

## **PTC Therapeutics Inc**

### **COMPANY AND PIPELINE OVERVIEW REPORT**

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

Publication Date: 12-Apr-2013

#### **THOMSON REUTERS**

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

[thomsonreuters.com](http://thomsonreuters.com)

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## 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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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.

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# PTC Therapeutics Inc

## COMPANY OVERVIEW

<b>Company Name</b>	PTC Therapeutics Inc
<b>Parent Company Name</b>	PTC Therapeutics Inc
<b>Website</b>	<a href="http://www.ptcbio.com/">http://www.ptcbio.com/</a>
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	6
<b>Number of Inactive Drugs</b>	8
<b>Number of Patents as Owner</b>	61
<b>Number of Patents as Third Party</b>	1
<b>Number of Deals</b>	23
<b>Key Indications</b>	Duchenne dystrophy, Cancer, Bacterial infection, Cystic fibrosis, Genetic disorder, Dengue virus infection, HIV infection, Methylmalonic acidemia, Becker muscular dystrophy, Cardiac failure, Hemophilia
<b>Key Target-based Actions</b>	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
<b>Key Technologies</b>	Small molecule therapeutic, Oral formulation, Antibiotic, Gene expression regulation, Oral suspension formulation, Translational trans-acting regulator, Drug screening, Drug combination, Analytical method, Chemical isolation, Condensational synthesis, Crystalline form, ELISA, Hydrolytic synthesis, Immunoglobulin, Monoclonal antibody, Oligonucleotide, Stereochemical 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

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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.

## 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 March 2013, PTC completed a \$60 million financing round.

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

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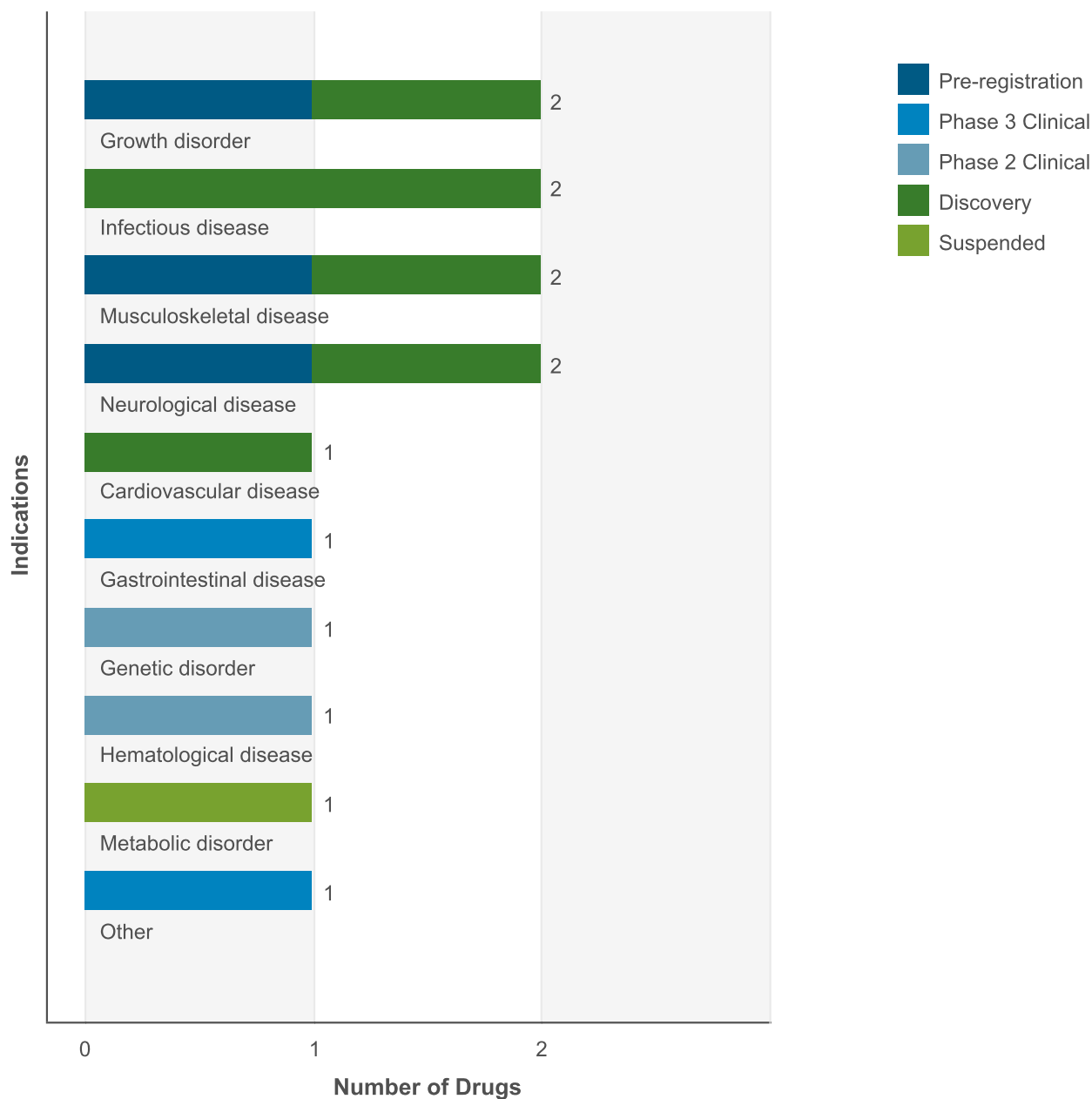
virus uses to replicate. The company's unique approach is based on interfering with an essential protein production process, called 'programmed ribosomal frameshifting', required by the virus for its survival.

