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PTC Therapeutics Inc

CORTELLIS COMPANY DETAILED PIPELINE REPORT

A comprehensive coverage of the the company's drug pipeline portfolio including detailed product records.

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ABOUT CORTELLIS COMPANY DETAILED PIPELINE 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.

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

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	9
Number of Patents as Owner	67
Number of Patents as Third Party	1
Number of Deals	23
Key Indications	Duchenne dystrophy,Cancer,Bacterial infection,Cystic fibrosis,Dengue virus infection,Genetic disorder,HIV infection,Methylmalonic acidemia,Becker muscular dystrophy,Cardiac failure,Hemophilia
Key Target-based Actions	SMN1 gene modulator,VEGF receptor antagonist,DNA gyrase inhibitor,Topoisomerase IV inhibitor,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,Regulator of nonsense transcripts modulator,Sarco endoplasmic calcium ATPase 2a stimulator,UTRN gene stimulator
Key Technologies	Small molecule therapeutic,Oral formulation,Antibiotic,Gene expression regulation,Oral suspension formulation,Systemic formulation unspecified,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. Celegene 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.

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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.

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 February 2014, PTC commenced a $75 million public offering of common stock; at that time, the underwriters were to be granted a 30-day option to purchase up to an additional 15% of the shares sold in the offering. Later in the same month, PTC priced the offering of 4,489,796 million shares of common stock at $24.50 per share and granted underwriters a 30-day option to purchase up to 673,469 additional shares of common stock.

In December 2013, the company was added to the NASDAQ Biotechnology Index.

In September 2013, the company was added to the Russell 2000 Index.

In May 2013, PTC filed a registration statement with the SEC for an IPO of its common stock. In June 2013, PTC priced the IPO of 8,372,000 shares at $15 per share. The underwriters would be granted a 30-day option to buy 1,255,800 additional shares. At that time, the company's common stock was approved for listing on the NASDAQ Global Select Market, under the ticker symbol 'PTCT'; later that month, the company commenced trading on the NASDAQ Stock Market on June 20, 2013. Later in June 2013, the company closed the IPO, with full exercise of underwriters' 30-day overallotment option; by August 2013, the company had raised gross proceeds of more than $144 million.

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 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.

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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.

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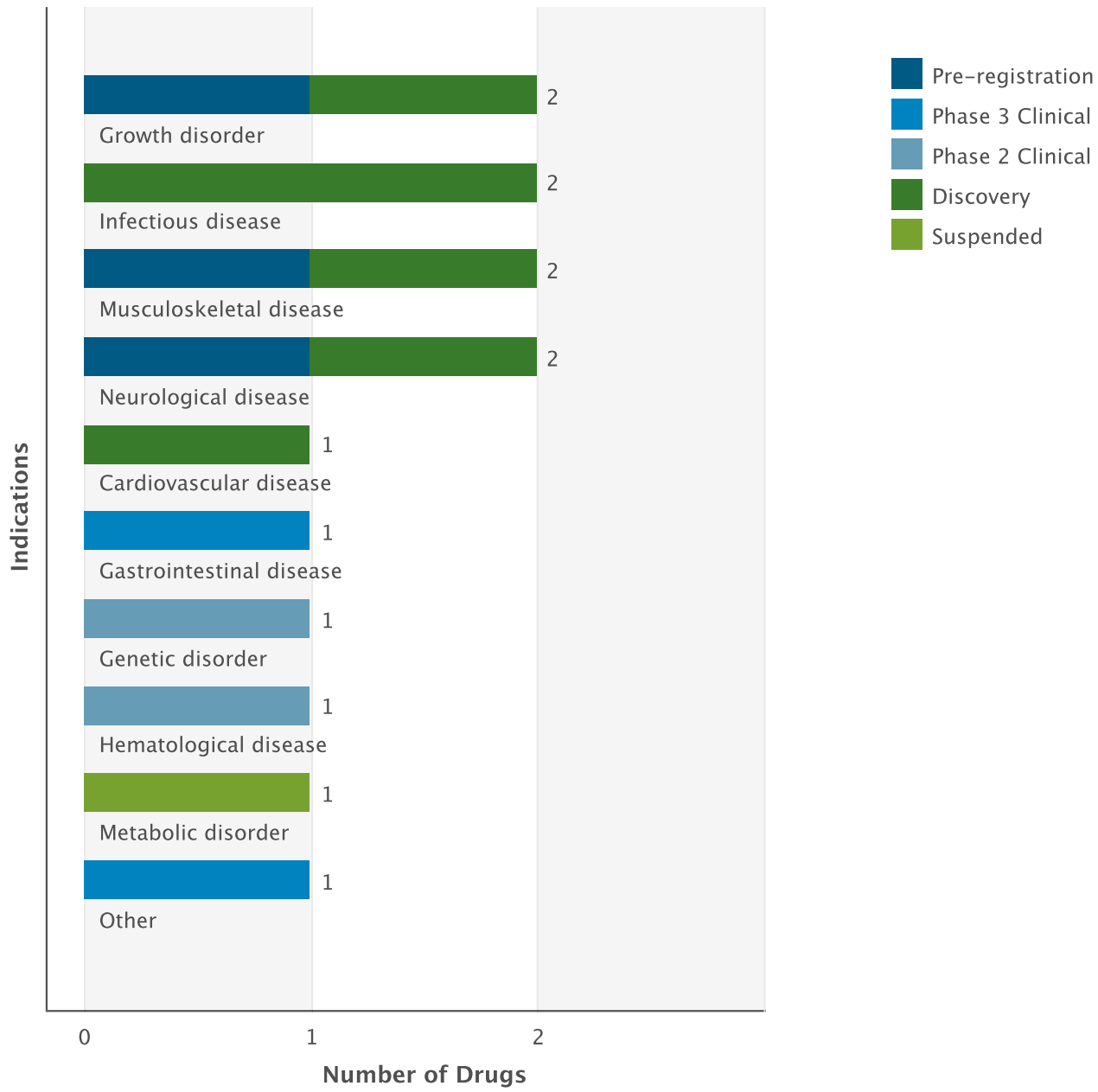
PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



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Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	2	4	6
Infectious disease	2	3	5
Growth disorder	2	2	4
Neoplasm	1	3	4
Gastrointestinal disease	1	2	3
Musculoskeletal disease	2	1	3
Inflammatory disease	0	3	3
Endocrine disease	0	3	3
Metabolic disorder	1	2	3
Cardiovascular disease	1	2	3
Hematological disease	1	1	2
Respiratory disease	1	1	2
Gynecology and obstetrics	0	2	2
Genetic disorder	1	1	2
Dermatological disease	0	1	1
Degeneration	0	1	1
Immune disorder	0	1	1

