

## Dicerna Pharmaceuticals Inc

### CORTELLIS COMPANY DETAILED PIPELINE REPORT

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

Publication Date: 15-Dec-2014

#### THOMSON REUTERS

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

[thomsonreuters.com](http://thomsonreuters.com)

[Return to Table of Contents](#)

## 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.

[Return to Table of Contents](#)



## 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.

[Return to Table of Contents](#)



TABLE OF CONTENTS

Company Overview..... 5

Company Profile..... 6

Product Portfolio Summary..... 7

Product Portfolio Drug Pipeline Detail..... 10

    Phase 1 Clinical..... 11

    Discovery..... 14

[Return to Table of Contents](#)

# Dicerna Pharmaceuticals Inc

## COMPANY OVERVIEW

<b>Company Name</b>	Dicerna Pharmaceuticals Inc
<b>Parent Company Name</b>	Dicerna Pharmaceuticals Inc
<b>Website</b>	<a href="http://www.dicerna.com/">http://www.dicerna.com/</a>
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	8
<b>Number of Inactive Drugs</b>	11
<b>Number of Patents as Owner</b>	23
<b>Number of Patents as Third Party</b>	1
<b>Number of Deals</b>	5
<b>Key Indications</b>	Cancer,Alpha-1 antitrypsin deficiency,Blood clotting disorder,Hyperoxaluria,Lung tumor,Breast tumor,Ovary tumor,Pancreas tumor,Renal tumor,Uterine cervix tumor,Viral infection
<b>Key Target-based Actions</b>	Androgen receptor modulator,Epidermal growth factor receptor modulator,TNF gene inhibitor,Alpha 1 antitrypsin modulator,Beta-catenin modulator,E6 gene inhibitor,E7 gene inhibitor,Factor IIa antagonist,Hypoxia inducible factor-1 alpha modulator,Hypoxia inducible factor-2 alpha inhibitor,K-Ras GTPase inhibitor,Myc proto-oncogene protein inhibitor,Oxidase inhibitor
<b>Key Technologies</b>	Oligonucleotide,Biological therapeutic,Parenteral formulation unspecified,Lipid,Intravenous formulation,Liposome formulation,PEGylated formulation,Infusion,Nanoparticle formulation,Nanoparticle formulation injectable,Small molecule therapeutic

## COMPANY PROFILE

### SUMMARY

Dicerna Pharmaceuticals is a private, venture-backed company that develops RNAi-based therapies based on its dicer substrate technology platform.

### LICENSING AGREEMENTS

In July 2009, Dicerna and Archemix entered into a cross-license agreement for their respective platforms, combining Dicerna's Dicer Substrate RNAi (DsiRNA) gene-silencing technology, and Archemix's intracellular delivery-enabling aptamer technology, with the aim of generating aptamer-DsiRNA therapeutics. The two companies would codevelop the technologies, and any emergent candidate drugs would be exclusively developed by Dicerna.

In March 2009, Dicerna Pharmaceuticals secured the exclusive, worldwide right to grant sublicenses to the Dicer Substrate RNAi (DsiRNA) intellectual property estate which it is licensed from the technology's inventor, and Dicerna cofounder, Dr John Rossi of City of Hope and Integrated DNA Technologies.

### EARLY R&D

In March 2010, Dicerna Pharmaceuticals and Ipsen entered an exclusive research collaboration to develop novel conjugates of Dicerna's DsiRNA molecules and Ipsen's peptide targeting vectors for oncology and endocrinology therapy areas. The companies could then collaborate further to advance the programs discovered into development and commercialization. Financial details were undisclosed.

### FINANCIAL

In January 2014, Dicerna priced an IPO of 6 million shares at \$15 per share and granted underwriters a 30-day option to purchase an additional 0.9 million shares. The offering was expected to close on February 04, 2014; in February 2014, the offering of 6.9million shares was closed with an expected net proceeds of approximately \$92.9 million.

In August 2013, Dicerna secured \$60 million in oversubscribed series C financing.

[Return to Table of Contents](#)



In August 2010, the company closed a \$25 million series B financing round. In October 2010, the company raised an additional \$4 million from a second round of series B financing.

