

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

COMPANY OVERVIEW

Company Name	Agios Pharmaceuticals Inc
Parent Company Name	Agios Pharmaceuticals Inc
Website	http://www.agios.com/
Country	US
Number of Drugs in Active Development	6
Number of Inactive Drugs	2
Number of Patents as Owner	24
Number of Patents as Third Party	1
Number of Deals	9
Key Indications	Cancer, Glutaric aciduria, Inborn error of metabolism, Metabolic disorder, Acute myelogenous leukemia, Colon tumor, Cholangiocarcinoma, Chondrosarcoma, Glioblastoma, Melanoma, Myelodysplastic syndrome, Myeloproliferative disorder, Non-small-cell lung cancer, Sickle
Key Target-based Actions	IDH1 gene inhibitor, IDH2 gene inhibitor, Pyruvate kinase stimulator, Pyruvate kinase muscle isozyme inhibitor, Pyruvate kinase muscle isozyme stimulator, Cytoplasmic isocitrate dehydrogenase inhibitor, Isocitrate dehydrogenase inhibitor, Pyruvate kinase 2 modulator, AMP activated protein kinase stimulator, Cytoplasmic isocitrate dehydrogenase modulator, EGFR gene modulator, IDH1 gene modulator, IDH2 gene modulator, KRAS gene modulator, Mitochondrial isocitrate dehydrogenase inhibitor, Mitochondrial isocitrate dehydrogenase modulator, Pyruvate kinase 2 stimulator, Pyruvate kinase muscle isozyme modulator
Key Technologies	Small molecule therapeutic, Oral formulation, Drug screening, Enzyme, Analytical method, Animal model, Chromatography analytical, DNA sequencing technology, Diagnostic method, Fluorescence, Immunodetection, Luminescence, Spectrometry

COMPANY PROFILE

SUMMARY

Agios Pharmaceuticals, founded as part of a seed financing by ARCH Venture Partners and Flagship Ventures, is a biopharmaceutical company focused on the development of therapeutics which target key metabolic pathways in order to disrupt the growth and survival of cancer cells.

COMPANY LOCATION

In November 2008, Agios opened a 21,000 sq ft state-of-the-art laboratory dedicated to cancer metabolism research in Cambridge, MA.

LICENSING AGREEMENTS

In December 2009, ABC2 (Accelerate Brain Cancer Cure) provided funding to Agios Pharmaceuticals for the development of IDH1 therapeutics and diagnostics in brain cancer (glioma). Agios received undisclosed funding.

FINANCIAL

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

In September 2013, Agios was added to Russell 2000, Russell 3000 and Russell Global Indexes.

In July 2013, Celgene purchased \$12.8 million of Agios common stock in a private placement.

In July 2013, the company priced its initial public offering of 5,888,888 common stock shares at \$18.00, each before underwriting discounts. Also, the underwriters were granted a 30-day option to buy up to 883,333 additional common

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stock shares. The offering was expected to close on or about July 29, 2013. At that time, the company had received approval to list its common stock shares on the NASDAQ Global Select Market, under the ticker symbol 'AGIO' and expected to begin trading on July 24, 2013. In July 2013, the company began trading on the NASDAQ stock market. Later that month, Agios closed the IPO raising net proceeds of \$111 million.

In November 2011, Agios raised \$78 million from an oversubscribed series C financing.

