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Celladon Corp

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

COMPANY OVERVIEW

Company Name	Celladon Corp
Parent Company Name	Celladon Corp
Website	http://www.celladon.net/
Country	US
Number of Drugs in Active Development	5
Number of Inactive Drugs	0
Number of Patents as Owner	8
Number of Patents as Third Party	3
Number of Deals	6
Key Indications	Cardiac failure,Congestive heart failure,Neurodegenerative disease,Diabetes mellitus,Ischemic heart disease,Pulmonary artery hypertension,Vascular fistula,Cancer,Ischemia,Myocardial infarction,Pulmonary hypertension
Key Target-based Actions	Sarco endoplasmic calcium ATPase 2a stimulator,Kit ligand,Sarco endoplasmic calcium ATPase 2b stimulator,ATP2A2 gene stimulator,Sarco endoplasmic calcium ATPase 2 modulator,Sarco endoplasmic calcium ATPase 2a modulator,Cardiac phospholamban modulator,NAD-dependent deacetylase sirtuin-1 stimulator,NFAT gene stimulator,NFKB gene stimulator,PLN gene inhibitor,SUMO1 gene stimulator,Sarco endoplasmic calcium ATPase 2b modulator,Sarco endoplasmic calcium ATPase modulator,Small ubiquitin related modifier 1 modulator,TP53 gene stimulator,Zinc finger protein gene stimulator
Key Technologies	Small molecule therapeutic,Biological therapeutic,Gene transfer system viral,Drug screening,Assay,Fluorescence,Gene transfer system,Immunodetection,Labeling system,Peptide,Polynucleotide sequence,Protein fusion,Vector expression,Virus recombinant

COMPANY PROFILE

SUMMARY

Celladon Corp, based in La Jolla, CA, develops molecular therapies for congestive heart failure. The company's first generation product enhances calcium cycling in the heart, delivered via a recombinant adeno-associated viral (rAAV) vector.

LOCATION

In May 2012, the company was to establish a subsidiary in The Netherlands to manage its European-based activities.

LICENSING AGREEMENTS

In November 2009, Celladon acquired exclusive rights to a technology from University of Minnesota to develop molecular therapies for cardiovascular diseases. The technology, measured by Fluorescence Resonance Energy Transfer (FRET), provides increased screening efficiency of compounds able to disrupt protein interactions that is implicated in cardiovascular disease. University of Minnesota received undisclosed funding from Celladon fo refine the assay further.

In January 2005, Targeted Genetics entered an agreement with Celladon to develop AAV-based gene therapy targeting the SERCA2a pathway for the treatment of congestive heart failure. Targeted Genetics agreed to commit $2 million towards the development, manufacture and preclinical development of the therapy, and Celladon would cover all other development, manufacture and preclinical development costs. Targeted Genetics would receive milestone payments and royalties. In March 2009, the agreement was amended where Celladon could use AAV in an expanded field. The company could use contract manufacturing organizations to manufacture Mydicar.

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In August 2014, the company planned to raise net proceeds from an underwritten public offering of 4,000,000 shares of common stock. At that time, the underwriters were to be granted a 30-day option to purchase up to an aggregate of 600,000 additional shares of common stock. Later that month, the underwritten public offering was priced at $9.50 per share and planned to raise gross proceeds of approximately $38.0 million. The public offering closed on August 18, 2014 with gross proceeds of $43.7 million raised.

In August 2014, Celladon entered a credit facility for up to $25 million of loans, with Hercules Technology Growth Capital Inc. At that time, the company had drawn a first tranche of $10 million, and a second tranche of up to $15 million could be drawn prior to May 31, 2015.

In October 2013, Celladon filed a registration statement S-1 form with the US Securities and Exchange Commission for a planned IPO of common stock. In January 2014, Celladon priced the underwritten public offering of 5.5 million shares at $8.00 per share. The underwriters would be granted a 30-day option to purchase up to 825,000 additional shares to cover overallotments. In February 2014, the underwriters fully exercised their option, and the offering was closed. The company raised total gross proceeds of $50.6 million and net proceeds of $44.3 million from the offering. In March 2014, the company was added to the Russell 2000 and Russell 3000 indexes, as part of the quarterly IPO update to the Russell indexes.

