

PTC Therapeutics Inc

COMPANY AND PIPELINE OVERVIEW REPORT

Coverage of the company and a summary of the drug pipeline portfolio.

Publication Date: 07-Feb-2013

THOMSON REUTERS

3 Times Square
New York, New York 10036
United States

Tel: +1 646 223 4000

thomsonreuters.com

[Return to Table of Contents](#)



ABOUT COMPANY AND PIPELINE OVERVIEW REPORT

Thomson Reuters provides the knowledge, tools, and expertise to help support drug discovery and development activities, IP portfolio optimization, identification of licensing and partnering opportunities, delivery of successful regulatory submissions, and the ability to keep current with the rapidly-changing pharmaceutical and chemical markets, supporting informed, early decisions.

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information. From drug discovery and development activities to patent reports, the latest deals, and partnering opportunities, *Cortellis* can provide the confidence to make the most informed business decisions, faster. *Cortellis for Competitive Intelligence* provides accurate and validated information on pharmaceutical and biotechnology companies globally, their drug pipelines, deals, patents, and clinical trials, plus breaking industry news and conference coverage. All contained in one simple, highly intuitive research platform.

Cortellis Company and Pipeline Overview reports are the first in a series of reports that track pharmaceutical and biotechnology companies worldwide. Further report offerings planned to follow include: Company Detailed Pipeline and Company Competitive Landscape reports. All *Cortellis for Competitive Intelligence* content is subject to the most comprehensive editorial review process available, conducted by scientists, pharma professionals, regulatory experts, and generics specialists. Featuring timely drug pipeline information expertly uncovered and integrated from over 400 global meetings each year, you'll always be on top of the latest developments.

Chosen by leading life sciences companies, their executives and investors, *Cortellis for Competitive Intelligence* accelerates your deal-making and gives you timely insights on the development landscape.

Discover undiscovered opportunities in drug development and licensing faster with *Thomson Reuters Cortellis™ for Competitive Intelligence*

DISCLAIMER

The information contained in this report is based on sources believed to be correct but Thomson Reuters does not guarantee the accuracy, timeliness, or completeness of this information. Opinions, if any, are those held by the author of any individual report or article at the time of initial publication and do not necessarily reflect the views of Thomson Reuters.

Information in this report on companies is intended for reference use only, and does not constitute a recommendation to buy or sell any particular security or other investment and does not constitute an offer to buy from or sell to any particular investor. Any company or securities mentioned in this report may not be suitable for any particular investor, depending on that investor's financial position and needs.

[Return to Table of Contents](#)



GLOSSARY

Number of Drugs in Active Development

Number of drugs associated with the company or subsidiary that are currently in active development, i.e. the development status for the drug(s) is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Number of Inactive Drugs

Number of drugs associated with the company or subsidiary that are currently classified as inactive, i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

Number of Patents as Owner

Number of patents associated with the company where the company is listed as owner; i.e. the relationship type (or way the patent refers to the company) is: Patent Assignee/Owner, Patent owner (not assignee), Licensee for development and marketing, Licensee – marketing only (Distributor), Patent assignee of family member, Inferred assignee.

Number of Patents as Third Party

Number of patents associated with the company where the company is listed as third party; i.e. the relationship type (or way the patent refers to the company) is: Patent assignee (not owner), Ex-Licensee for development and marketing, Ex-Licensee marketing only (Distributor), Customer of technology, Ex-Customer of technology, Patent opponent or infringer, Affiliate organization of inventor, Owner of underlying technology.

Patents summary table

This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

Number of Deals

A count of deals where the company or one of its subsidiaries is the primary company.

Key Indications

Displays top ten key indications for the company and its subsidiaries based on frequency (indications occurring with high and identical frequency are always included, and this may result in more than ten Key Indications being listed). Includes both indications associated with patents where the company is patent owner and indications associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

Key Target-based Actions

Displays top ten key target-based actions for the company and its subsidiaries based on frequency (actions occurring with high and identical frequency are always included, and this may result in more than ten Key Target-based Actions being listed). Includes both target-based actions associated with patents where the company patent owner and target-based actions associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended. A target-based action is one that is associated with a target.

