

# **Epizyme Inc**

# **CORTELLIS COMPANY DETAILED PIPELINE REPORT**

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

Publication Date: 19-Jun-2013

# **THOMSON REUTERS**

3 Times Square New York, New York 10036 United States

Tel: +1 646 223 4000

thomsonreuters.com



# 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.

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

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

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

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

# DISCLAIMER

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

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



# **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.

# **TABLE OF CONTENTS**

Company Overview	5
Company Profile	6
Product Portfolio Summary	7
Product Portfolio Drug Pipeline Detail	10
Phase 2 Clinical	11
Phase 1 Clinical	16
Discovery	20



# **Epizyme Inc**

# **COMPANY OVERVIEW**

Company Name	Epizyme Inc
Parent Company Name	Epizyme Inc
Website	http://www.epizyme.com/
Country	US
Number of Drugs in Active Development	3
Number of Inactive Drugs	0
Number of Patents as Owner	8
Number of Patents as Third Party	3
Number of Deals	7
Key Indications	Cancer,Leukemia,Non-Hodgkin lymphoma,Neurological disease,Acute lymphoblastic leukemia,Acute myelogenous leukemia,Asthma,Atherosclerosis,Diabetes mellitus,Diffuse large B-cell lymphoma,Follicle center lymphoma,Hematological neoplasm,Hypercholesterolemia,Hyperinsulinemia,Hyperlipidemia,Inflammator y bowel disease,Insulin dependent diabetes,Insulin resistance,Lipid metabolism disorder,Multiple
Key Target-based Actions	Methyltransferase inhibitor,Enhancer of zeste homolog 2 inhibitor,Histone lysine methyltransferase inhibitor,DOT1L gene inhibitor,WHSC1 gene inhibitor,Histone H3 K79 methyltransferase inhibitor,AMP activated protein kinase modulator,Beta adrenoceptor modulator,CLK4 protein kinase inhibitor,Flt3 tyrosine kinase inhibitor,JMJD1A gene modulator,PCSK9 gene inhibitor,PDGF receptor alpha antagonist,PPAR alpha modulator,PPAR gamma coactivator 1-alpha modulator,Proprotein convertase PC9 inhibitor,Ros1 tyrosine kinase receptor inhibitor,TrkA receptor antagonist,TrkC
Key Technologies	Small molecule therapeutic,Oral formulation,Systemic formulation unspecified,Drug screening,Antibody,Drug combination,Gene transfer system,Genetic screening,Humanized antibody,Immunodetection,Immunofluorescence,Intravenous formulation,Liposome formulation,Oligonucleotide,PCR based

# **COMPANY PROFILE**

### **SUMMARY**

Epizyme Inc was established in 2007 and is focused on the development of drugs based on epigenetics.

### **FINANCIAL**

In June 2013, Epizyme closed its initial public offering of 5,913,300 shares of its common stock at a public offering price of \$15.00 per share, before underwriting discounts, including 771,300 shares of common stock issued upon the exercise in full by the underwriters of their option to purchase additional shares at the same price to cover over-allotments. At that time, the company expected net proceeds of approximately \$79.8 million.

In April 2013, Epizyme filed a registration statement on Form S-1 with the US Securities and Exchange Commission relating to the proposed initial public offering of common stock; at that time, the company had not determine the number of shares and the price range for the offering. In May 2013, Epizyme priced its IPO of 5,142,000 common stock shares at \$15 per share. The underwriters were also granted a 30-day overallotment option to purchase up to an additional 771,300 common stock shares. The offering was expected to close on June 05, 2013 and at that time, the shares were scheduled to begin trading on the NASDAQ Global Market on May 31, 2013 under the ticker symbol 'EPZM'. In June 2013, the offering closed. The underwriters exercised in full the overallotment purchase option; the company expected to raise net proceeds of approximately \$79.8 million.

In February 2008, the company raised \$14 million from a series A financing round.



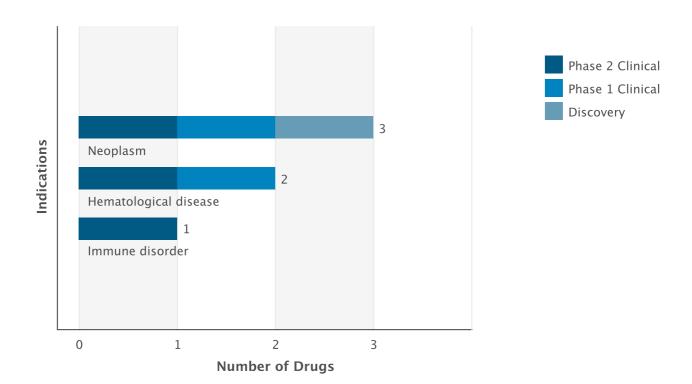
In October 2009, the company raised \$32 million in a series B financing round. In December 2009, the company raised a further \$8 million from a second closing of the series B financing round, bringing the total raised to \$40 million.