In January 2014, the company's shares were traded under the symbol "CLDN" on the NASDAQ Global Market on January 30, 2014.

In February 2012, Celladon completed a $43 million equity financing. In May 2012, the company announced additional capital proceeds from a second close of the financing, bringing the total capital proceeds to $53 million.

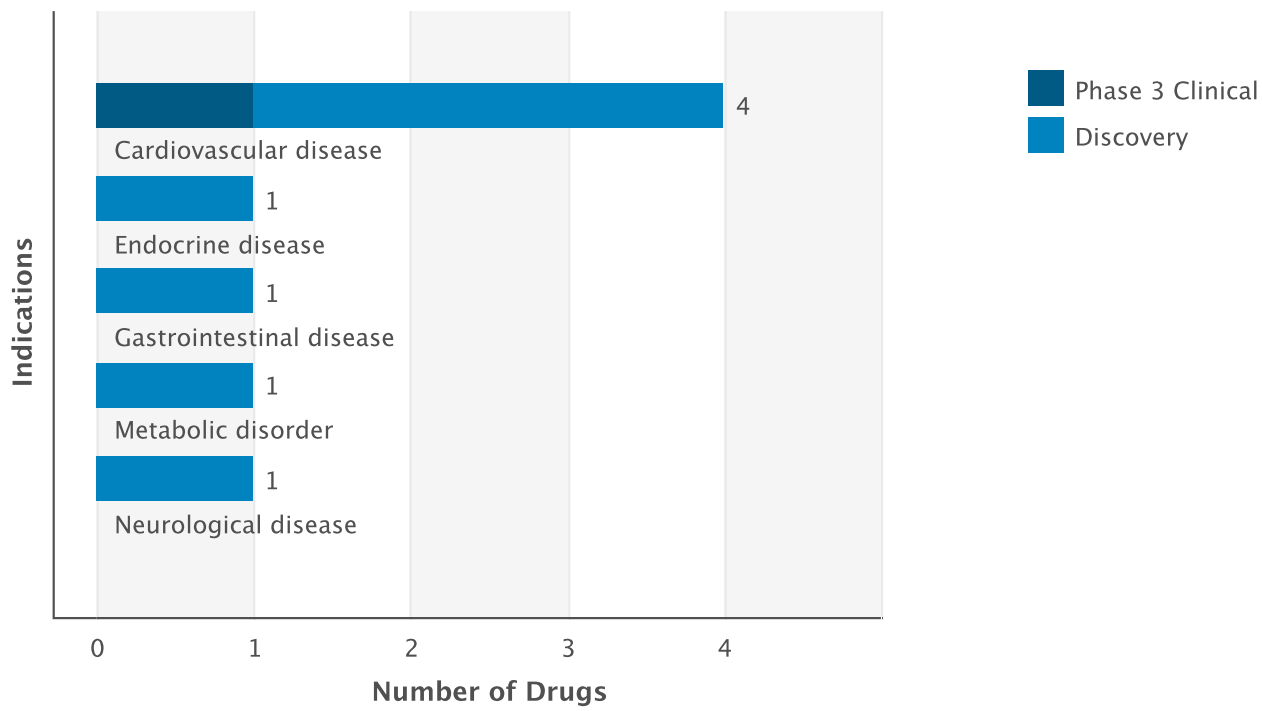
In December 2005, Celladon raised $30 million in a series B venture financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