Key Technologies

Displays top ten key technologies for the company and its subsidiaries based on frequency (technologies occurring with high and identical frequency are always included, and this may result in more than ten Key Technologies being listed). Includes both key technologies associated with patents where the company relationship is patent owner and key technologies associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

[Return to Table of Contents](#)



TABLE OF CONTENTS

Company Overview..... 5

Company Profile..... 6

Product Portfolio Summary..... 7

Product Portfolio Drugs..... 12

[Return to Table of Contents](#)

PTC Therapeutics Inc

COMPANY OVERVIEW

Company Name	PTC Therapeutics Inc
Parent Company Name	PTC Therapeutics Inc
Website	http://www.ptcbio.com/
Country	US
Number of Drugs in Active Development	6
Number of Inactive Drugs	8
Number of Patents as Owner	59
Number of Patents as Third Party	1
Number of Deals	23
Key Indications	Duchenne dystrophy, Cancer, Cystic fibrosis, Genetic disorder, Bacterial infection, Dengue virus infection, HIV infection, Methylmalonic acidemia, Becker muscular dystrophy, Cardiac failure, Hemophilia
Key Target-based Actions	VEGF receptor antagonist, ErbB2 tyrosine kinase receptor inhibitor, Hepatitis C virus protein NS4B inhibitor, IGF gene stimulator, IL-6 antagonist, MSTN gene inhibitor, Polycomb complex protein BMI-1 inhibitor, SMN1 gene modulator, Sarco endoplasmic calcium ATPase 2a stimulator, UTRN gene
Key Technologies	Small molecule therapeutic, Oral formulation, Antibiotic, Gene expression regulation, Oral suspension formulation, Translational trans-acting regulator, Drug screening, Drug combination, Analytical method, Condensational synthesis, Hydrolytic synthesis

COMPANY PROFILE

SUMMARY

PTC Therapeutics Inc, headquartered in South Plainfield, NJ, is a biopharmaceutical company focused on the development of oral small molecule drugs that target post-transcriptional control processes, including for genetic disorders, oncology and infectious diseases.

LICENSING AGREEMENTS

By December 2006, PTC Therapeutics and the Spinal Muscular Atrophy (SMA) Foundation entered research collaboration for the identification and development of therapeutics for spinal muscular atrophy. In May 2009, PTC Therapeutics and the SMA Foundation expanded their research collaboration.

In September 2007, PTC granted Celgene an option to collaborate on the development of orally bioavailable, small-molecules using PTC's GEMS technology, against two oncology targets. Celgene made a \$20 million equity investment in PTC, and if the option is exercised, Celgene would gain exclusive rights to any products and PTC would receive research funding, milestone payments per target and royalties. In September 2009, Celgene exercised its option.

In January 2007, PTC entered a collaboration with Pfizer for the research and development of up to ten target compounds using PTC's gene expression (GEMS) technology. PTC has received \$10 million upfront and could receive a further \$121 in milestones per target, plus research fees. Pfizer would purchase an equity stake of \$10 million in PTC and would receive exclusive worldwide rights to any compounds identified. PTC would receive royalties on any compounds commercialized from the collaboration.

In June 2006, PTC entered into an agreement with CV Therapeutics to research and develop orally bioavailable small molecules for five targets using PTC's GEMS technology. Under the agreement PTC would receive an upfront payment of \$2million and two loans totaling \$8 million. CV would retain the option to license the compounds and would pay PTC royalties and milestone payments up to \$335 million if it commercialized any products arising from the agreement.

In June 2006, PTC agreed to use its gene expression modulation by small molecules technology to identify and develop small-molecule drugs for spinal muscular atrophy using \$1.6 million in funding from The Spinal Muscular Atrophy Foundation. In December 2007, PTC and the SMA expanded the research collaboration. Under the terms of the amended agreement, the SMA agreed to provide an additional \$1.6 million in funding to PTC.