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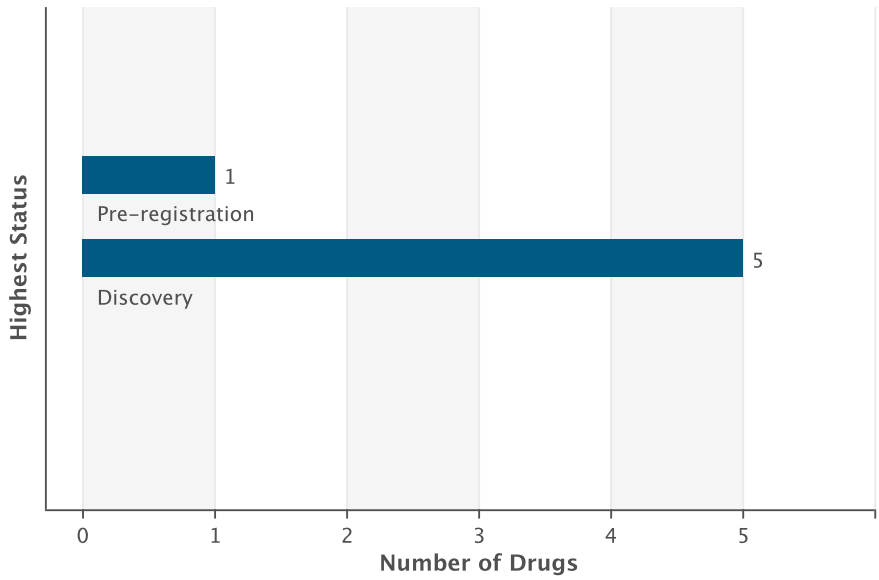
Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Pre-registration	1
Discovery	5
Discontinued	4
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

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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	23	0	23
Genitourinary disease	5	0	5
Growth disorder	14	0	14
Hematological disease	10	0	10
Degeneration	3	0	3
Andrology	1	0	1
Immune disorder	18	0	18
Musculoskeletal disease	17	0	17
Neoplasm	30	0	30
Ocular disease	7	0	7
Genetic disorder	13	0	13
Metabolic disorder	16	0	16
Neurological disease	24	0	24
Nutritional disorder	9	0	9
Respiratory disease	14	0	14
Infectious disease	22	1	23
Inflammatory disease	26	1	27
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 DRUG PIPELINE DETAIL

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

ataluren

ataluren SNAPSHOT

Drug Name	ataluren
Key Synonyms	ataluren;Translarna
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	Regulator of nonsense transcripts modulator
Other Actions	Ribosome binding agent
Technologies	Oral suspension formulation;Oral formulation;Small molecule therapeutic
Last Change Date	28-Jan-2014

ataluren DEVELOPMENT PROFILE

SUMMARY

PTC Therapeutics is developing ataluren (PTC-124; Translarna), an orally active small molecule that overrides nonsense stop translation signals and allows proteins to be correctly translated, for the potential treatment of genetic disorders, primarily Duchenne muscular dystrophy (DMD),.

In December 2012, the EMA validated the filing of PTC's MAA in Europe for ataluren in the treatment of nonsense mutation DMD (nmDMD). In July 2013, PTC submitted responses to the Day 120 Questions to the EMA ; by November 2013, the CHMP had scheduled a scientific advisory group meeting. In January 2014, the CHMP adopted a negative opinion and recommended refusal of the marketing authorization. The company planned to request a re-examination of the CHMP's decision, with a final outcome expected in 2Q14, at which point a confirmatory phase III study was expected to be more fully enrolled. In March 2011, an NDA filing in DMD was anticipated in 1H11.

Development in other indications is ongoing, including cystic fibrosis (CF), Becker muscular dystrophy (BMD), and hemophilia associated with defective protein production. In July 2009, a phase III trial in patients with CF due to a nonsense mutation (nmCF) began ; in June 2012, positive topline data were reported. In November 2013, the company planned an MAA filing nmCF in 1Q14.In July 2009, a phase IIa study for hemophilia A and B was initiated.

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By June 2005, PTC was also exploring the use of the drug for other diseases associated with genetic disorders, such as neurofibromatosis, retinitis pigmentosa, epidermolysis and lysosomal storage disorders. In June 2010, a phase II trial for methylmalonic acidemia (MMA) was initiated; however, by October 2011, the trial had been suspended.

Genzyme (a wholly owned subsidiary of Sanofi) was previously codeveloping the drug for CF. In July 2009, Genzyme and PTC initiated a phase III trial . In February 2012, Sanofi confirmed that development had been discontinued.

ataluren DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Duchenne dystrophy	EU	Pre-registration	06-Dec-2012
PTC Therapeutics Inc	Becker muscular dystrophy	Australia	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Becker muscular dystrophy	Canada	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Becker muscular dystrophy	Europe	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Becker muscular dystrophy	Israel	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Becker muscular dystrophy	US	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Cystic fibrosis	Canada	Phase 3 Clinical	30-Jul-2009
PTC Therapeutics Inc	Cystic fibrosis	Europe	Phase 3 Clinical	30-Jul-2009
PTC Therapeutics Inc	Cystic fibrosis	Israel	Phase 3 Clinical	30-Jul-2009
PTC Therapeutics Inc	Cystic fibrosis	US	Phase 3 Clinical	30-Jul-2009
PTC Therapeutics Inc	Duchenne dystrophy	Australia	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Duchenne dystrophy	Brazil	Phase 3 Clinical	24-Jan-2014
PTC Therapeutics Inc	Duchenne dystrophy	Canada	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Duchenne dystrophy	Chile	Phase 3 Clinical	24-Jan-2014
PTC Therapeutics Inc	Duchenne dystrophy	Israel	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Duchenne dystrophy	South Korea	Phase 3 Clinical	24-Jan-2014
PTC Therapeutics Inc	Duchenne dystrophy	US	Phase 3 Clinical	29-Feb-2008
PTC Therapeutics Inc	Hemophilia	Canada	Phase 2 Clinical	31-Aug-2009
PTC Therapeutics Inc	Hemophilia	France	Phase 2 Clinical	31-Aug-2009
PTC Therapeutics Inc	Hemophilia	Italy	Phase 2 Clinical	31-Aug-2009