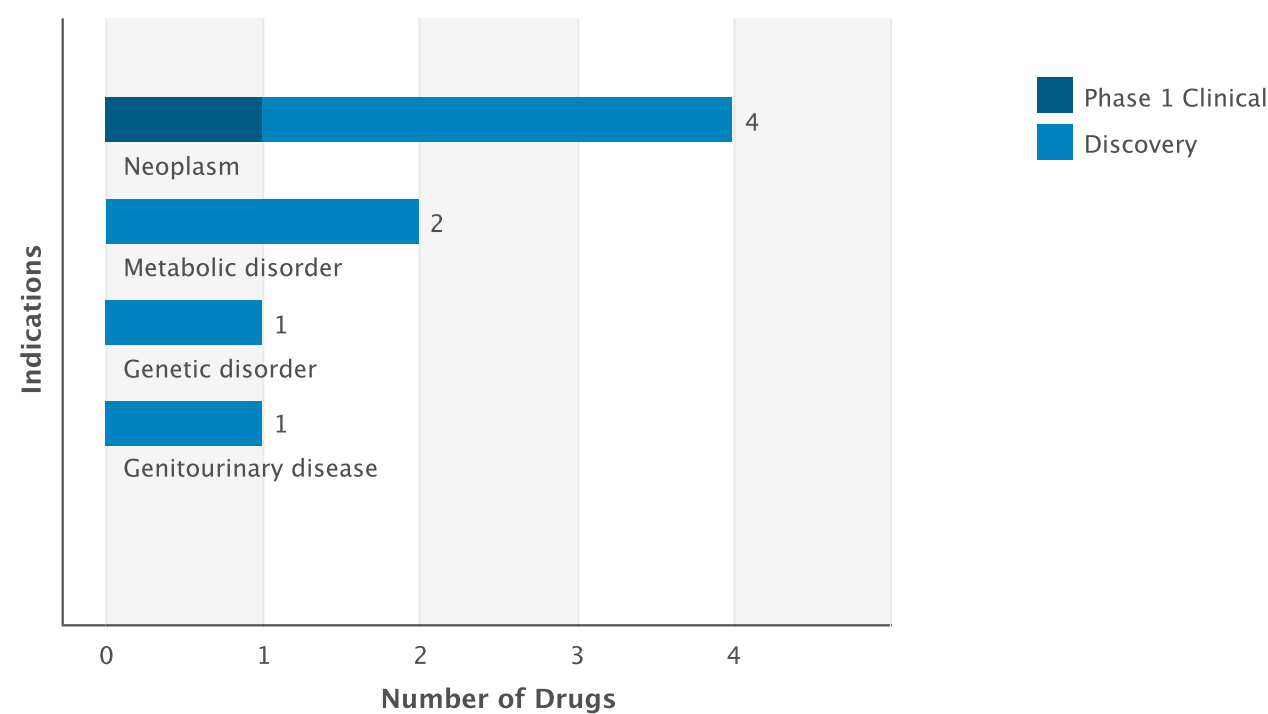
In July 2008, Agios raised \$33 million from a series A financing round. Proceeds would be used to advance development of the company's pipeline.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



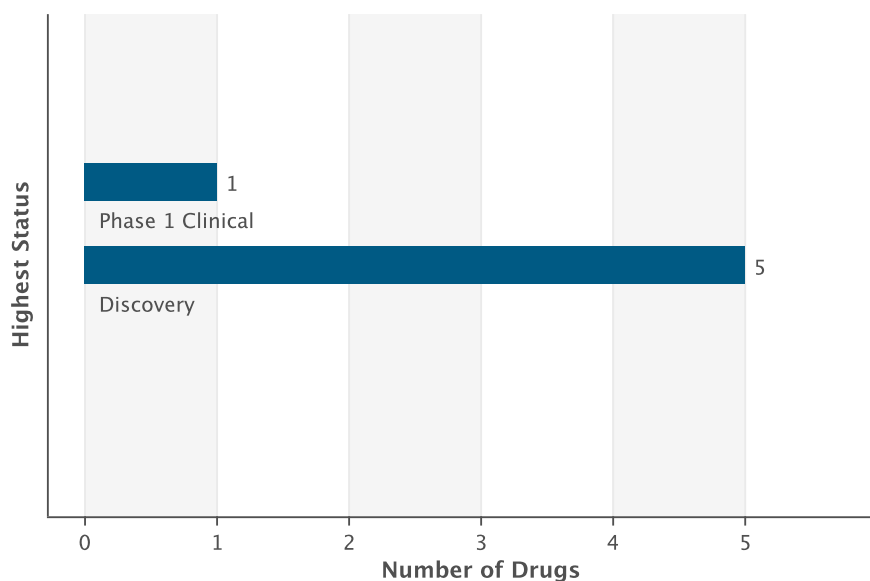
Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	4	2	6
Metabolic disorder	2	0	2
Genetic disorder	1	0	1
Genitourinary disease	1	0	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
Phase 1 Clinical	1
Discovery	5
No Development Reported	2