# PRODUCT PORTFOLIO SUMMARY

### **DRUGS**

# **Drugs by Indication**

Active Drugs by Indication Chart

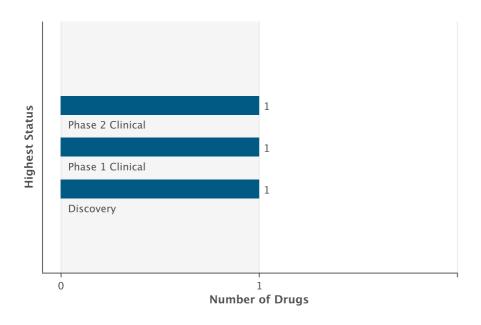


# Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	3	0	3
Hematological disease	2	0	2
Immune 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 2 Clinical	1
Phase 1 Clinical	1
Discovery	1

# **DEALS**

Deal Type	Prin	cipal	Pai	tner	Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	1	0	1	0	2
Drug - Funding	2	0	0	0	2
Drug - Development/Commercialization License	3	0	0	0	3
Drug - Development Services	0	0	1	0	1



## **CLINICAL TRIALS**

# Trials by Condition Studied

Condition Studied	Ongoing	All
Hematological disease	1	2
Neoplasm	1	2
Immune disorder	0	1

# Trials by Phase

Phase	Ongoing	All
Phase 1	1	2

### **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 1, 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	0	3	3
Endocrine disease	0	2	2
Gastrointestinal disease	0	2	2
Hematological disease	2	0	2
Immune disorder	1	1	2
Musculoskeletal disease	0	1	1
Neoplasm	8	1	9
Ocular disease	0	1	1
Metabolic disorder	0	3	3
Neurological disease	3	1	4
Nutritional disorder	0	1	1
Respiratory disease	0	1	1



Inflammatory disease	0	1	1
Dermatological disease	0	1	1

<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.



# PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

### E-7438

### E-7438 SNAPSHOT

Drug Name	E-7438
Key Synonyms	
Originator Company	Epizyme Inc
Active Companies	Eisai Co Ltd;Epizyme Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Cancer;Non-Hodgkin lymphoma
Target-based Actions	Enhancer of zeste homolog 2 inhibitor; Histone lysine methyltransferase inhibitor
Other Actions	Anticancer
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	14-Jun-2013

## **E-7438 DEVELOPMENT PROFILE**

# **SUMMARY**

Epizyme and licensee Eisai are developing E-7438 (EPZ-6438; structure shown), a lead from several small molecule EZH2 (enhancer of zeste homolog 2) inhibitors, including EPZ-5687 (EPZ-005687), from a program of histone methyltransferase inhibitors, for the potential oral treatment of cancers, including non-Hodgkin's lymphoma and breast cancer,.. In June 2013, a phase I/II trial was initiated.

### **E-7438 DEVELOPMENT STATUS**

### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Epizyme Inc	Non-Hodgkin lymphoma	US	Phase 2 Clinical	13-Jun-2013
Eisai Co Ltd	Cancer	Japan	Discovery	10-Mar-2011

# E-7438 CHEMICAL STRUCTURES

Confidence Level:
5
Туре
Research Code
Research Code

# E-7438 DRUG NAMES

Names	Туре
EPZ-005687	Research Code
EPZ-6438	Research Code
EZH2 inhibitors (cancer), EpiZyme/Eisai	
EPZ-5687	Research Code
E-7438	Research Code

# E-7438 CLINICAL TRIALS

# Trials by Phase and Condition Studied

	se 4 nical		se 3 nical		se 2 nical	Phase 1 Clinical		Phase Unspecified		Total	
On- going	All	On- going	All	On- going	All	On- going	All	On- going	All	On- going	All
Non-Hodgkin lymphoma											
0	0	0	0	0	0	0	1	0	0	0	1

# Total Trials by Phase and Status

	se 4 nical		se 3 nical		se 2 nical		Phase 1 Phase Clinical 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	0	0	0	1	0	0	0	1

### **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

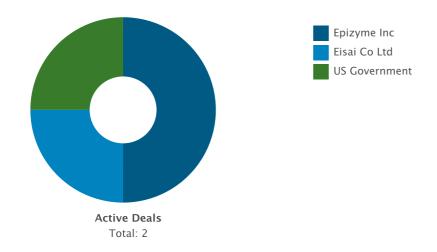
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