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Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Hemophilia	US	Phase 2 Clinical	23-Jul-2009
PTC Therapeutics Inc	Genetic disorder	US	Phase 1 Clinical	13-Jul-2004
PTC Therapeutics Inc	Methylmalonic acidemia	Europe	Suspended	31-Oct-2011
Genzyme Corp	Becker muscular dystrophy	US	Discontinued	02-Sep-2011
Genzyme Corp	Cystic fibrosis	Canada	Discontinued	08-Feb-2012
Genzyme Corp	Cystic fibrosis	Europe	Discontinued	08-Feb-2012
Genzyme Corp	Cystic fibrosis	Israel	Discontinued	08-Feb-2012
Genzyme Corp	Cystic fibrosis	US	Discontinued	08-Feb-2012
Genzyme Corp	Duchenne dystrophy	US	Discontinued	02-Sep-2011
Genzyme Corp	Genetic disorder	US	Discontinued	02-Sep-2011

ataluren CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
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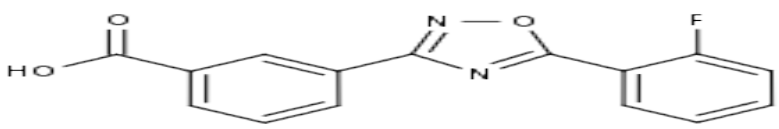

Name	Type
ataluren	PINN; USAN
Translarna	Trade Name
PTC-124	Research Code

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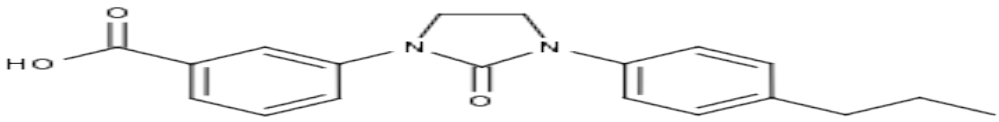
CAS Registry Number:	Confidence Level:
	4



ataluren DRUG NAMES

Names	Type
cystic fibrosis therapy, PTC Therapeutics	
PTC-124	Research Code
Translarna	Trade Name
ataluren	PINN, USAN
mRNA modulators (genetic disorders), PTC Therapeutics	
Duchenne muscular dystrophy therapy, PTC Therapeutics	

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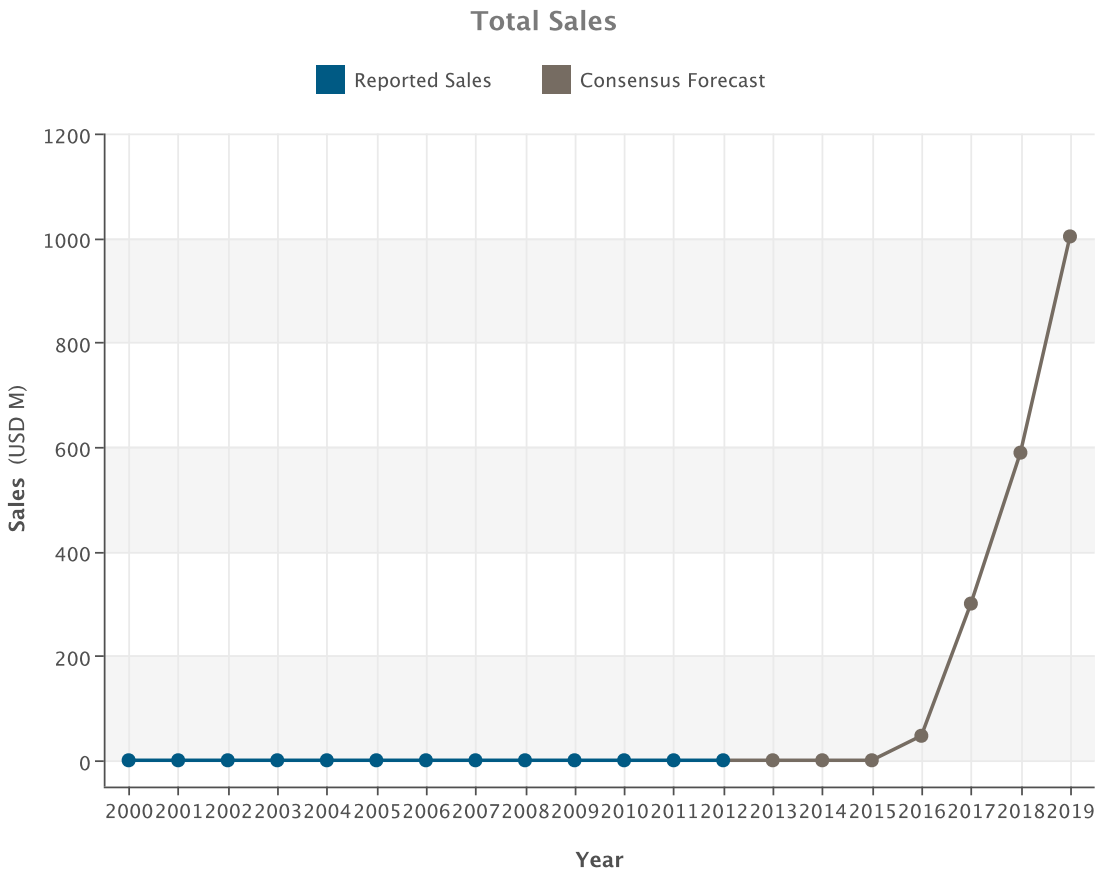
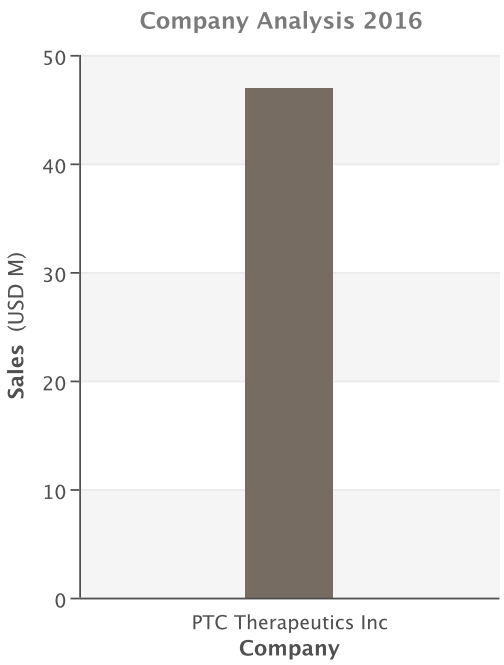
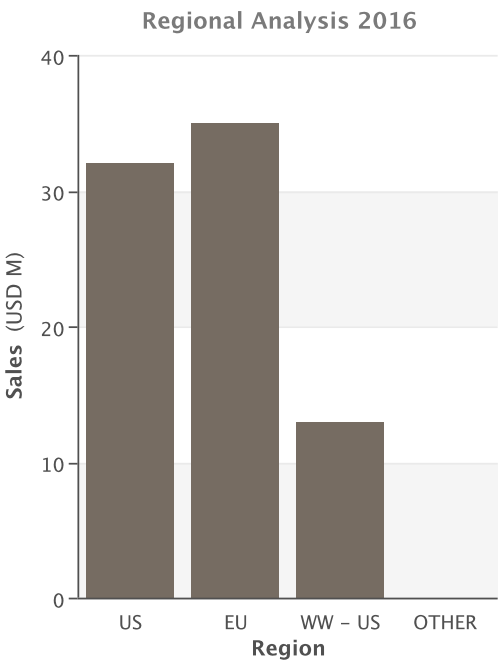
ataluren SALES AND FORECASTS