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	1	0	1
Patent - Exclusive Rights	0	0	1	0	1
Drug - Funding	3	0	0	0	3
Drug - Early Research/Development	0	0	1	0	1
Drug - Development/Commercialization License	1	0	1	0	2
Drug - Development Services	0	0	1	0	1

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CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	1	1
Hematological disease	1	1

Trials by Phase

Phase	Ongoing	All
Phase 1	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 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
Endocrine disease	1	0	1
Gastrointestinal disease	7	0	7
Genitourinary disease	2	0	2
Hematological disease	12	0	12
Andrology	2	0	2
Immune disorder	1	0	1
Musculoskeletal disease	6	0	6
Neoplasm	19	1	20
Genetic disorder	5	0	5
Neurological disease	1	0	1
Respiratory disease	6	0	6
Gynecology and obstetrics	1	0	1
Dermatological disease	5	0	5

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* 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

AG-221

AG-221 SNAPSHOT

Drug Name	AG-221
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Agios Pharmaceuticals Inc;Celgene Corp
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Glutaric aciduria;Cancer
Target-based Actions	IDH2 gene inhibitor
Other Actions	CD15 agonist;Anticancer
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	14-Feb-2014

AG-221 DEVELOPMENT PROFILE

SUMMARY

Agios and Celgene are developing AG-221, a mutated isocitrate dehydrogenase-2 (IDH-2m) inhibitor, from a program of isocitrate dehydrogenase (IDH) inhibitors (Agios IDH), for the potential oral treatment of cancers, including acute myeloid leukemia ,,,. The companies are also investigating the drug for type II D-2 hydroxyglutaric aciduria. In August 2013, a phase I trial in advanced hematologic cancer subjects with IDH-2 mutation was initiated in the US. In July 2013, the program was listed as being under development for type II D-2 hydroxyglutaric aciduria. In January 2014, the company was expecting to complete proof-of-concept studies for cancer in 2015. At that time, late development was expected to initiate in 2015 and complete in 2017.

AG-221 DEVELOPMENT STATUS

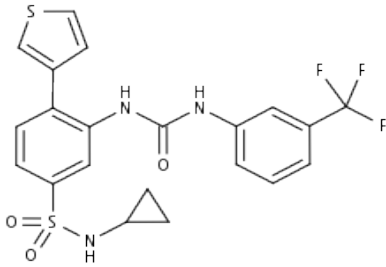
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Cancer	US	Phase 1 Clinical	08-Aug-2013
Celgene Corp	Cancer	US	Phase 1 Clinical	08-Aug-2013
Agios Pharmaceuticals Inc	Glutaric aciduria	US	Discovery	26-Jul-2013
Celgene Corp	Glutaric aciduria	US	Discovery	26-Jul-2013

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AG-221 CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
	4
	
Name	Type
AGI-6780	Research Code

AG-221 DRUG NAMES

Names	Type
isocitrate dehydrogenase-2 inhibitor (oral, cancer/type II D-2 hydroxyglutaric aciduria), Agios/Celgene AGI-6780	
	Research Code
AG-221	Research Code

AG-221 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
Hematological neoplasm											
0	0	0	0	0	0	1	1	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	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)