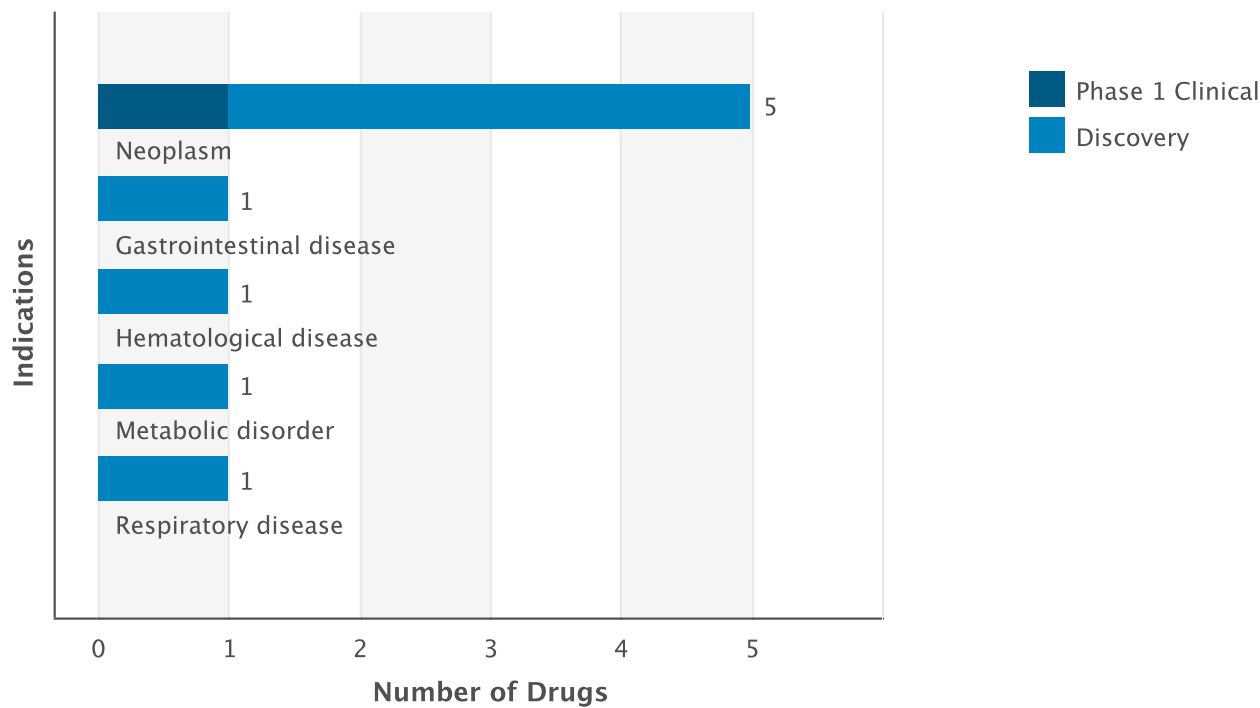
In July 2008, the company raised \$8.4 million in series A financing. By that time, Dicerna had raised \$21.4 million in series A financing.

## PRODUCT PORTFOLIO SUMMARY

### DRUGS

#### Drugs by Indication

Active Drugs by Indication Chart



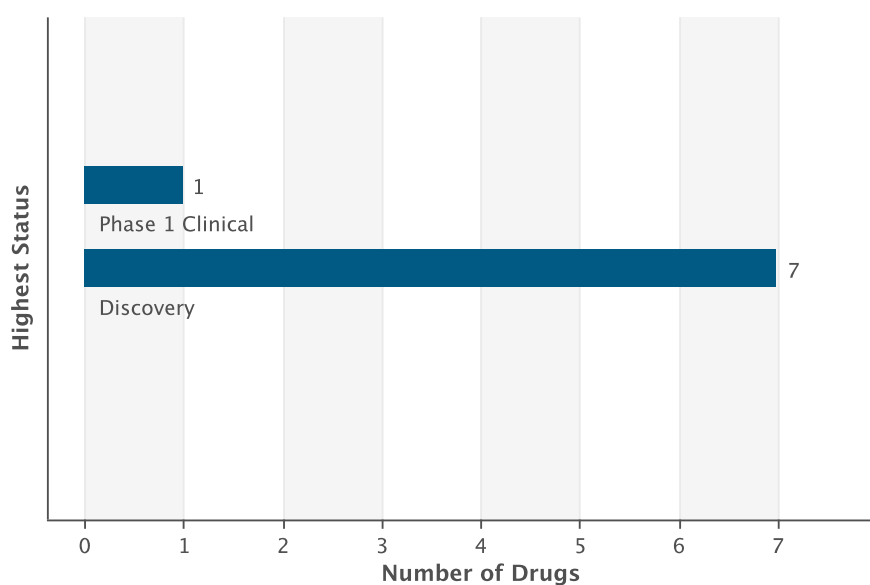
[Return to Table of Contents](#)

## Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	5	7	12
Gastrointestinal disease	1	2	3
Unidentified indication	0	2	2
Inflammatory disease	0	2	2
Andrology	0	1	1
Genitourinary disease	0	1	1
Infectious disease	0	1	1
Metabolic disorder	1	0	1
Hematological disease	1	0	1
Respiratory disease	1	0	1

## Drugs by Highest Status

### Active Drugs by Highest Status Chart



[Return to Table of Contents](#)

## Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 1 Clinical	1
Discovery	7
No Development Reported	11

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Funding	1	0	0	0	1
Drug - Early Research/Development	0	0	1	0	1
Drug - Development/Commercialization License	1	0	0	0	1
Technology - Delivery/Formulation	0	0	2	0	2

## CLINICAL TRIALS

### Trials by Condition Studied

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

[Return to Table of Contents](#)





## PATENTS \*

Indication	As Owner	As Third Party	Total
Cardiovascular disease	2	0	2
Endocrine disease	4	0	4
Gastrointestinal disease	11	1	12
Genitourinary disease	5	0	5
Hematological disease	2	0	2
Andrology	2	0	2
Immune disorder	3	0	3
Musculoskeletal disease	2	0	2
Neoplasm	23	1	24
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1
Respiratory disease	5	0	5
Infectious disease	7	1	8
Inflammatory disease	2	1	3
Otorhinolaryngological disease	1	0	1
Gynecology and obstetrics	3	0	3

\* This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

[Return to Table of Contents](#)

## PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

### DCR-M1711

#### DCR-M1711 SNAPSHOT

<b>Drug Name</b>	DCR-M1711
<b>Key Synonyms</b>	
<b>Originator Company</b>	Dicerna Pharmaceuticals Inc
<b>Active Companies</b>	Dicerna Pharmaceuticals Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 1 Clinical
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Myc proto-oncogene protein inhibitor
<b>Other Actions</b>	siRNA agent;Anticancer
<b>Technologies</b>	Oligonucleotide;Nanoparticle formulation injectable;Intravenous formulation;Infusion;Biological therapeutic
<b>Last Change Date</b>	07-Nov-2014

#### DCR-M1711 DEVELOPMENT PROFILE

##### SUMMARY

Dicerna is developing DCR-M1711 (DCR-MYC), a lead from series of anti-Myc Dicer-substrate siRNAs (DsiRNAs), formulated in EnCore lipid nanoparticles, using Dicerna's Dicer Substrate Technology, for the potential injectable treatment of cancer including hepatocellular carcinoma (HCC), multiple myeloma, lymphoma and other solid tumors,,,. In April 2014, a phase I trial was initiated ; in October 2014, data were expected in mid-to-late 2015 ; in November 2014, a second phase I trial in HCC was expected to be initiated by the end of 2014.

#### DCR-M1711 DEVELOPMENT STATUS

##### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Cancer	US	Phase 1 Clinical	06-Apr-2014

[Return to Table of Contents](#)



## DCR-M1711 DRUG NAMES

Names	Type
DCR-M1711	Research Code
DCR-MYC	Research Code
anti-Myc DsiRNAs (cancer), Dicerna	

## DCR-M1711 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
Solid tumor											
0	0	0	0	0	0	1	1	0	0	1	1
Lymphoma											
0	0	0	0	0	0	1	1	0	0	1	1
Multiple myeloma											
0	0	0	0	0	0	1	1	0	0	1	1