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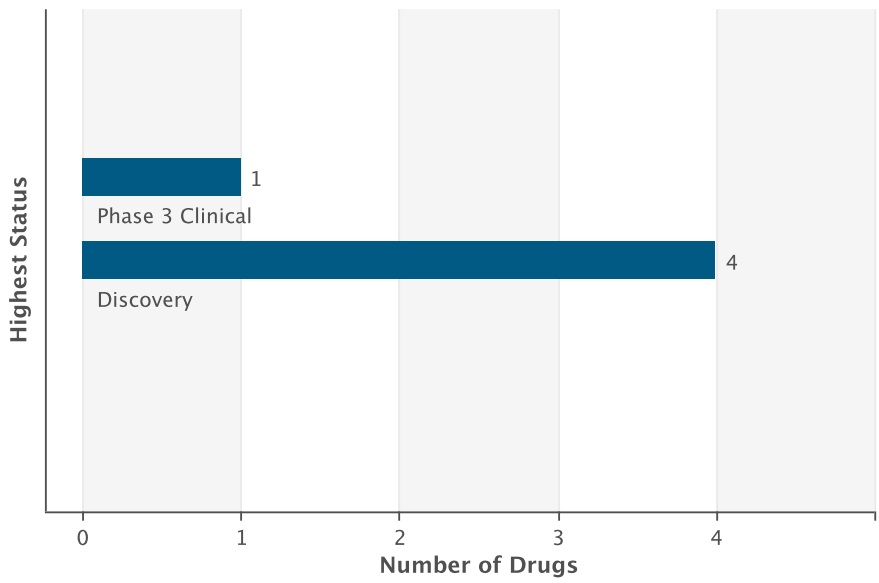
Drugs by Indication Table

Indication	Active	Inactive	Total
Cardiovascular disease	4	0	4
Endocrine disease	1	0	1
Neurological disease	1	0	1
Gastrointestinal disease	1	0	1
Metabolic disorder	1	0	1

Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 3 Clinical	1
Discovery	4

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DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	1	0	1
Drug - Funding	1	0	0	0	1
Drug - Development/Commercialization License	1	0	2	0	3
Drug - Manufacturing/Supply	0	0	1	0	1

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Cardiovascular disease	3	4

Trials by Phase

Phase	Ongoing	All
Phase 2	3	4

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	6	3	9
Endocrine disease	1	0	1
Gastrointestinal disease	1	0	1
Genitourinary disease	2	0	2
Degeneration	1	0	1
Immune disorder	3	0	3
Neoplasm	2	2	4
Metabolic disorder	2	0	2

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Neurological disease	1	2	3
Respiratory disease	1	0	1
Infectious disease	0	2	2
Inflammatory disease	1	2	3

* 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

Mydicar

Mydicar SNAPSHOT

Drug Name	Mydicar
Key Synonyms	Mydicar
Originator Company	AmpliPhi Biosciences Corp
Active Companies	Celladon Corp
Inactive Companies	AmpliPhi Biosciences Corp
Highest Status	Phase 3 Clinical
Active Indications	Congestive heart failure;Vascular fistula
Target-based Actions	Sarco endoplasmic calcium ATPase 2a modulator
Other Actions	Adeno-associated virus based gene therapy;Cardioprotectant
Technologies	Intra-arterial formulation;Infusion;Biological therapeutic;Gene transfer system viral
Last Change Date	12-Aug-2014

Mydicar DEVELOPMENT PROFILE

SUMMARY

Celladon, under license from Targeted Genetics, is developing Mydicar (AAV1/Serca2a), a gene therapy that uses an adeno-associated virus (AAV) vector technology to deliver the sarcoplasmic reticulum ATPase 2a (SERCA 2a) gene, for the potential treatment of congestive heart failure (CHF),. In July 2014, the company is also investigating Mydicar for the potential treatment of arteriovenous fistula. By January 2012, phase II/III studies had been initiated. In July 2014, a phase IIa trial for arteriovenous fistula was planned.

Celladon is also investigating a series of small-molecule SERCA 2a activators for the potential treatment of heart failure and an inhalant formulation of Mydicar, for the potential treatment of pulmonary arterial hypertension.

Targeted Genetics was previously evaluating a recombinant AAV vector to deliver the AC6 gene for the treatment of congestive heart failure.

Mydicar DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Celladon Corp	Congestive heart failure	US	Phase 3 Clinical	31-Jan-2012

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Company	Indication	Country	Development Status	Date
Celladon Corp	Congestive heart failure	Europe	Phase 2 Clinical	11-Dec-2012
Celladon Corp	Vascular fistula	US	Discovery	07-Jul-2014
AmpliPhi Biosciences Corp	Congestive heart failure	US	Discontinued	02-Mar-2009

Mydicar DRUG NAMES

Names	Type
gene therapy (SERCA 2a), Celladon/Targeted Genetics	
congestive heart failure AAV-based gene therapy, Targeted Genetics/Celladon	
sarcoplasmic reticulum ATPase 2a gene therapy (CHF), Celladon/Targeted Genetics	
AAV1/Serca2a	
SERCA 2a gene therapy (heart failure), Celladon/Targeted Genetics	
Mydicar	Trade Name