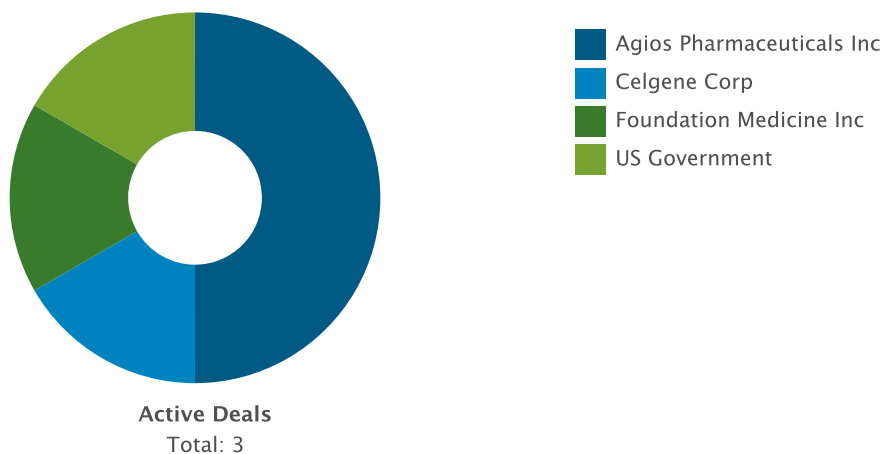
Phase 1 Clinical

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

AG-221 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

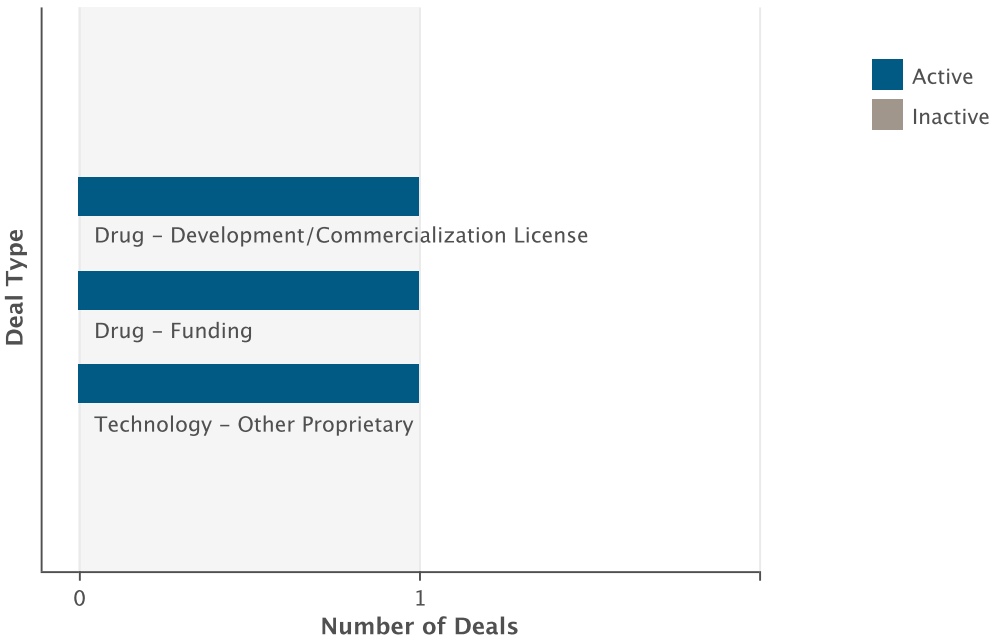


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Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Agios Pharmaceuticals Inc	2	0	1	0	3
Foundation Medicine Inc	1	0	0	0	1
Celgene Corp	0	0	1	0	1
US Government	0	0	1	0	1

Deals by Type Chart



Deals by Type Table

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

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AG-120

AG-120 SNAPSHOT

Drug Name	AG-120
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Celgene Corp;Agios Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	IDH1 gene inhibitor
Other Actions	Anticancer
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	14-Feb-2014

AG-120 DEVELOPMENT PROFILE

SUMMARY

Agios and Celgene are investigating AG-120 (presumed to be AGI-14100), a mutated isocitrate dehydrogenase-1 (IDH-1) inhibitor, from a program of isocitrate dehydrogenase (IDH) inhibitors (Agios IDH), for the potential oral treatment of cancer,,,. By April 2010, preclinical development was underway ; in November 2011, the program was in lead optimization. By January 2014, an IND had been filed and first-in-human phase I studies in patients with advanced solid and hematological malignancies were planned to start in early 2014. At that time, proof-of-concept studies were expected to initiate in 2014 and complete in 2016; late development was expected to initiate in 2016 and complete in 2017.

