Current Agreements

Dealdoc

Licensing agreement for Spinal Muscular Atrophy (SMA) programme

Roche
PTC Therapeutics

Nov 29 2011

Licensing agreement for Spinal Muscular Atrophy (SMA) programme

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:	Bigbiotech Bigpharma Biotech Pharmaceutical
Brand name:	Evrysdi
Compound name:	Risdiplam
Asset type:	Compound Product
Therapy areas:	Central Nervous System » Spinal cord Central Nervous System » Weakness Musculoskeletal » Spinal muscular atrophy Pediatrics
Technology types:	Biological compounds 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 10 : milestone triggered
Royalty rates, %:	n/d : double-digit royalties on commercial sales

Termsheet

August 2020

PTC Therapeutics achieved a $20 million milestone payment from Roche under its License and Collaboration Agreement.

The milestone payment was triggered by the first commercial sale of Evrysdi (risdiplam) in the U.S.

Evrysdi was approved by the Food and Drug Administration on Aug. 7, for the treatment of spinal muscular atrophy (SMA) in adults and children 2 months of age and older.

August 2013

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.

Press Release

April 2021

PTC Therapeutics Announces Commercial Milestone Payment Following the Recent European Approval of Evrysdi

SOUTH PLAINFIELD, N.J., April 1, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that a $20 million milestone payment was triggered by the first commercial sale of Evrysdi™ (risdiplam) in the European Union under its License and Collaboration Agreement with Roche. Approval for Evrysdi from the European Medicines Agency was received on March 30 for the treatment of spinal muscular atrophy (SMA) in adults and children 2 months and older.

"We are happy to see the rapid adoption of Evrysdi in the EU which speaks to the need for new treatments for SMA patients," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We are delighted that an effective at-home therapy will be available to SMA patients. We recognize that a large proportion of SMA patients in the EU are currently not receiving an approved therapy."

Roche is working closely with reimbursement and assessment bodies in European countries to enable broad and rapid access to SMA patients. Evrysdi is immediately accessible to patients in Germany and will be accessible from early April to patients in France through the cohort Temporary Authorization for Use. Evrysdi has currently been approved in 38 countries and submitted for Health Authority review in a further 33 countries.

Evrysdi is based on PTC science and is commercialized in the United States by Genentech, a member of the Roche Group. Roche led the clinical development of Evrysdi as part of a collaboration with the SMA Foundation and PTC Therapeutics.

About Spinal Muscular Atrophy (SMA) Spinal muscular atrophy (SMA) is a severe, progressive neuromuscular disease that can be fatal. It affects approximately 1 in 10,000 babies and when untreated is the leading genetic cause of infant mortality. SMA is caused by a mutation of the survival motor neuron 1 (SMN1) gene, which leads to a deficiency of SMN protein. This protein is found throughout the body and is essential to the function of nerves that control muscles and movement. Without it, nerve cells cannot function correctly, leading to progressive muscle weakness over time. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

About Evrysdi™ (risdiplam) Evrysdi™ (risdiplam) is a survival motor neuron 2 (SMN2)-directed RNA splicing modifier designed to treat spinal muscular atrophy (SMA) caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi is designed to distribute evenly to all parts of the body, including the central nervous system (CNS). Evrysdi is administered daily at home in liquid form by mouth or feeding tube. The U.S. Food and Drug Administration approved Evrysdi for the treatment of SMA for adults and children 2 months and older on August 7, 2020 and the European Medicines Agency approved Evrysdi on March 30 for the treatment of 5q SMA in patients two months of age and older, with a clinical diagnosis of SMA Type 1, Type 2 or Type 3 or with one to four SMN2 copies. Evrysdi is marketed in the United States by Genentech, a member of the Roche Group.

About the Evrysdi™ (risdiplam) Clinical Studies

FIREFISH (NCT02913482) is an open-label, two-part pivotal clinical trial in infants with Type 1 SMA. Part 1 was a dose-escalation study in 21 infants with the primary objective of assessing the safety profile of risdiplam in infants and determining the dose for Part 2. Part 2 is a pivotal, single-arm study of risdiplam in 41 infants with Type 1 SMA treated for two years followed by an open-label extension. The primary objective of Part 2 was to assess efficacy as measured by the proportion of infants sitting without support after 12 months of treatment, as assessed in the Gross Motor Scale of the Bayley Scales of Infant and Toddler Development - Third Edition (BSID-III) (defined as sitting without support for five seconds). The study met its primary endpoint.