## PRODUCT PORTFOLIO SUMMARY

### DRUGS

#### Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

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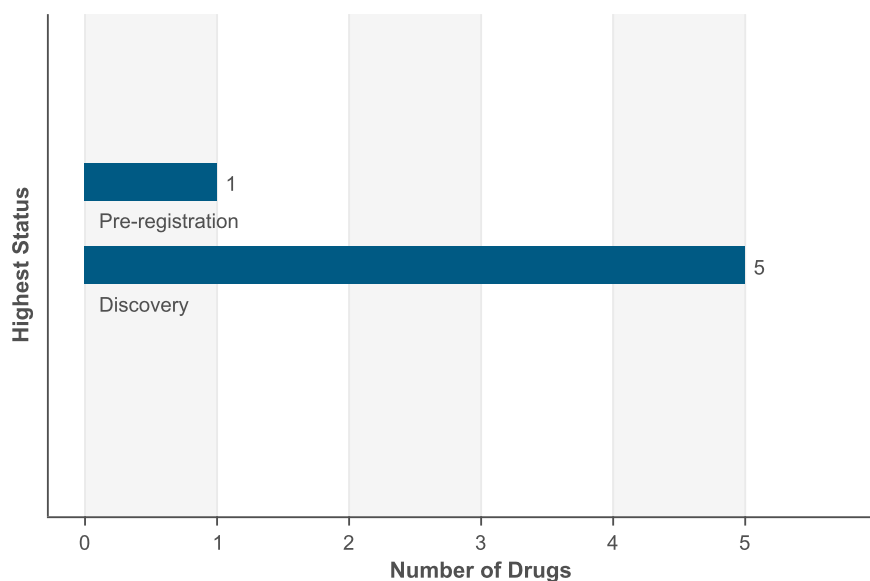
Indication	Active	Inactive	Total
Neurological disease	2	3	5
Infectious disease	2	3	5
Neoplasm	1	3	4
Cardiovascular disease	1	2	3
Musculoskeletal disease	2	1	3
Gastrointestinal disease	1	2	3
Growth disorder	2	1	3
Inflammatory disease	0	3	3
Endocrine disease	0	3	3
Metabolic disorder	1	2	3
Gynecology and 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

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## Drugs by Highest Status

Active Drugs by Highest Status Chart



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 - Development/Commercialization License	3	0	0	0	3
Technology - Target Validation	1	0	0	0	1
Drug - Early Research/Development	3	0	0	0	3
Drug - Commercialization License	1	0	0	0	1

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## CLINICAL TRIALS

### Trials by Condition Studied

Condition Studied	Ongoing	All
Neurological disease	2	10
Growth disorder	2	9
Musculoskeletal disease	2	9
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	2	5
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

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Gastrointestinal disease	22	0	22
Genitourinary disease	5	0	5
Growth disorder	9	0	9
Hematological disease	10	0	10
Degeneration	3	0	3
Andrology	1	0	1
Immune disorder	20	0	20
Musculoskeletal disease	17	0	17
Neoplasm	32	0	32
Ocular disease	7	0	7
Genetic disorder	15	0	15
Metabolic disorder	16	0	16
Neurological disease	21	0	21
Nutritional disorder	9	0	9
Respiratory disease	13	0	13
Infectious disease	22	1	23
Inflammatory disease	28	1	29
Gynecology and obstetrics	4	0	4
Dermatological disease	11	0	11

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

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## PRODUCT PORTFOLIO DRUGS

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

### ataluren

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

### antibacterial program, PTC Therapeutics

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

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### Duchenne muscular dystrophy program, PTC Therapeutics

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

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

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

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### 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	Translation pathway modulator, Antiviral
Technologies	Small molecule therapeutic
Last Change Date	22-Oct-2012

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