

# **Celladon Corp**

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

A comprehensive coverage of the company and a summary of the drug pipeline portfolio.

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# ABOUT COMPANY AND PIPELINE OVERVIEW REPORT

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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 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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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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**PLEASE NOTE:** the financials section where present in the report includes: Share Information, Stock Performance chart (including the consensus recommendation), and Major Shareholders. Financial Performance presents graphical and tabular data on Worldwide Sales, Operating Income and Net Income over time, together with a Quarterly earnings update. Balance Sheet lists Assets, Liabilities and Stockholders Equity, and Forecasts includes: EPS Forecast and Other Forecasts. The data reported in all sections (except share information and major stock holders) are correct as of the publication date of the report (and not the download date). For share information and major stock holders the data is correct for the date shown with these sections



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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, Virus recombinant, Biological therapeutic, Stem cell therapy, Gene transfer system viral, Drug screening, Assay, Fluorescence, Gene transfer system, Immunodetection, Labeling system, Peptide, Polynucleotide sequence, Protein fusion, Vector expression

#### **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.

#### **FINANCIAL**



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.

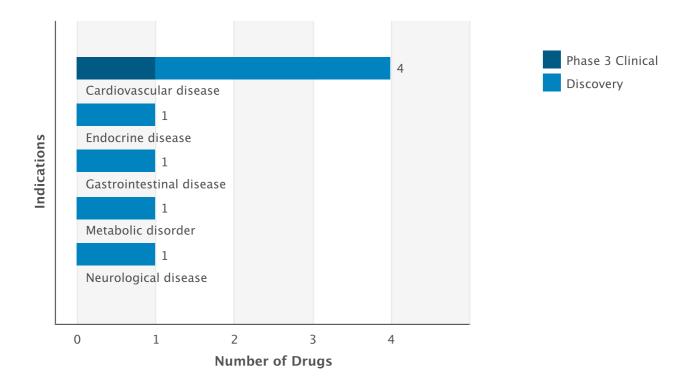
In December 2005, Celladon raised \$30 million in a series B venture financing.

#### PRODUCT PORTFOLIO SUMMARY

#### **DRUGS**

#### **Drugs by Indication**

Active Drugs by Indication Chart



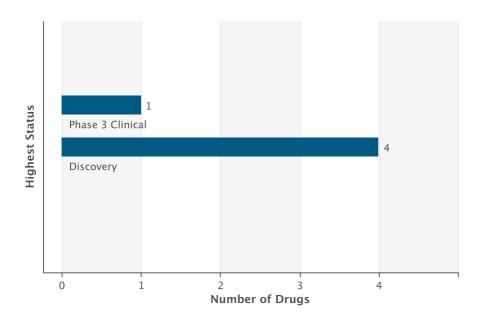


# Drugs by Indication Table

Indication	Active	Inactive	Total
Cardiovascular disease	4	0	4
Gastrointestinal disease	1	0	1
Neurological disease	1	0	1
Metabolic disorder	1	0	1
Endocrine disease	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	Prin	cipal	Pai	tner	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



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

<sup>\*</sup> 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

# **Mydicar**

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	Cardioprotectant, Adeno-associated virus based gene therapy
Technologies	Intra-arterial formulation, Infusion, Biological therapeutic, Gene transfer system viral
Last Change Date	12-Aug-2014

# SERCA 2a activators (heart failure), Celladon

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

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# SERCA2b agonists (diabetes/ neurodegenerative diseases), Celladon

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

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

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



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

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	Gene therapy, Cardiac agent
Technologies	Virus recombinant, Stem cell therapy, Biological therapeutic
Last Change Date	05-Dec-2014



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