[Return to Table of Contents](#)



EARLY R&D

In September 2009, PTC Therapeutics and Roche entered an exclusive research collaboration and licensing agreement for the development of orally bioavailable small molecules utilizing PTC's Gene Expression Modulation by Small-molecules (GEMS) discovery technology. The companies would jointly select four CNS disease targets to be the initial focus of the collaboration. PTC would receive a \$12 million upfront payment, R&D funding, up to \$239 million in milestone payments per target and up to double digit royalties. Roche would have the option to add four more targets for further indications in exchange for additional cash payments.

By August 2008, PTC had discovery programs in spinal muscular atrophy (SMA), viral infection, fungal infection and other therapeutic areas including cardiovascular, metabolic and CNS disorders.

By December 2005, the company had an anemia and a musculoskeletal program.

In April 2004, PTC identified an enzyme complex underlying the metabolism of tRNA and mRNA.

FINANCIAL

In July 2012, the company closed \$30 million financing round.

In December 2009, the company completed a \$50 million financing round.

In April 2007, PTC withdrew a registration statement it filed with the SEC for an initial public offering (IPO) of its common stock. The company believed it had sufficient capital to meet its planned needs and thus decided to postpone the IPO. In March 2006, PTC filed a registration statement relating to a proposed IPO of shares of its common stock, although this statement had not yet become active. The company stated that all shares would be sold by PTC with JP Morgan Securities Inc to act as co-lead manager and Pacific Growth Equities LLC acting as co-manager.

In November 2005, PTC raised \$26.6 million from a private placement. The funds would be used for clinical development of PTC-124, preclinical oncology and antiviral programs and for drug discovery.

In January 2004, PTC raised \$35 million through a private placement of series E preferred stock. The proceeds would be used to support the clinical development of PTC-124, as well the continued advancement of several programs in lead optimization. In June 2004, PTC raised an additional \$15 million from the series E private placement.

By June 2003, PTC had raised \$56 million.

R&D GRANTS

In July 2007, the NIH awarded PTC a 5-year, \$15.4 million U54 grant for research in Duchenne muscular dystrophy.

In September 2004, PTC was awarded a \$1 million grant by the Parent Project Muscular Dystrophy to identify small molecules for the treatment of Duchenne muscular dystrophy.

In November 2003, PTC was awarded a Phase I SBIR grant from the NIH to identify inhibitors of bacterial ribonuclease P (RNase P) as potential antibiotics.

In May 2003, PTC was awarded a Phase II small business technology transfer grant from the NIAID to develop a virus-cell-based assay using HIV-1 vector systems for the discovery of potential anti-HIV drugs.

In April 2003, PTC was awarded a grant from the Department of Defense Neurofibromatosis Research Program to investigate compounds that promote read-through of nonsense mutations as a potential treatment for neurofibromatosis type 1.

In September 2001, PTC received \$40 million to advance drug discovery efforts based on its proprietary integrated RNA biology and chemistry programs. Capital raised in this round brings PTC's total funding to date to over \$56 million.

In July 2001, PTC was awarded a second Phase I SBIR grant from the NIH. This grant would support a drug discovery program to disrupt the HIV Tat-TAR interaction, necessary for the production of infectious virions. PTC has utilized its TRAC (targeted ribonucleic acid chemistry) platform technology to identify proteins that bind to specific RNA sequences, such as the TAR stem-loop structure.

In December 2000, PTC was awarded a Phase I SBIR grant from the National Institutes of Health. Funding from this grant will be used to advance PTC's RNA-based approach for developing novel HIV drugs that target the mechanism the virus uses to replicate. The company's unique approach is based on interfering with an essential protein production process, called 'programmed ribosomal frameshifting', required by the virus for its survival.