### 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)

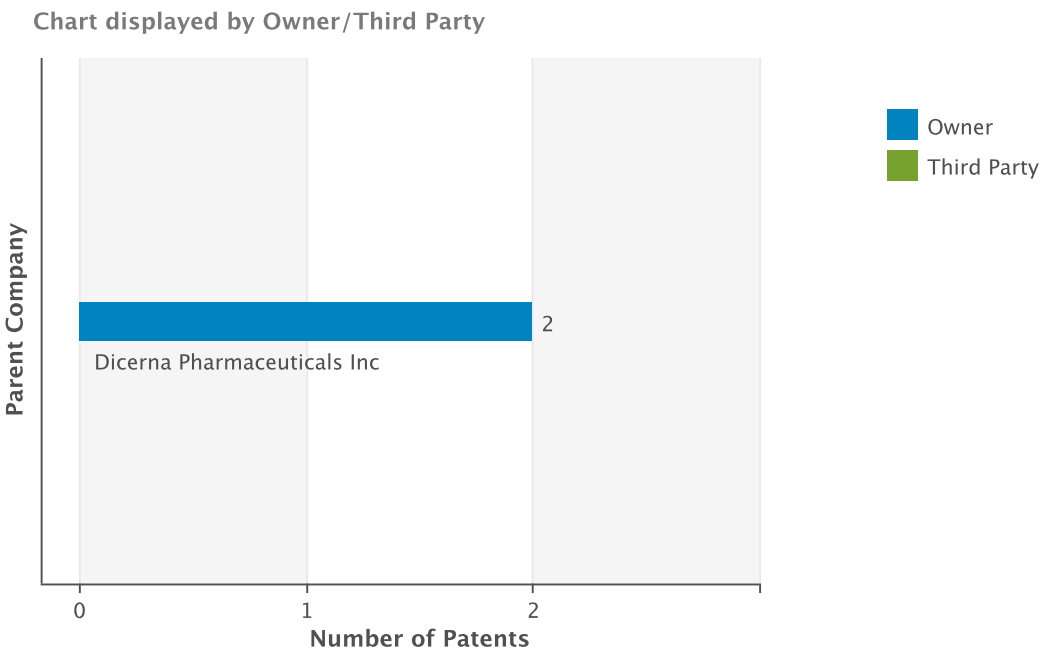
#### Phase 1 Clinical

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

[Return to Table of Contents](#)