SUNFISH (NCT02908685) is a two part, double-blind, placebo controlled pivotal study in people aged 2 to 25 years with Types 2 or 3 SMA. Part 1 (n=51) determined the dose for the confirmatory Part 2. Part 2 (n=180) evaluated motor function using the Motor Function Measure 32

(MFM-32) scale at 12 months. MFM-32 is a validated scale used to evaluate fine and gross motor function in people with neurological disorders, including SMA. The study met its primary endpoint.

Clinical Trial Safety Data The safety profile of Evrysdi™ was established across FIREFISH and SUNFISH pivotal trials. The most common adverse reactions in later-onset SMA (incidence of at least 10 percent of patients treated with Evrysdi™ and more frequently than control) were fever, diarrhea, and rash. The most common adverse reactions in infantile-onset SMA were similar to those observed in later-onset SMA patients. Additionally, the most common adverse reactions (incidence of at least 10 percent) were upper respiratory tract infection, pneumonia, constipation, and vomiting.

In addition to FIREFISH and SUNFISH, Evrysdi™ is being evaluated in a broad range of people with SMA, including in:

JEWELFISH (NCT03032172) is an open-label exploratory trial designed to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) in people with SMA aged 6 months to 60 years who received other investigational or approved SMA therapies for at least 90 days prior to receiving Evrysdi™. The study has completed recruitment (n=174).

RAINBOWFISH (NCT03779334) is an open-label, single-arm, multi-center study, investigating the efficacy, safety, pharmacokinetics, and pharmacodynamics of risdiplam in babies (~n=25), from birth to 6 weeks old (at first dose), with genetically diagnosed SMA, who are not yet presenting symptoms. The study is currently recruiting.

About PTC Therapeutics, Inc. PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, LinkedIn, Instagram and on Twitter at @PTCBio.

August 2020

PTC Therapeutics Announces First Commercial Milestone Payment Following U.S. FDA Approval of Evrysdi™ (risdiplam)

SOUTH PLAINFIELD, N.J., Aug. 26, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the company achieved a $20 million milestone payment from Roche under its License and Collaboration Agreement. The milestone payment was triggered by the first commercial sale of Evrysdi™ (risdiplam) in the U.S. Evrysdi™ was approved by the Food and Drug Administration (FDA) on Aug. 7, for the treatment of spinal muscular atrophy (SMA) in adults and children 2 months of age and older.

"We are excited that Evrysdi™ is able to reach U.S. SMA patients so quickly following FDA approval," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "This is especially relevant during the COVID-19 global pandemic, given that Evrysdi™ can be delivered directly to patients at home."

Evrysdi™ is a survival motor neuron 2 (SMN2)-directed RNA splicing modifier designed to treat SMA caused by mutations in chromosome 5q that lead to SMN protein deficiency. Evrysdi™ is administered daily at home in liquid form by mouth or feeding tube. Evrysdi™ (risdiplam) is based on PTC science and is commercialized in the United States by Genentech, a member of the Roche Group. Roche led the clinical development of Evrysdi™ as part of a collaboration with the SMA Foundation and PTC Therapeutics.

About Spinal Muscular Atrophy (SMA)

Spinal muscular atrophy (SMA) is a severe, inherited, progressive neuromuscular disease that causes devastating muscle atrophy and disease-related complications. It is the most common genetic cause of infant mortality and one of the most common rare diseases, affecting approximately one in 11,000 babies. SMA leads to the progressive loss of nerve cells in the spinal cord that control muscle movement. Depending on the type of SMA, an individual's physical strength and their ability to walk, eat or breathe can be significantly diminished or lost.

SMA is caused by a mutation in the survival motor neuron 1 (SMN1) gene that results in a deficiency of SMN protein. SMN protein is found throughout the body and increasing evidence suggests SMA is a multi-system disorder and the loss of SMN protein may affect many tissues and cells, which can stop the body from functioning.

About PTC Therapeutics, Inc.

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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8 August 2013

PTC Therapeutics, Inc. Announces Achievement of Major Milestone in SMA Collaboration

8/8/2013 10:00:12 AM

PTC Therapeutics 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 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.

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29 November 2011

Roche (RHHBY) and PTC Therapeutics, Inc. Sign $490 Million Deal for Genetic Disease Program

BASEL, Switzerland, SOUTH PLAINFIELD, N.J., and NEW YORK, Nov. 29, 2011 /PRNewswire/ -- 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

Filing Data

Not available.

Contract

Not available.