CHARTS







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COMMENTARY

CONSENSUS SALES INFORMATION

Consensus forecast data for PTC Therapeutics are presented.

REGIONAL DEVELOPMENT AND MARKETING RIGHTS

In July 2008, Genzyme (now a wholly owned subsidiary of Sanofi) acquired a license from originator PTC to commercialize ataluren worldwide, excluding the US and Canada [925978]. However, in September 2011, the agreement was restructured; PTC regained worldwide rights, with Genzyme retaining an option for commercialization in indications other than nonsense mutation Duchenne/Becker muscular dystrophy outside Canada and the US [1219337]. Sanofi had discontinued development by February 2012 [1260944].

ataluren CLINICAL TRIALS

Trials by Phase and Condition Studied

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Duchenne dystrophy											
0	0	2	3	0	5	0	1	0	0	2	9
Cystic fibrosis											
0	0	0	2	0	4	0	1	0	0	0	7
Muscular dystrophy											
0	0	0	0	0	3	0	0	0	0	0	3
Factor VIII deficiency											
0	0	0	0	0	1	0	0	0	0	0	1
Becker muscular dystrophy											
0	0	0	1	0	0	0	0	0	0	0	1
Factor IX deficiency											
0	0	0	0	0	1	0	0	0	0	0	1
Methylmalonic acidemia											
0	0	0	0	0	1	0	0	0	0	0	1

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Total Trials by Phase and Status

Phase 4 Clinical		Phase 3 Clinical		Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total	
On-going	All	On-going	All	On-going	All	On-going	All	On-going	All	On-going	All
Total by Phase and Status											
0	0	2	5	0	11	0	1	0	0	2	17

Phase Definitions

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

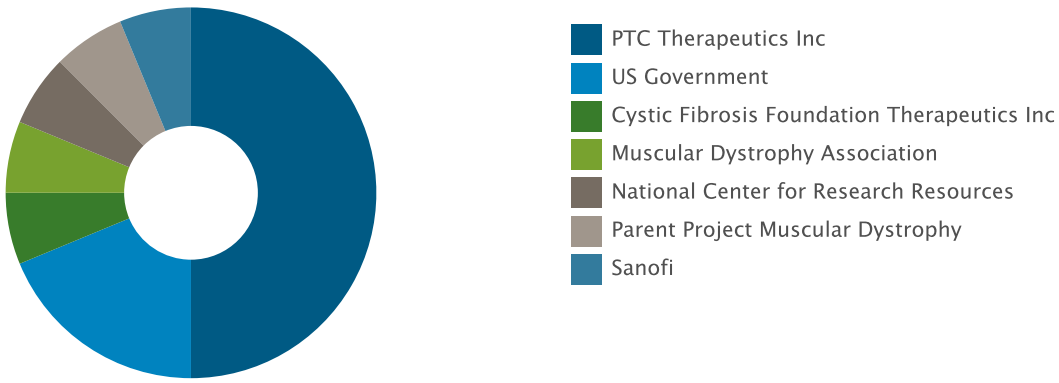
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

ataluren DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 8

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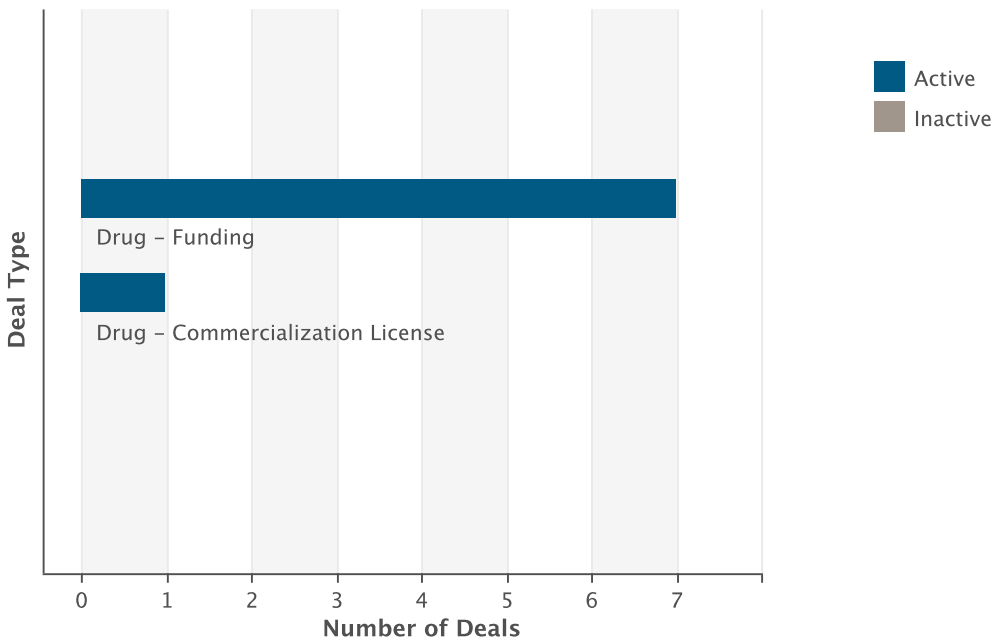
Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	8	0	0	0	8
US Government	0	0	3	0	3
National Center for Research Resources	0	0	1	0	1
Parent Project Muscular Dystrophy	0	0	1	0	1
Sanofi	0	0	1	0	1
Cystic Fibrosis Foundation Therapeutics Inc	0	0	1	0	1
Muscular Dystrophy Association	0	0	1	0	1

Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	7	0	7
Drug - Commercialization License	1	0	1

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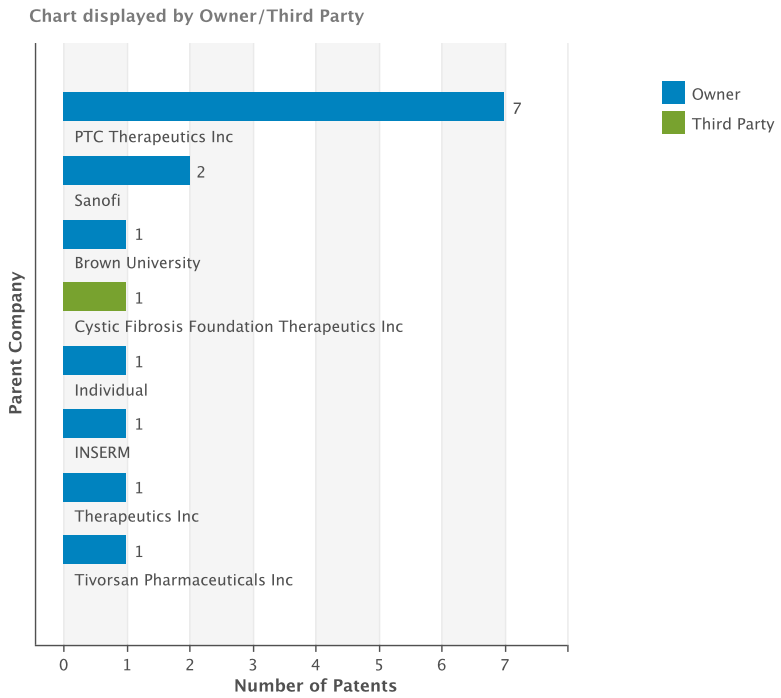
PATENTS

Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
PTC Therapeutics Inc	7	0	7
Sanofi	2	0	2
Individual	1	0	1
Tivorsan Pharmaceuticals Inc	1	0	1
Therapeutics Inc	1	0	1
Brown University	1	0	1
INSERM	1	0	1
Cystic Fibrosis Foundation Therapeutics Inc	0	1	1

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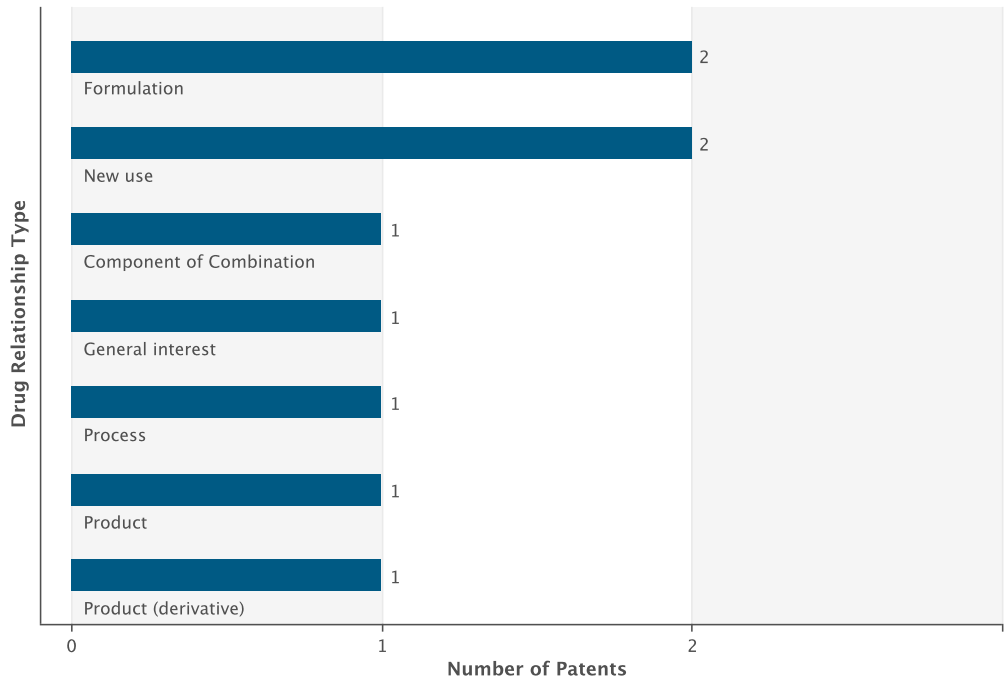
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Formulation	2
New use	2
Component of Combination	1
Product	1
Process	1
Product (derivative)	1
General interest	1

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Duchenne muscular dystrophy program, PTC Therapeutics

Duchenne muscular dystrophy program, PTC Therapeutics SNAPSHOT

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	MSTN gene inhibitor;IGF gene stimulator;UTRN gene stimulator
Other Actions	
Technologies	Small molecule therapeutic
Last Change Date	04-Oct-2012

Duchenne muscular dystrophy program, PTC Therapeutics DEVELOPMENT PROFILE

SUMMARY

PTC Therapeutics is investigating small-molecule therapeutics that target post-transcriptional control of gene expression, to increase levels of utrophin and the muscle-specific form of insulin-like growth factor (mIGF-1) and decrease levels of myostatin, for the potential treatment of Duchenne muscular dystrophy (DMD). In September 2010, preclinical testing of multiple compounds was underway in a mdx mouse model. In October 2012, this was still the case.