Mydicar 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
Congestive heart failure											
0	0	0	0	1	2	0	0	0	0	1	2
Systolic heart failure											
0	0	0	0	1	1	0	0	0	0	1	1
Cardiac failure											
0	0	0	0	1	1	0	0	0	0	1	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	0	0	3	4	0	0	0	0	3	4

Phase Definitions

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

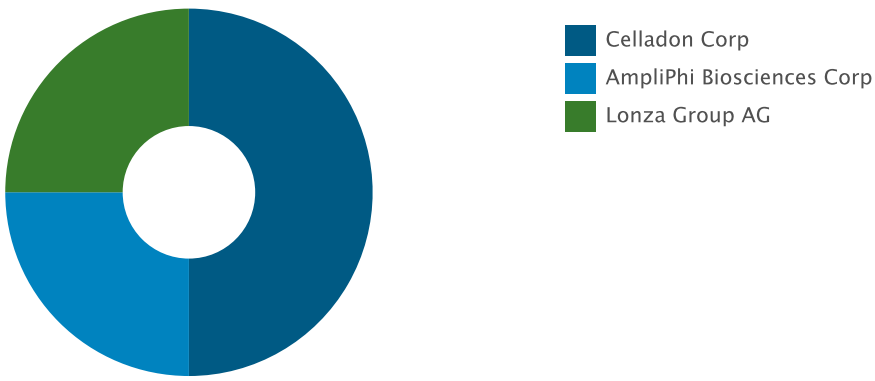
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

Mydicar DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 2

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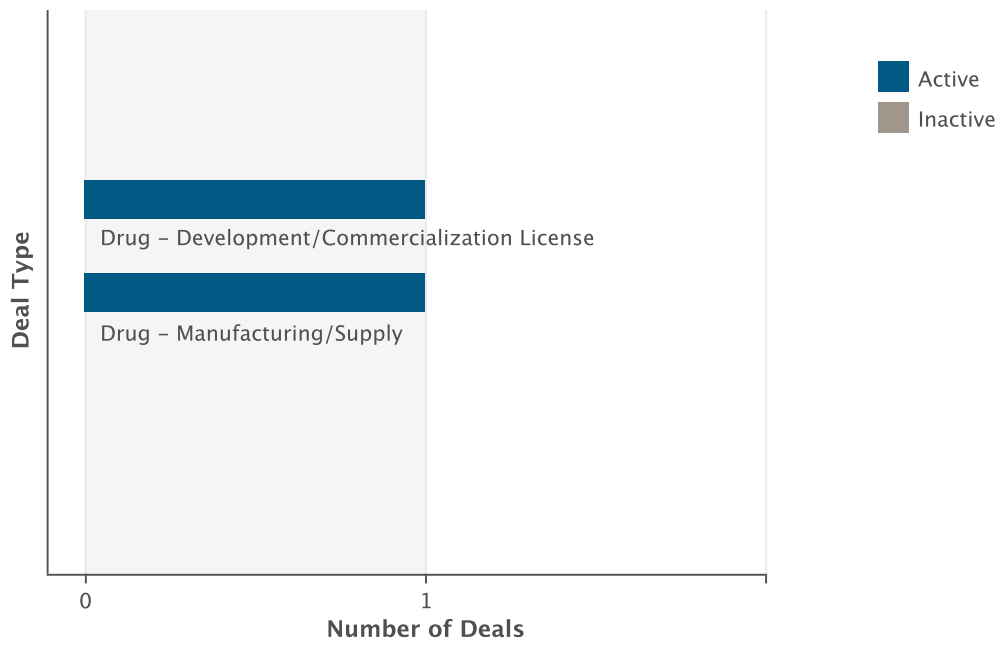
Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Celladon Corp	0	0	2	0	2
Lonza Group AG	1	0	0	0	1
AmpliPhi Biosciences Corp	1	0	0	0	1

Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	0	1
Drug - Manufacturing/Supply	1	0	1

