for patients and families affected by this devastating and currently incurable condition."

The SMA program was initially developed by PTC Therapeutics in partnership with the SMA Foundation. The SMA Foundation was established in 2003 to accelerate the development of a treatment for SMA. In November 2011, Roche gained an exclusive worldwide license to PTC's SMA program. PTC received a $30 million upfront payment, is entitled to $10 million based upon the selection of a development candidate and may receive up to an additional $450 million upon successful completion of other development and commercialization milestones, plus tiered royalties on worldwide net product sales. Development is overseen by a joint steering committee comprised of members from Roche, PTC and the SMA Foundation.

Karen Chen, Chief Scientific Officer of the SMA Foundation, stated, "We are highly encouraged by the continued progress. Achieving this milestone brings us closer to developing a treatment for thousands of patients with SMA. The SMA Foundation is eager to support and propel treatments forward for patients and look forward to the continued success of our collaboration with Roche and PTC."

ABOUT PTC THERAPEUTICS, INC. PTC is a biopharmaceutical company focused on the discovery and development of orally-administered, proprietary small molecule drugs that target post-transcriptional control processes. While PTC's discovery programs are directed at targets in multiple therapeutic areas, PTC is focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. PTC's internally-discovered pipeline addresses multiple therapeutic areas, including neuromuscular disorders, oncology and infectious diseases. For more information on the company, please visit our website www.ptcbio.com.

FORWARD LOOKING STATEMENTS: Any statements in this press release about future expectations, plans and prospects for the Company, including statements about the receipt of a milestone payment from Roche, the development of and potential market for the Company's SMA product candidate and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" section of the final prospectus for our initial public offering, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views only as of the date of this release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.
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
Filing Data

Not available.

Contract

Not available.