Duchenne muscular dystrophy program, PTC Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Duchenne dystrophy	US	Discovery	31-Dec-2003

Duchenne muscular dystrophy program, PTC Therapeutics DRUG NAMES

Names	Type
Duchenne muscular dystrophy program, PTC Therapeutics	
Project Catalyst (DMD), PTC Therapeutics/Parent Project Muscular Dystrophy	

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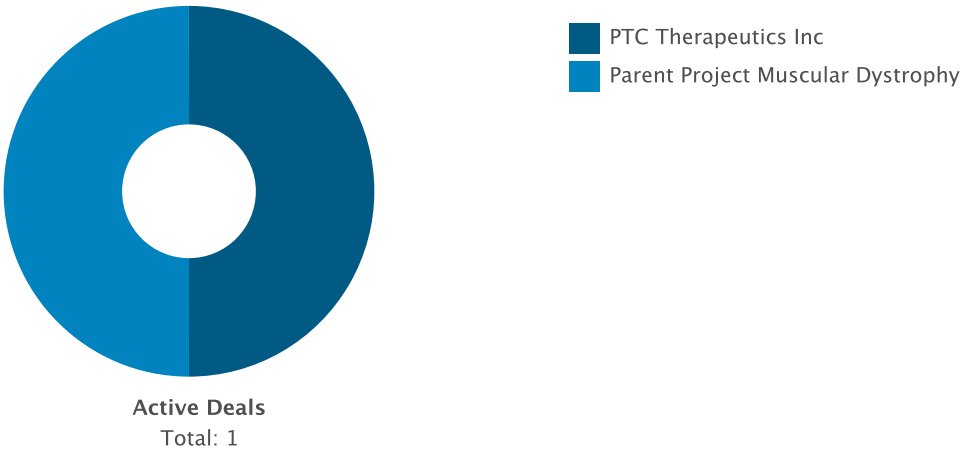
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	1	0	0	0	1
Parent Project Muscular Dystrophy	0	0	1	0	1

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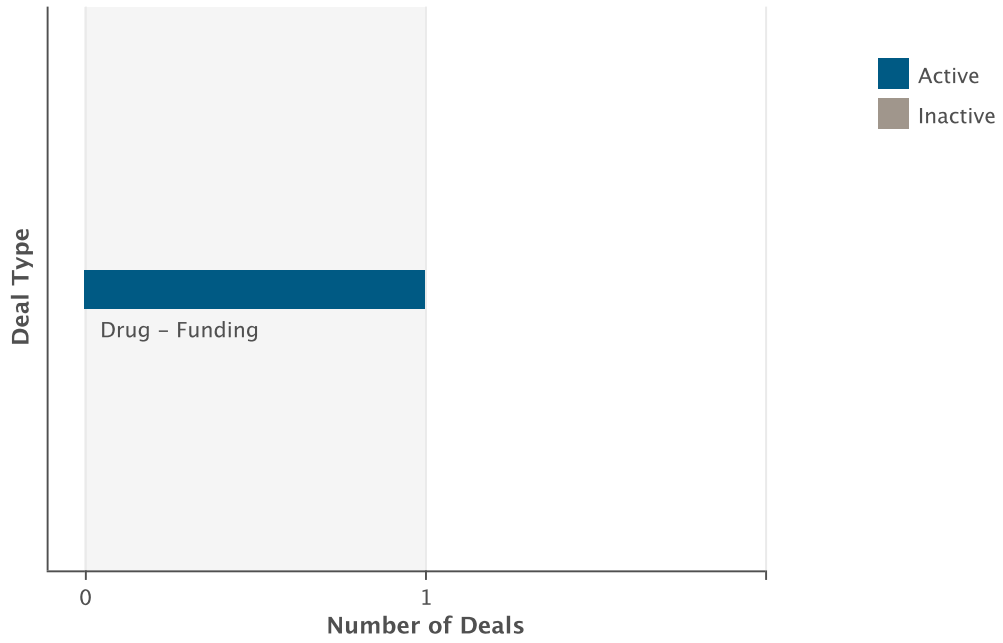
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

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antibacterial program, PTC Therapeutics

antibacterial program, PTC Therapeutics SNAPSHOT

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	DNA gyrase inhibitor;Topoisomerase IV inhibitor
Other Actions	Translation pathway modulator;Antibacterial;Synergist
Technologies	Small molecule therapeutic;Antibiotic
Last Change Date	19-Sep-2013

antibacterial program, PTC Therapeutics DEVELOPMENT PROFILE

SUMMARY

PTC Therapeutics is investigating broad-spectrum, small-molecule antibacterial agents which target post-transcriptional control mechanisms and modulate mRNA, for the potential treatment of various bacterial infection, including multidrug-resistant (MDR) Gram-negative bacterial infection and those associated with biowarfare or agents,. By October 2006, lead optimization studies were underway ; in December 2011, these were ongoing. In September 2013, preclinical data were presented.

antibacterial program, PTC Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Bacterial infection	US	Discovery	06-Dec-2005

antibacterial program, PTC Therapeutics CHEMICAL STRUCTURES

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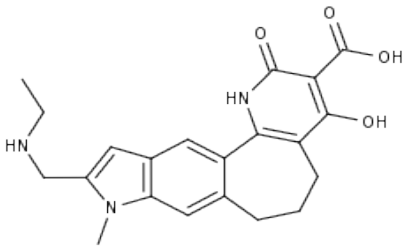
CAS Registry Number:	Confidence Level:
	4



Name	Type
PTC-QZ	Research Code

antibacterial program, PTC Therapeutics DRUG NAMES

Names	Type
antibacterial program, PTC Therapeutics	
PTC-QJ	Research Code
PTC-QZ	Research Code

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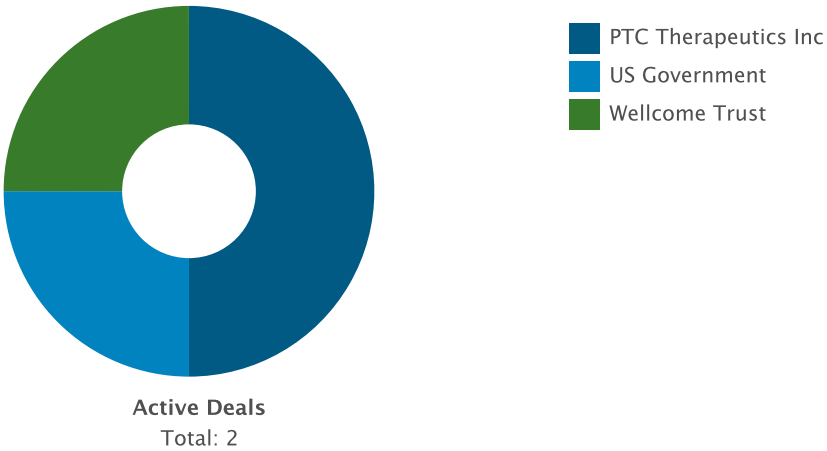
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	2	0	0	0	2
US Government	0	0	1	0	1
Wellcome Trust	0	0	1	0	1