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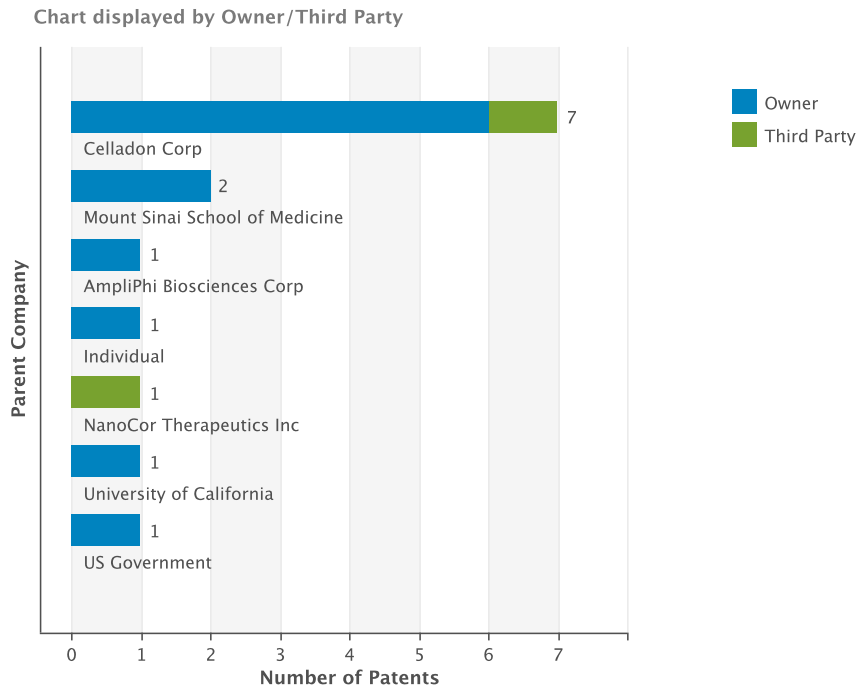
PATENTS

Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Celladon Corp	6	1	7
Mount Sinai School of Medicine	2	0	2
University of California	1	0	1
NanoCor Therapeutics Inc	0	1	1
Individual	1	0	1
AmpliPhi Biosciences Corp	1	0	1
US Government	1	0	1

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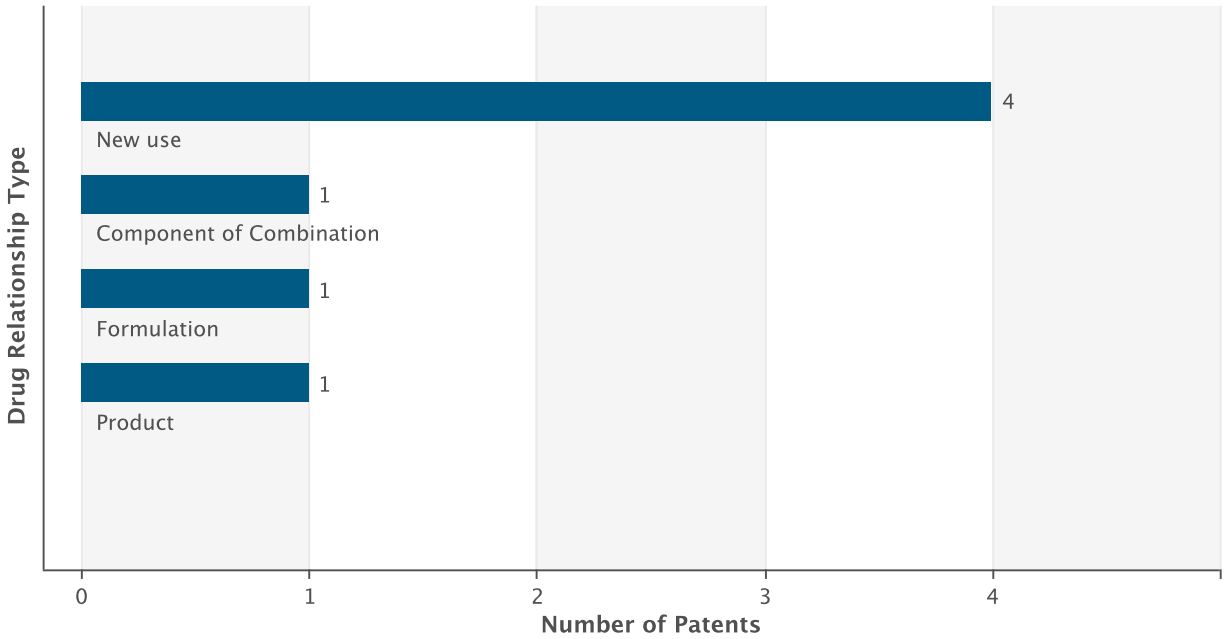
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	4
Formulation	1
Component of Combination	1
Product	1