### Phase 1 Clinical

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

### **E-7438 DEALS AND PATENTS**

# DEALS Deals by Parent Company Chart

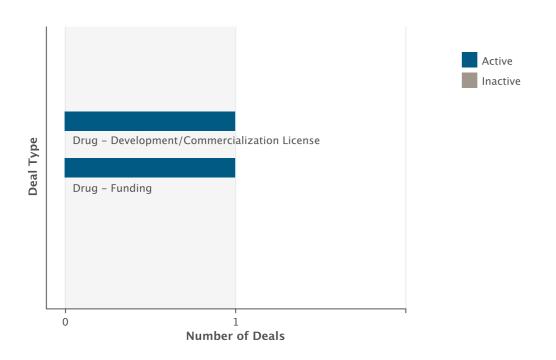




# **Deals by Parent Company Table**

Company Name	Principal Active Inactive		Partner Active Inactive		Total
Epizyme Inc	2	0	0	0	2
US Government	0	0	1	0	1
Eisai Co Ltd	0	0	1	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

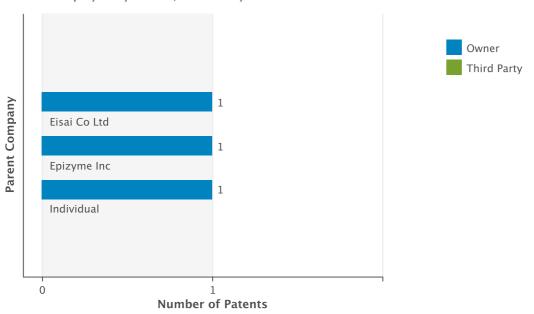
Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1
Drug - Development/Commercialization License	1	0	1



## **PATENTS**

# **Patents by Parent Company Chart**

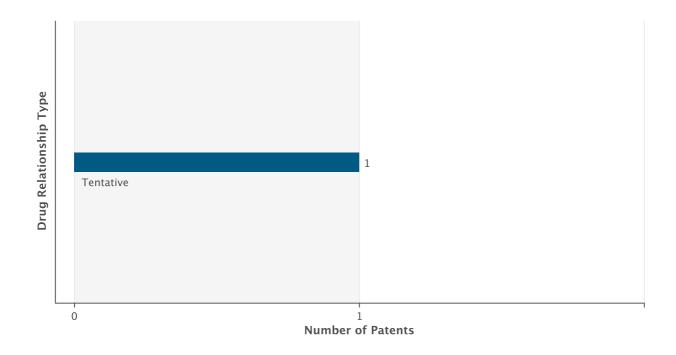
Chart displayed by Owner/Third Party



# **Patents by Parent Company Table**

Company Name	As Owner	As Third Party	Total
Eisai Co Ltd	1	0	1
Epizyme Inc	1	0	1
Individual	1	0	1

# **Patents by Drug Relationship Type Chart**



# **Patents by Drug Relationship Type Table**

Drug Relationship	Total
Tentative	1

# **EPZ-5676**

### **EPZ-5676 SNAPSHOT**

Drug Name	EPZ-5676
Key Synonyms	
Originator Company	Epizyme Inc
Active Companies	Celgene Corp;Epizyme Inc
Inactive Companies	GlaxoSmithKline plc
Highest Status	Phase 1 Clinical
Active Indications	Leukemia
Target-based Actions	DOT1L gene inhibitor;Methyltransferase inhibitor
Other Actions	Anticancer
Technologies	Systemic formulation unspecified;Small molecule therapeutic
Last Change Date	19-Apr-2013

## **EPZ-5676 DEVELOPMENT PROFILE**

# **SUMMARY**

Epizyme and ex-US licensee Celgene are developing EPZ-5676 (structure shown), a small molecule S-adenosyl methionine competitive DOT1L inhibitor, for the potential treatment of mixed lineage leukemia (MLL). In September 2012, a phase I US trial was initiated.

This compound may have emerged from a program of histone methyltransferase (HMT) inhibitors on which Epizyme collaborated with GlaxoSmithKline.

Epizyme is using Abbott's FISH technology to develop a DOT1L companion diagnostic for the drug.

# **EPZ-5676 DEVELOPMENT STATUS**

# **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Epizyme Inc	Leukemia	US	Phase 1 Clinical	05-Sep-2012
Celgene Corp	Leukemia	World	Discovery	05-Sep-2012

## **EPZ-5676 CHEMICAL STRUCTURES**



CAS Registry Number:	Confidence Level:
	4
T Z Z Z Z Z Z Z Z Z Z Z Z Z Z Z Z Z Z Z	NH <sub>2</sub> N N N N N N N N N N N N N N N N N N N
Name	Туре
EPZ-5676	Research Code

# **EPZ-5676 DRUG NAMES**

Names	Туре
DOT1L inhibitors (mixed lineage leukemia), Epizyme/GlaxoSmithKline	
EPZ-5676	Research Code
EPZ-004777	Research Code
DOT1L inhibitors (leukemia), Epizyme/GSK	