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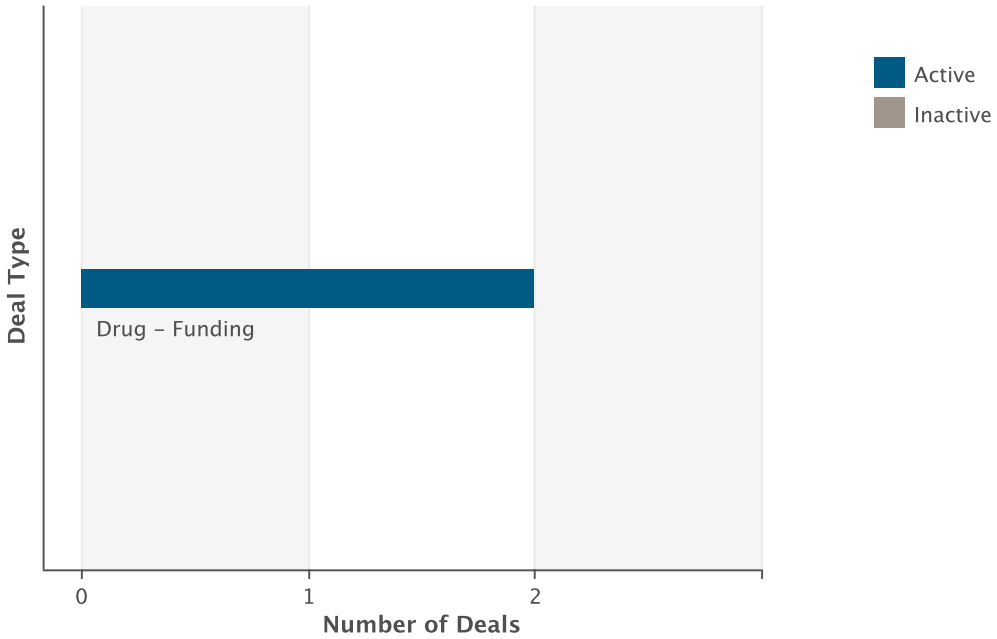
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	2	0	2

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SERCA 2a activators (heart failure), PTC Therapeutics

SERCA 2a activators (heart failure), PTC Therapeutics SNAPSHOT

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

SERCA 2a activators (heart failure), PTC Therapeutics DEVELOPMENT PROFILE

SUMMARY

PTC Therapeutics is investigating sarcoplasmic reticulum ATPase 2a (SERCA 2a) activators for the potential improvement of heart function in patients with Duchenne and Becker muscular dystrophy (DBMD). In October 2012, the program was listed as being in discovery.

SERCA 2a activators (heart failure), PTC Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Cardiac failure	US	Discovery	01-Feb-2011

SERCA 2a activators (heart failure), PTC Therapeutics DRUG NAMES

Names	Type
SERCA 2a activators (heart failure), PTC Therapeutics	
sarcoplasmic reticulum ATPase 2a activators (heart failure), PTC Therapeutics	

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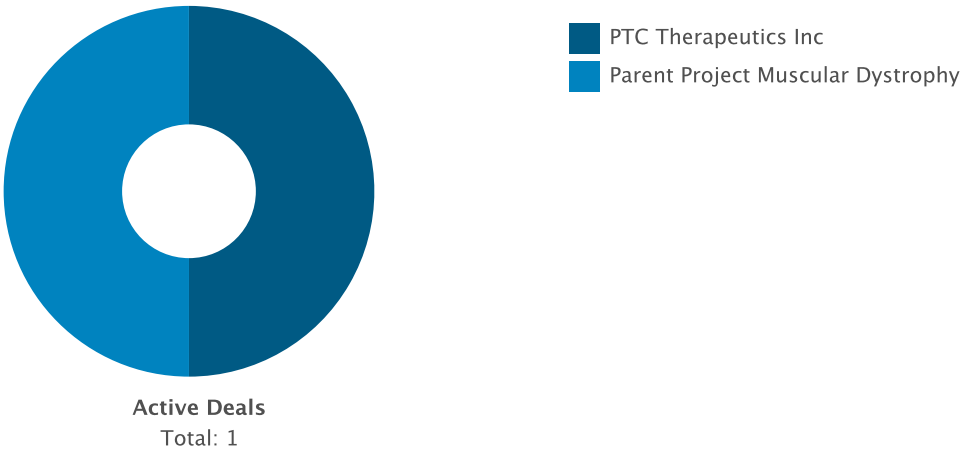
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	1	0	0	0	1
Parent Project Muscular Dystrophy	0	0	1	0	1

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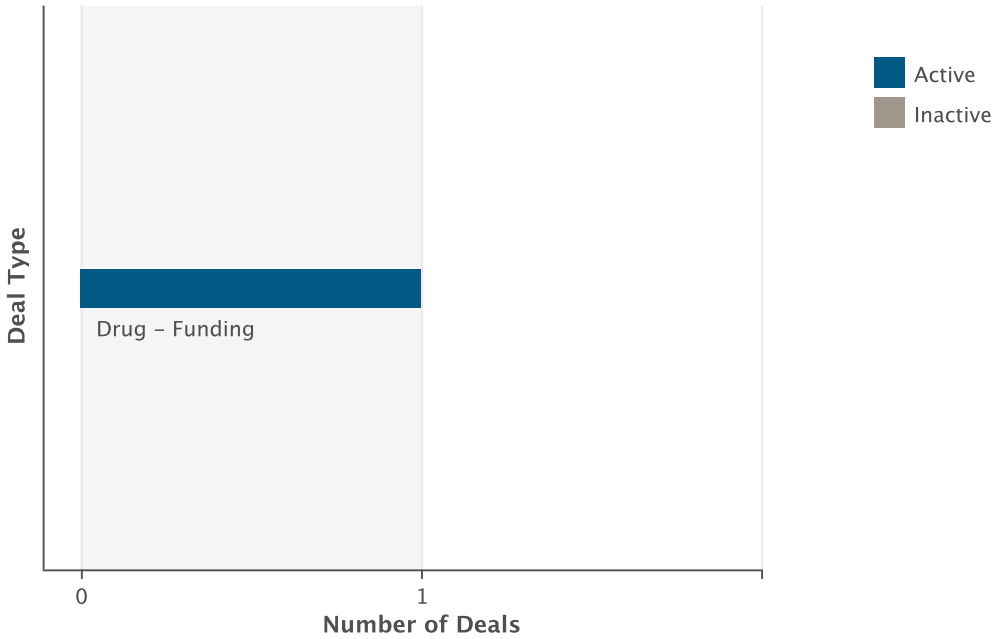
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

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

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

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

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

SUMMARY

PTC Therapeutics is investigating a program of orally-active, small molecule Bmi-1 protein inhibitors, including PTC-596, discovered using PTC's GEMS screening technique, for the potential treatment of cancer, particularly chemotherapy-resistant types, including glioblastoma and radiation-resistant cancer. In July 2012, advanced evaluation of PTC-596 was planned.