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mSCF-based gene therapy (cardiac ischemia), Celladon

mSCF-based gene therapy (cardiac ischemia), Celladon SNAPSHOT

Drug Name	mSCF-based gene therapy (cardiac ischemia), Celladon
Key Synonyms	
Originator Company	Celladon Corp
Active Companies	Celladon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Ischemic heart disease
Target-based Actions	Kit ligand
Other Actions	Cardiac agent;Gene therapy
Technologies	Biological therapeutic
Last Change Date	28-Oct-2014

mSCF-based gene therapy (cardiac ischemia), Celladon DEVELOPMENT PROFILE

SUMMARY

Celladon, under a license from its investor Enterprise Partners Venture Capital, is investigating membrane-bound form of the stem cell factor gene (mSCF; Kit) as a gene therapy for the potential treatment of cardiac ischemia. In September 2014, preclinical data were published.

mSCF-based gene therapy (cardiac ischemia), Celladon DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Celladon Corp	Ischemic heart disease	US	Discovery	21-Jul-2014

mSCF-based gene therapy (cardiac ischemia), Celladon DRUG NAMES

Names	Type
mSCF-based gene therapy (cardiac ischemia), Celladon	

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SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon

SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon SNAPSHOT

Drug Name	SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon
Key Synonyms	Mydicar
Originator Company	Mount Sinai School of Medicine
Active Companies	Celladon Corp
Inactive Companies	Mount Sinai School of Medicine
Highest Status	Discovery
Active Indications	Pulmonary artery hypertension
Target-based Actions	Sarco endoplasmic calcium ATPase 2a modulator
Other Actions	Antihypertensive;Adeno-associated virus based gene therapy
Technologies	Inhalant formulation;Virus recombinant;Biological therapeutic;Gene transfer system viral
Last Change Date	18-Sep-2014

SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon DEVELOPMENT PROFILE

SUMMARY

Celladon, under license from Mount Sinai School of Medicine is investigating an inhalant formulation of Mydicar (AAV1/Serca2a), a gene therapy that uses an adeno-associated virus (AAV) vector technology to deliver the sarcoplasmic reticulum ATPase 2a (SERCA 2a) gene, for the potential treatment of pulmonary arterial hypertension (PAH). In July 2013, preclinical data were reported. At that time, preclinical studies in large animal models were underway and the company planned to initiate clinical trials in the 'near future'.

SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Celladon Corp	Pulmonary artery hypertension	US	Discovery	31-Dec-2012
Mount Sinai School of Medicine	Pulmonary artery hypertension	US	Discontinued	31-Dec-2012

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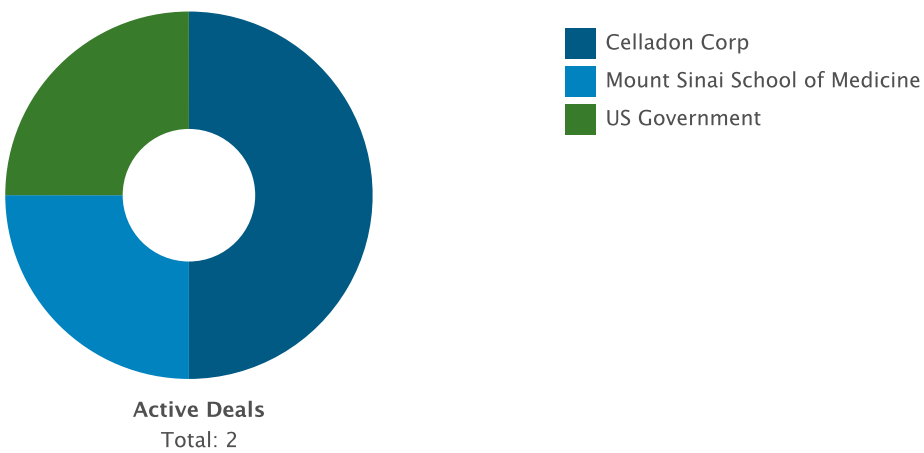
SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon DRUG NAMES