AG-120 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Cancer	US	Discovery	10-Dec-2009
Celgene Corp	Cancer	US	Discovery	15-Apr-2010

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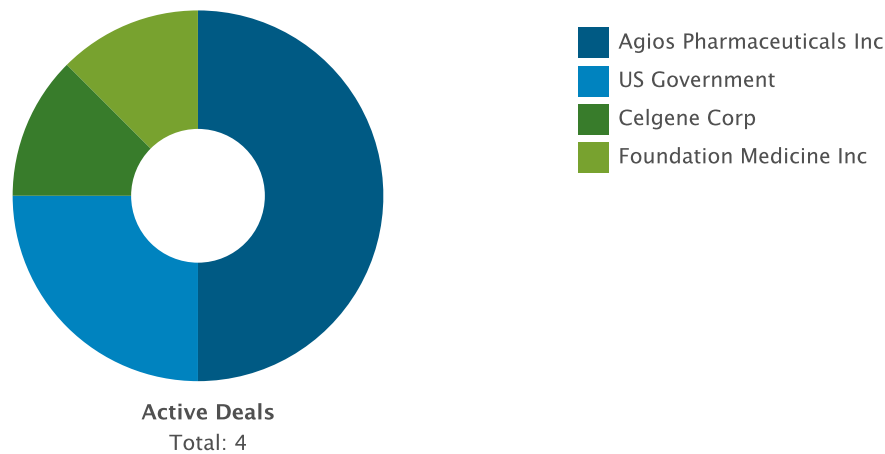
AG-120 DRUG NAMES

Names	Type
Agios IDH	
isocitrate dehydrogenase-1 inhibitor (oral, cancer), Agios/Celgene	
IDH inhibitors (cancer), Agios/Celgene	
isocitrate dehydrogenase inhibitors (cancer), Agios/Celgene	
AGI-14100	Research Code
AG-120	Research Code
IDH-1/IDH-2 inhibitors (cancer), Agios/Celgene	

AG-120 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

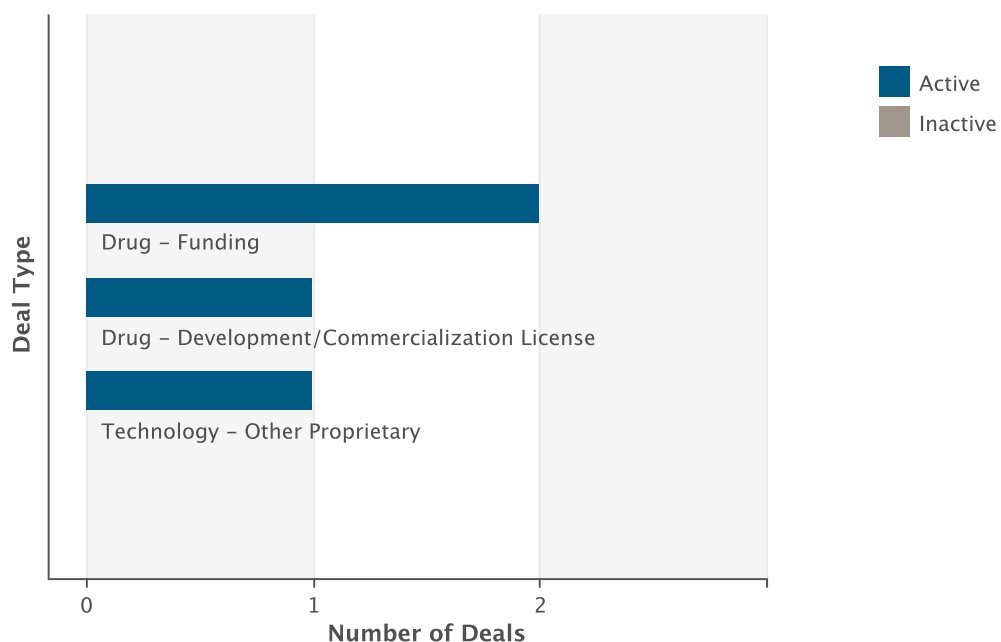


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Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Agios Pharmaceuticals Inc	3	0	1	0	4
US Government	0	0	2	0	2
Foundation Medicine Inc	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	2	0	2
Technology - Other Proprietary	1	0	1
Drug - Development/Commercialization License	1	0	1