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

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Cancer	US	Discovery	07-Jun-2010

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

Names	Type
small molecule Bmi-1 inhibitors (cancer), PTC Therapeutics	
PTC-596	Research Code

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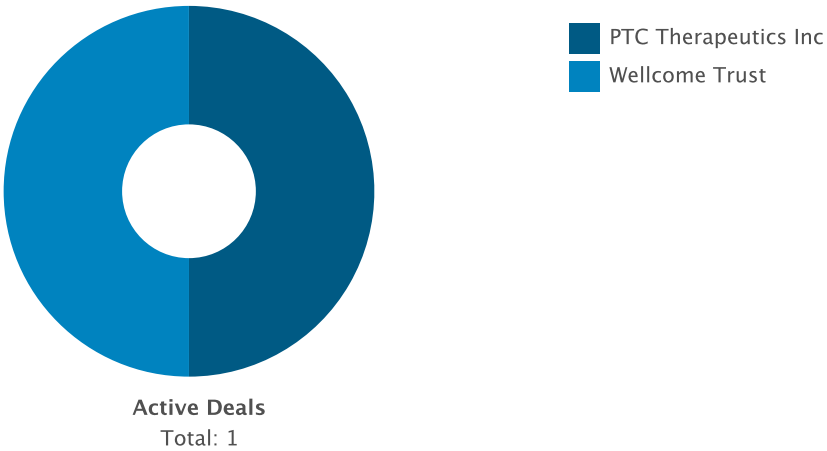
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	1	0	0	0	1
Wellcome Trust	0	0	1	0	1

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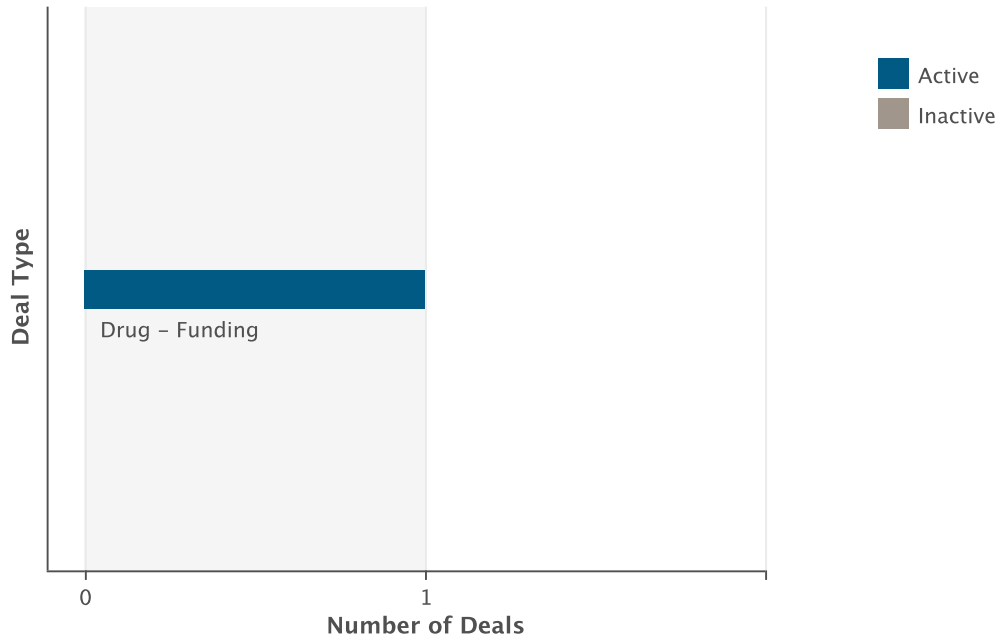
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

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antiviral program, PTC Therapeutics

antiviral program, PTC Therapeutics SNAPSHOT

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

antiviral program, PTC Therapeutics DEVELOPMENT PROFILE

SUMMARY

PTC Therapeutics is investigating a series of small molecules which target post-transcriptional control mechanisms and modulate mRNA, for the potential treatment of HIV and Dengue virus infection,. In August 2008, lead optimization studies were underway ; in October 2012, this was still the case.

antiviral program, PTC Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
PTC Therapeutics Inc	Dengue virus infection	US	Discovery	04-Oct-2012
PTC Therapeutics Inc	HIV infection	US	Discovery	04-Oct-2012

antiviral program, PTC Therapeutics DRUG NAMES

Names	Type
antiviral program, PTC Therapeutics	

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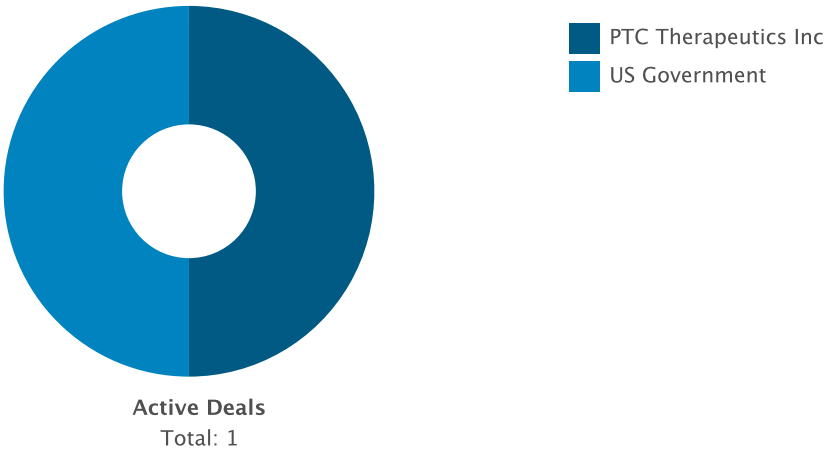
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
PTC Therapeutics Inc	1	0	0	0	1
US Government	0	0	1	0	1

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Deals by Type Chart

Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

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