Names	Type
SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon	
SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Mount Sinai School of Medicine	
Mydicar	Trade Name
AAV1/Serca2a	

SERCA 2a gene therapy (inhalant, pulmonary artery hypertension), Celladon DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Celladon Corp	1	0	1	0	2
Mount Sinai School of Medicine	1	0	0	0	1
US Government	0	0	1	0	1

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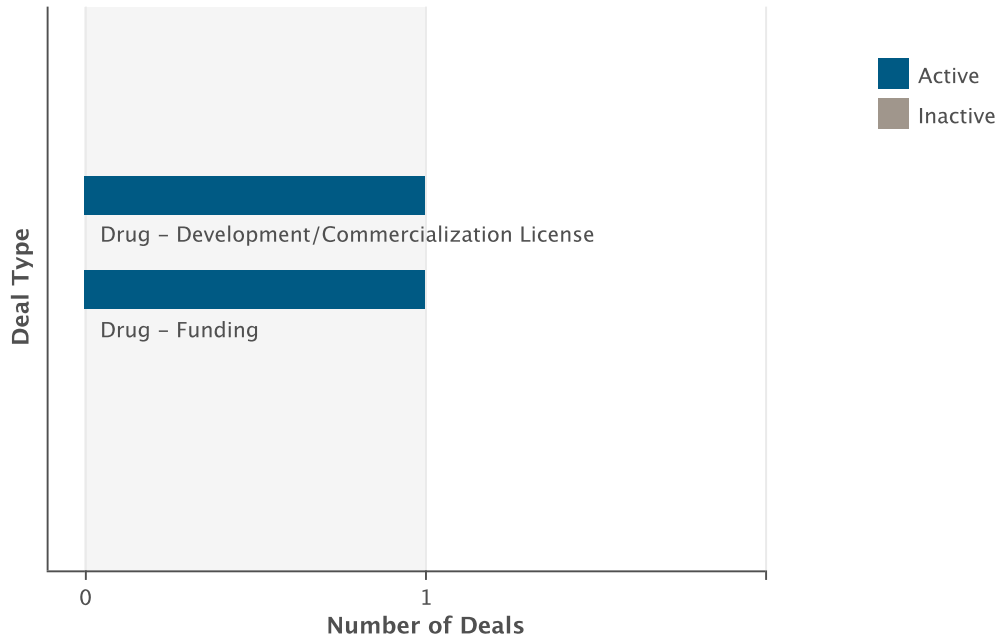
Deals by Type Chart



Deals by Type Table

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

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SERCA 2a activators (heart failure), Celladon

SERCA 2a activators (heart failure), Celladon SNAPSHOT

Drug Name	SERCA 2a activators (heart failure), Celladon
Key Synonyms	
Originator Company	Celladon Corp
Active Companies	Celladon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Cardiac failure
Target-based Actions	Sarco endoplasmic calcium ATPase 2a stimulator
Other Actions	Cardiac agent
Technologies	Small molecule therapeutic
Last Change Date	13-Sep-2011

SERCA 2a activators (heart failure), Celladon DEVELOPMENT PROFILE

SUMMARY

Celladon is investigating small molecule sarcoplasmic reticulum ATPase 2a (SERCA 2a) activators, including CDN-1054, CDN-1229 and CDN-1001, for the potential iv or oral treatment of heart failure. In September 2011, development was ongoing.

Celladon and Targeted Genetics are also developing Mydicar, an adeno-associated virus vector which delivers the SERCA 2a gene, for the potential treatment of congestive heart failure.