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AG-348

AG-348 SNAPSHOT

Drug Name	AG-348
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Agios Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Metabolic disorder
Target-based Actions	Pyruvate kinase stimulator
Other Actions	
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	14-Feb-2014

AG-348 DEVELOPMENT PROFILE

SUMMARY

Agios Pharmaceuticals is investigating AG-348, an oral small molecule activator of pyruvate kinase (PK) receptor, for the potential treatment of PK deficiency. In July 2013, the drug was in preclinical development. By November 2013, IND-enabling preclinical studies have been initiated . By January 2014, IND activities have been substantially completed and a natural history study was being launched; at that time, the company was planning to initiate first-in-human trials in mid-2014.

AG-348 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Metabolic disorder	US	Discovery	26-Jul-2013

AG-348 DRUG NAMES

Names	Type
AG-348	Research Code
pyruvate kinase activator (oral, PK deficiency), Agios Pharmaceuticals	

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PKM2 inhibitors (cancer), Agios/Celgene

PKM2 inhibitors (cancer), Agios/Celgene SNAPSHOT

Drug Name	PKM2 inhibitors (cancer), Agios/Celgene
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Celgene Corp;Agios Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	Pyruvate kinase muscle isozyme inhibitor
Other Actions	Anticancer;Glycolysis pathway modulator
Technologies	Small molecule therapeutic
Last Change Date	21-Jun-2013

PKM2 inhibitors (cancer), Agios/Celgene DEVELOPMENT PROFILE

SUMMARY

Agios and Celgene are investigating pyruvate kinase M2 (PKM2) inhibitors for the potential treatment of cancer. Preclinical development was underway by April 2010 ; in November 2011, the program was in lead optimization ; data were published in September 2012.

PKM2 inhibitors (cancer), Agios/Celgene DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Cancer	US	Discovery	09-Jun-2009
Celgene Corp	Cancer	US	Discovery	15-Apr-2010

PKM2 inhibitors (cancer), Agios/Celgene DRUG NAMES

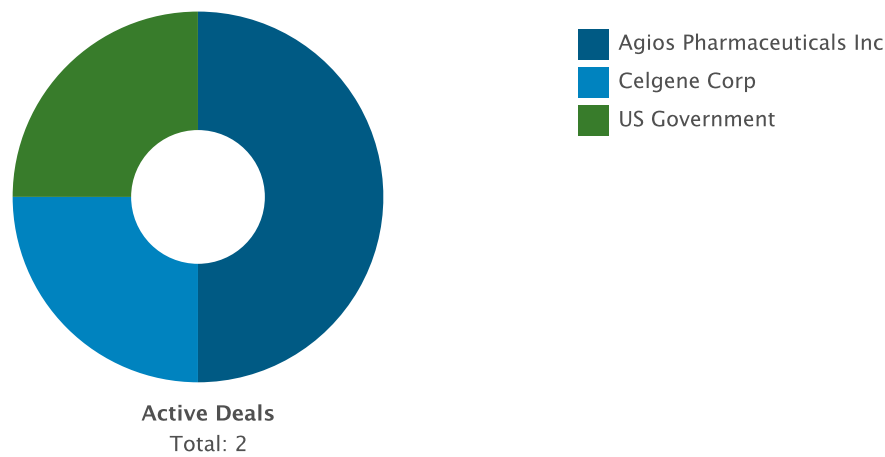
Names	Type
AGX-257	Research Code
pyruvate kinase M2 inhibitors (cancer), Agios/Celgene	
PKM2 inhibitors (cancer), Agios/Celgene	