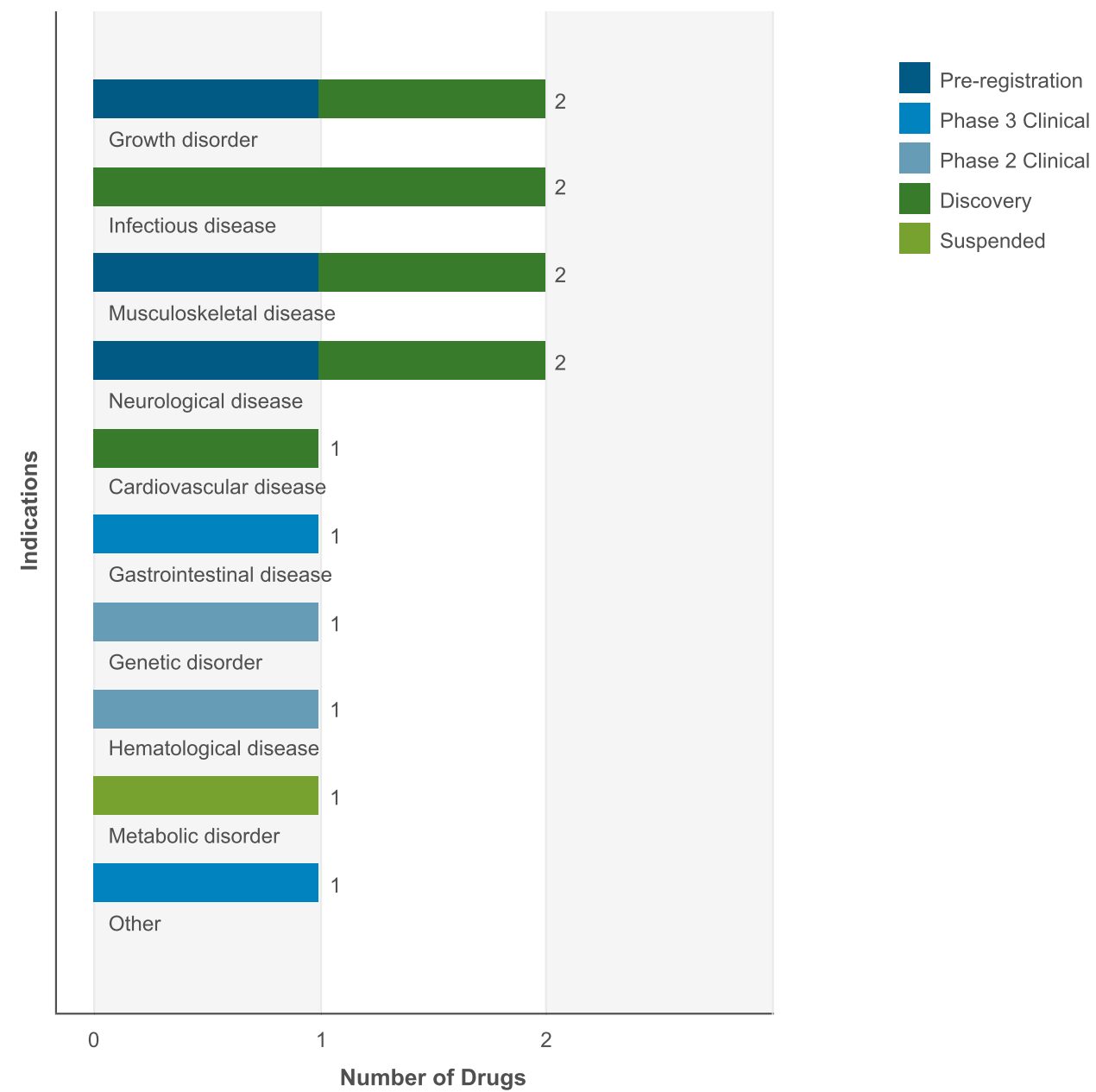
[Return to Table of Contents](#)