SERCA 2a activators (heart failure), Celladon DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Celladon Corp	Cardiac failure	US	Discovery	09-Nov-2008

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SERCA 2a activators (heart failure), Celladon DRUG NAMES

Names	Type
CDN-1229	Research Code
SERCA 2a activators (heart failure), Celladon	
CDN-1054	Research Code
CDN-1001	Research Code

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SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon

SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon SNAPSHOT

Drug Name	SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon
Key Synonyms	
Originator Company	Celladon Corp
Active Companies	Celladon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Diabetes mellitus;Neurodegenerative disease
Target-based Actions	Sarco endoplasmic calcium ATPase 2b stimulator
Other Actions	Hypoglycemic agent
Technologies	Small molecule therapeutic
Last Change Date	25-Feb-2014

SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon DEVELOPMENT PROFILE

SUMMARY

Celladon is investigating allosteric sarcoplasmic reticulum ATPase 2b (SERCA2b) agonists, which correct the Ca2+ imbalance in the endoplasmic reticulum, for the potential treatment of diabetes and neurodegenerative diseases.

SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Celladon Corp	Diabetes mellitus	US	Discovery	23-Jan-2013
Celladon Corp	Neurodegenerative disease	US	Discovery	24-Feb-2014

SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon DRUG NAMES

Names	Type
SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon	

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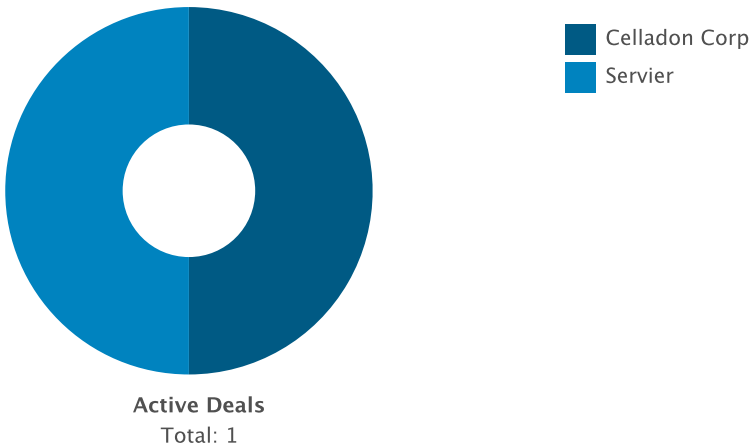
DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Servier	0	0	1	0	1
Celladon Corp	1	0	0	0	1

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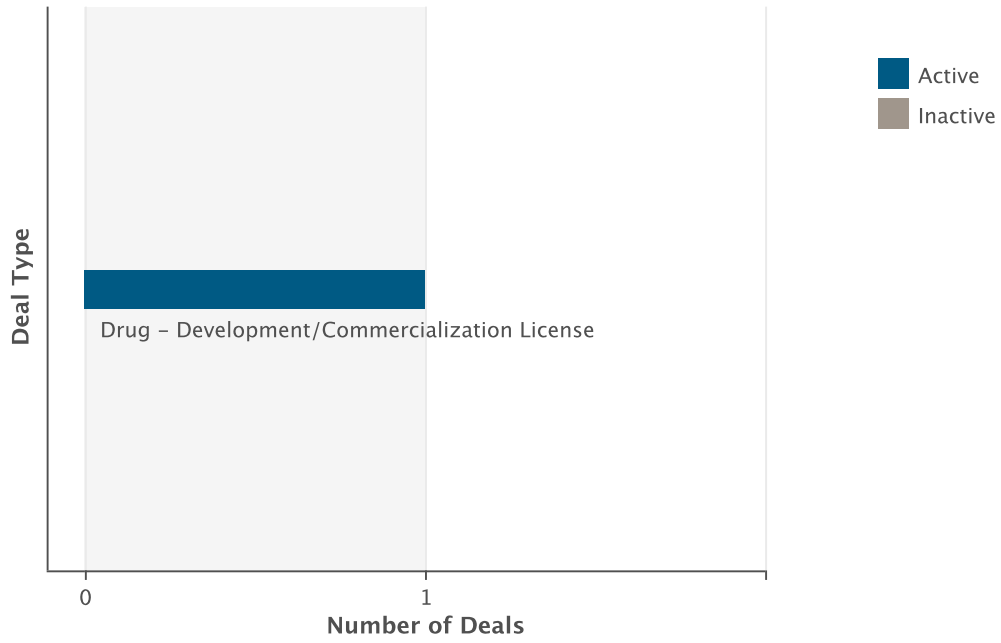
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	0	1

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