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PKM2 inhibitors (cancer), Agios/Celgene DEALS AND PATENTS

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Deals by Parent Company Chart

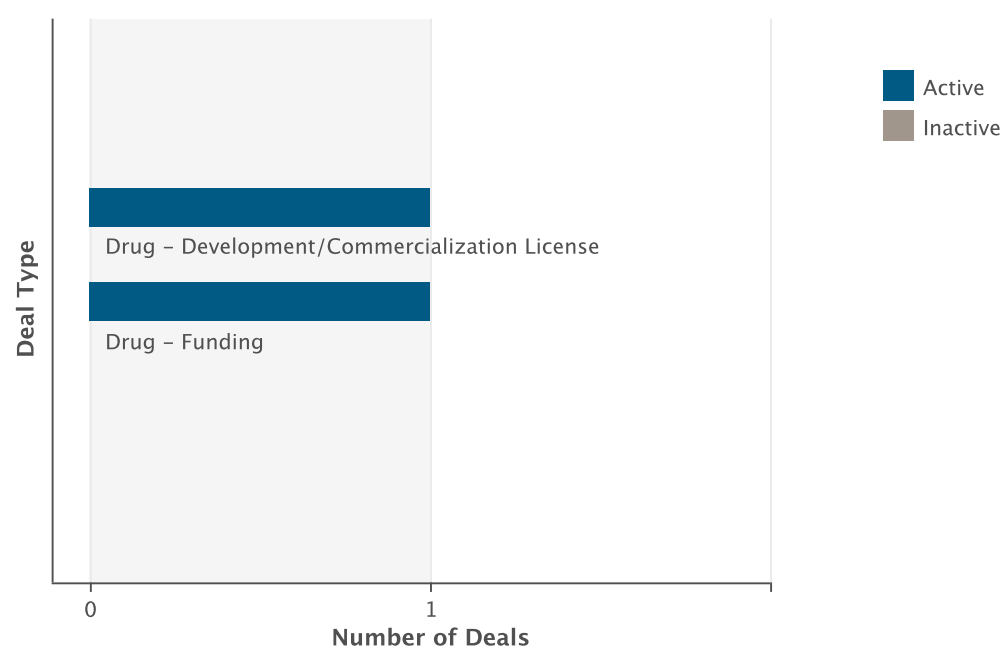


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Agios Pharmaceuticals Inc	2	0	0	0	2
Celgene Corp	0	0	1	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 - Development/Commercialization License	1	0	1
Drug - Funding	1	0	1

small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals

small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals SNAPSHOT

Drug Name	small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Agios Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Inborn error of metabolism
Target-based Actions	
Other Actions	Unspecified drug target
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	26-Jul-2013

small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals DEVELOPMENT PROFILE

SUMMARY

Agios Pharmaceuticals is investigating small molecule therapeutics for the potential oral treatment of inborn errors of metabolism,. By July 2013, the company had initiated exploratory and early target validation for four programs. In July 2012, the company planned to advance the program into clinic. In February 2013, the company was seeking to outlicense the program.

small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Inborn error of metabolism	US	Discovery	05-Jul-2012

small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals DRUG NAMES

Names	Type
small molecule therapeutics (oral, inborn errors of metabolism), Agios Pharmaceuticals	
small molecule therapeutics (inborn errors of metabolism), Agios Pharmaceuticals	

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cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene

cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene SNAPSHOT

Drug Name	cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene
Key Synonyms	
Originator Company	Agios Pharmaceuticals Inc
Active Companies	Agios Pharmaceuticals Inc; Celgene Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Anticancer
Technologies	Oral formulation; Small molecule therapeutic
Last Change Date	27-Jul-2013

cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene DEVELOPMENT PROFILE

SUMMARY

Agios Pharmaceuticals, in collaboration with Celgene, is investigating a cancer metabolism inhibitor, for the potential oral treatment of cancer. In July 2013, the program was in discovery.

cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Agios Pharmaceuticals Inc	Cancer	US	Discovery	26-Jul-2013
Celgene Corp	Cancer	US	Discovery	26-Jul-2013

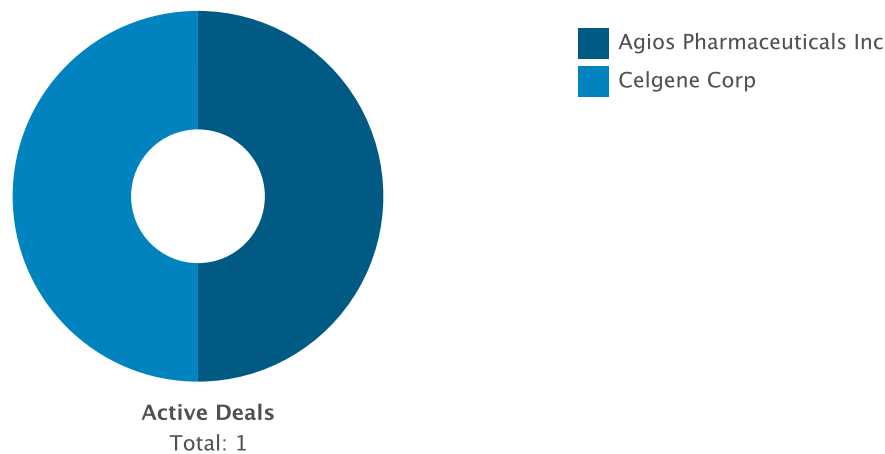
cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene DRUG NAMES

Names	Type
cancer metabolism inhibitor (oral, cancer), Agios Pharmaceuticals/Celgene	

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DEALS

Deals by Parent Company Chart

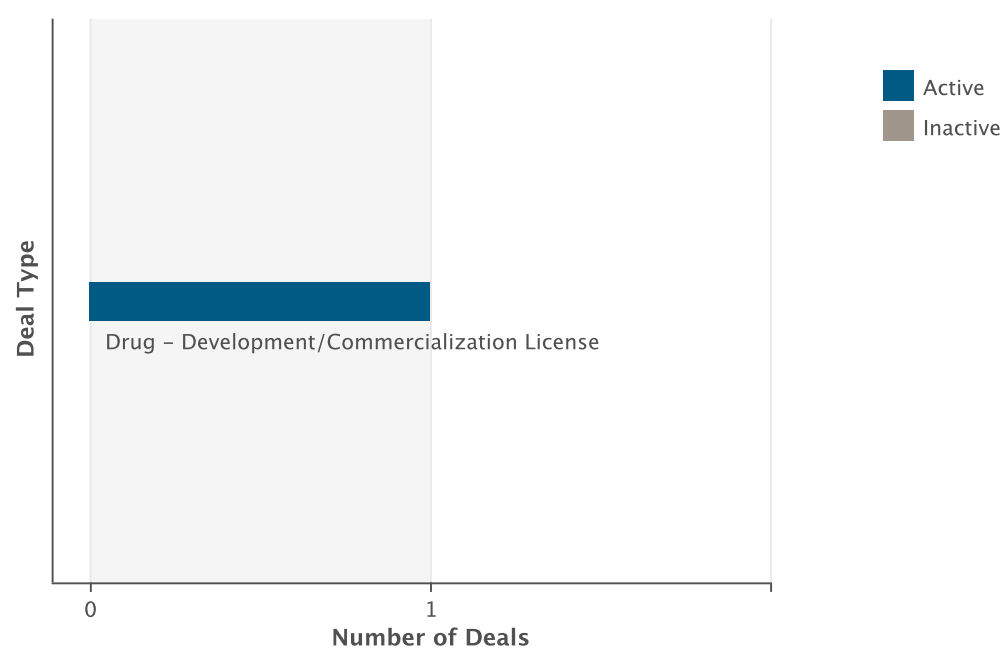


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Celgene Corp	0	0	1	0	1
Agios Pharmaceuticals Inc	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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