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

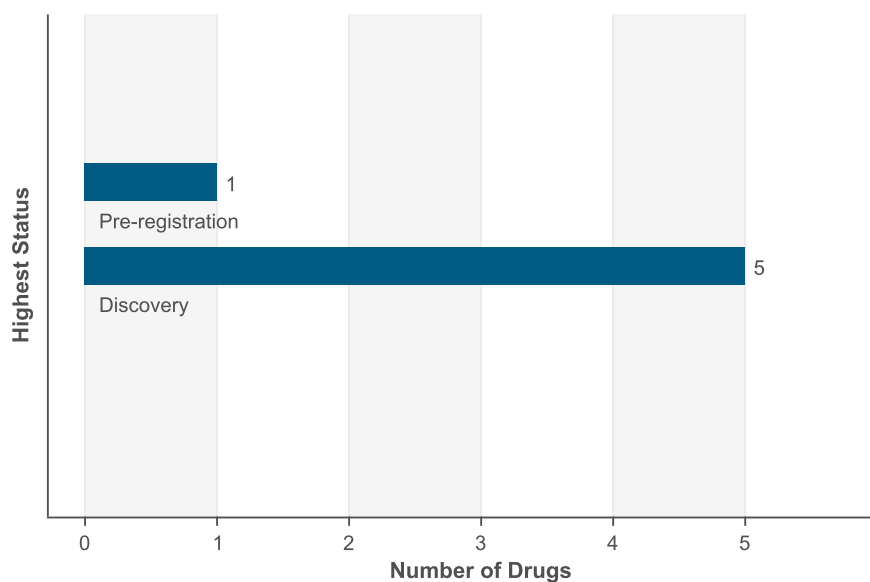
Indication	Active	Inactive	Total
Neurological disease	2	3	5

[Return to Table of Contents](#)

Infectious disease	2	3	5
Neoplasm	1	3	4
Cardiovascular disease	1	2	3
Musculoskeletal disease	2	1	3
Gastrointestinal disease	1	2	3
Growth disorder	2	1	3
Inflammatory disease	0	3	3
Endocrine disease	0	3	3
Metabolic disorder	1	2	3
Gynecology and obstetrics	0	2	2
Respiratory disease	1	1	2
Hematological disease	1	1	2
Genetic disorder	1	1	2
Dermatological disease	0	1	1
Immune disorder	0	1	1
Degeneration	0	1	1

Drugs by Highest Status

Active Drugs by Highest Status Chart



[Return to Table of Contents](#)

Drugs by Highest Status Table

Development Status	Number of Drugs
Pre-registration	1
Discovery	5
Discontinued	3
No Development Reported	5

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	2	0	0	0	2
Drug - Funding	13	0	0	0	13
Drug - Early Research/Development	3	0	0	0	3
Drug - Development/Commercialization License	3	0	0	0	3
Drug - Commercialization License	1	0	0	0	1
Technology - Target Validation	1	0	0	0	1

[Return to Table of Contents](#)



CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Neurological disease	1	9
Growth disorder	1	8
Musculoskeletal disease	1	8
Gastrointestinal disease	0	7
Respiratory disease	0	7
Neoplasm	1	6
Dermatological disease	0	3
Gynecology and obstetrics	0	3
Endocrine disease	0	3
Genetic disorder	0	1
Metabolic disorder	0	1
Hematological disease	0	1

Trials by Phase

Phase	Ongoing	All
Phase 3	1	4
Phase 2	0	12
Phase 1	1	6

Phase Definitions

Phase 3 Clinical

Includes Phase 3, Phase 3b, Phase 3a, Phase 2/3 (where enrolment count is 300 or over)

Phase 2 Clinical

Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

Phase 1 Clinical

Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

PATENTS *

Indication	As Owner	As Third Party	Total
Cardiovascular disease	12	1	13
Endocrine disease	8	0	8

[Return to Table of Contents](#)