# **EPZ-5676 CLINICAL TRIALS**

# Trials by Phase and Condition Studied

Pha Clin			se 3 nical	Pha Clin	se 2 nical	Pha Clir	se 1 lical	Pha Unspe		То	tal
On- going	All	On- going	All	On- going	All	On- going	All	On- going	All	On- going	All
Leukemia											
0	0	0	0	0	0	1	1	0	0	1	1
Hematological neoplasm											
0	0	0	0	0	0	1	1	0	0	1	1

## Total Trials by Phase and Status

Phase 4 Clinical			se 3 nical	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	0	0	1	1	0	0	1	1

## **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

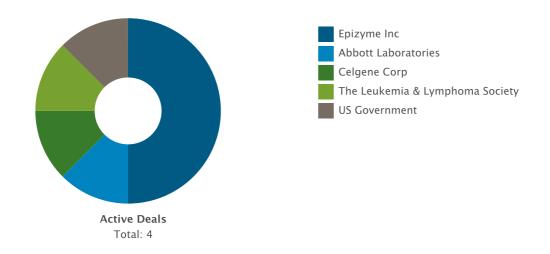
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

### Phase 1 Clinical

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

## **EPZ-5676 DEALS AND PATENTS**

# DEALS Deals by Parent Company Chart





# **Deals by Parent Company Table**

Company Name	Prin Active	cipal Inactive		tner Inactive	Total
Epizyme Inc	2	0	2	0	4
US Government	0	0	1	0	1
Abbott Laboratories	1	0	0	0	1
The Leukemia & Lymphoma Society	1	0	0	0	1
Celgene Corp	0	0	1	0	1

# **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
Technology - Other Proprietary	1	0	1
Drug - Development Services	1	0	1



# histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline

# histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline SNAPSHOT

Drug Name	histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline
Key Synonyms	
Originator Company	Epizyme Inc
Active Companies	GlaxoSmithKline plc;Epizyme Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	WHSC1 gene inhibitor;Methyltransferase inhibitor
Other Actions	Anticancer
Technologies	Small molecule therapeutic
Last Change Date	28-Apr-2012

histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline DEVELOPMENT PROFILE

## **SUMMARY**

Epizyme and licensee GlaxoSmithKline (GSK) are investigating a program of small molecule histone methyltransferase (HMT) inhibitors, presumably including WHSC1 inhibitors, for the potential treatment of cancer and other diseases,. In October 2009, the company was planning proof-of-concept studies. In November 2010, development of the program was ongoing ; in April 2012, this was still the case.

histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline DEVELOPMENT STATUS

# **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Epizyme Inc	Cancer	US	Discovery	08-Oct-2009
GlaxoSmithKline plc	Cancer	UK	Discovery	10-Jan-2011

## histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline DRUG NAMES

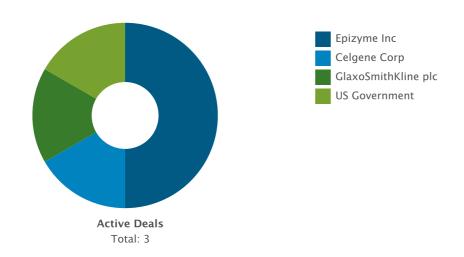
Names	Туре
WHSC1 inhibitors (cancer), Epizyme/GSK	
histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline	



# histone methyltransferase inhibitors (cancer), Epizyme/GlaxoSmithKline DEALS AND PATENTS

DEALS

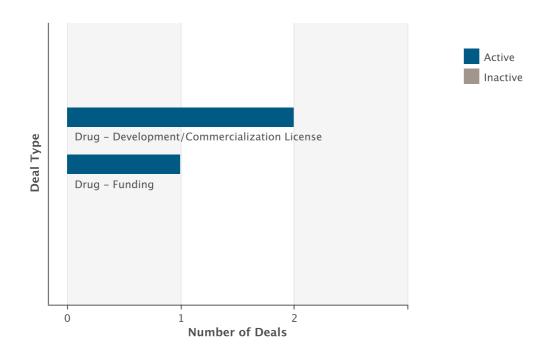
Deals by Parent Company Chart



# **Deals by Parent Company Table**

Company Name	Prin Active	cipal Inactive	Par Active	tner Inactive	Total
Epizyme Inc	3	0	0	0	3
Celgene Corp	0	0	1	0	1
US Government	0	0	1	0	1
GlaxoSmithKline plc	0	0	1	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	2	0	2
Drug - Funding	1	0	1

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

For more information about *Cortellis for Competitive Intelligence*, visit: http://cortellis.thomsonreuters.com/cortellis\_for\_you/?cid=thomsonone.

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

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

THOMSON REUTERS