Gastrointestinal disease	23	0	23
Genitourinary disease	6	0	6
Growth disorder	9	0	9
Hematological disease	11	0	11
Degeneration	3	0	3
Andrology	1	0	1
Immune disorder	19	0	19
Musculoskeletal disease	18	0	18
Neoplasm	31	0	31
Ocular disease	7	0	7
Genetic disorder	14	0	14
Metabolic disorder	17	0	17
Neurological disease	21	0	21
Nutritional disorder	9	0	9
Respiratory disease	12	0	12
Infectious disease	19	1	20
Inflammatory disease	27	1	28
Gynecology and obstetrics	5	0	5
Dermatological disease	13	0	13

* This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

[Return to Table of Contents](#)

PRODUCT PORTFOLIO DRUGS

PLEASE NOTE: Highest status refers to highest development of that drug for one of the active companies

ataluren

Drug Name	ataluren
Key Synonyms	Translarna, ataluren
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	Genzyme Corp
Highest Status	Pre-registration
Active Indications	Cystic fibrosis, Methylmalonic acidemia, Hemophilia, Genetic disorder, Becker muscular dystrophy, Duchenne dystrophy
Target-based Actions	
Other Actions	Ribosome binding agent
Technologies	Translational trans-acting regulator, Oral formulation, Oral suspension formulation, Small molecule therapeutic
Last Change Date	19-Dec-2012

antibacterial program, PTC Therapeutics

Drug Name	antibacterial program, PTC Therapeutics
Key Synonyms	
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Bacterial infection
Target-based Actions	
Other Actions	Antibacterial, Translation pathway modulator
Technologies	Antibiotic, Small molecule therapeutic
Last Change Date	04-Oct-2012

[Return to Table of Contents](#)

Duchenne muscular dystrophy program, PTC Therapeutics

Drug Name	Duchenne muscular dystrophy program, PTC Therapeutics
Key Synonyms	
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Duchenne dystrophy
Target-based Actions	IGF gene stimulator, UTRN gene stimulator, MSTN gene inhibitor
Other Actions	
Technologies	Small molecule therapeutic
Last Change Date	04-Oct-2012

small molecule Bmi-1 inhibitors (cancer), PTC Therapeutics

Drug Name	small molecule Bmi-1 inhibitors (cancer), PTC Therapeutics
Key Synonyms	
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	Polycomb complex protein BMI-1 inhibitor
Other Actions	Anticancer
Technologies	Oral formulation, Small molecule therapeutic
Last Change Date	21-Nov-2012

[Return to Table of Contents](#)

SERCA 2a activators (heart failure), PTC Therapeutics

Drug Name	SERCA 2a activators (heart failure), PTC Therapeutics
Key Synonyms	
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cardiac failure
Target-based Actions	Sarco endoplasmic calcium ATPase 2a stimulator
Other Actions	
Technologies	Small molecule therapeutic
Last Change Date	04-Oct-2012

antiviral program, PTC Therapeutics

Drug Name	antiviral program, PTC Therapeutics
Key Synonyms	
Originator Company	PTC Therapeutics Inc
Active Companies	PTC Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Dengue virus infection, HIV infection
Target-based Actions	
Other Actions	Antiviral, Translation pathway modulator
Technologies	Small molecule therapeutic
Last Change Date	22-Oct-2012

[Return to Table of Contents](#)

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information.

For more information about *Cortellis for Competitive Intelligence*, visit:

http://cortellis.thomsonreuters.com/cortellis_for_you/?cid=thomsonone.

For subscription information, e-mail scientific.lifesciences@thomsonreuters.com.

© 2012 Thomson Reuters. All rights reserved.
Republication or redistribution of Thomson Reuters content, including by framing or similar means, is prohibited without the prior written consent of Thomson Reuters. 'Thomson Reuters' and the Thomson Reuters logo are registered trademarks and trademarks of Thomson Reuters and its affiliated companies.

[Return to Table of Contents](#)