PATENTS

Patents by Parent Company Chart

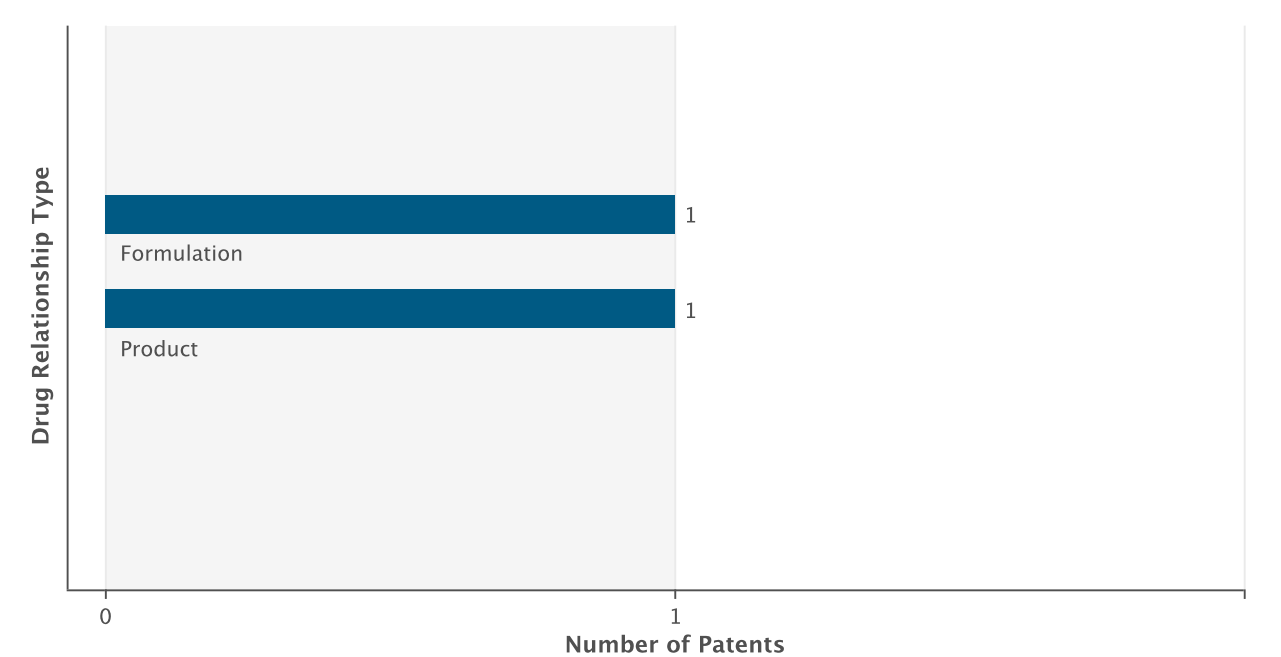


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Dicerna Pharmaceuticals Inc	2	0	2

[Return to Table of Contents](#)

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	1
Formulation	1

[Return to Table of Contents](#)

## DsiRNAs-targeting beta catenin (cancer), Dicerna

### DsiRNAs-targeting beta catenin (cancer), Dicerna SNAPSHOT

Drug Name	DsiRNAs-targeting beta catenin (cancer), Dicerna
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	Beta-catenin modulator
Other Actions	siRNA agent;Anticancer
Technologies	Oligonucleotide;Biological therapeutic
Last Change Date	03-Dec-2014

### DsiRNAs-targeting beta catenin (cancer), Dicerna DEVELOPMENT PROFILE

#### SUMMARY

Dicerna was investigating Dicer-substrate siRNAs (DsiRNAs) that target beta-catenin, for the potential treatment of cancer. In December 2012, preclinical lead development was ongoing. In November 2014, preclinical data were presented .

### DsiRNAs-targeting beta catenin (cancer), Dicerna DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Cancer	US	Discovery	05-Dec-2012

### DsiRNAs-targeting beta catenin (cancer), Dicerna DRUG NAMES

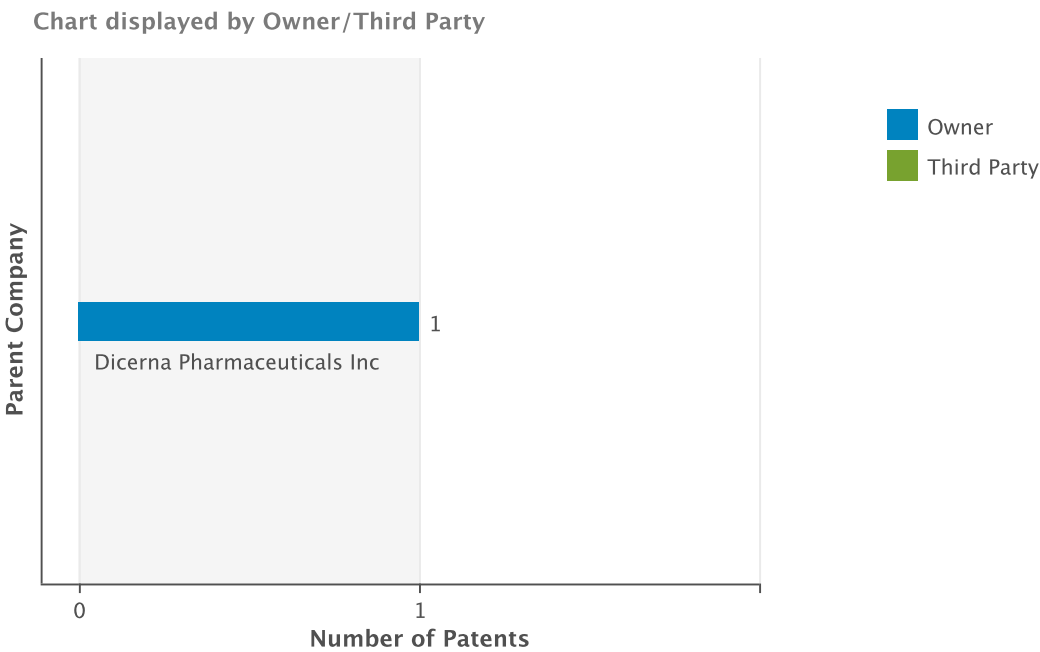
Names	Type
beta-catenin modulator (Dicer-substrate siRNAs, cancer), Dicerna	
DsiRNAs-targeting beta catenin (cancer), Dicerna	

[Return to Table of Contents](#)



PATENTS

Patents by Parent Company Chart

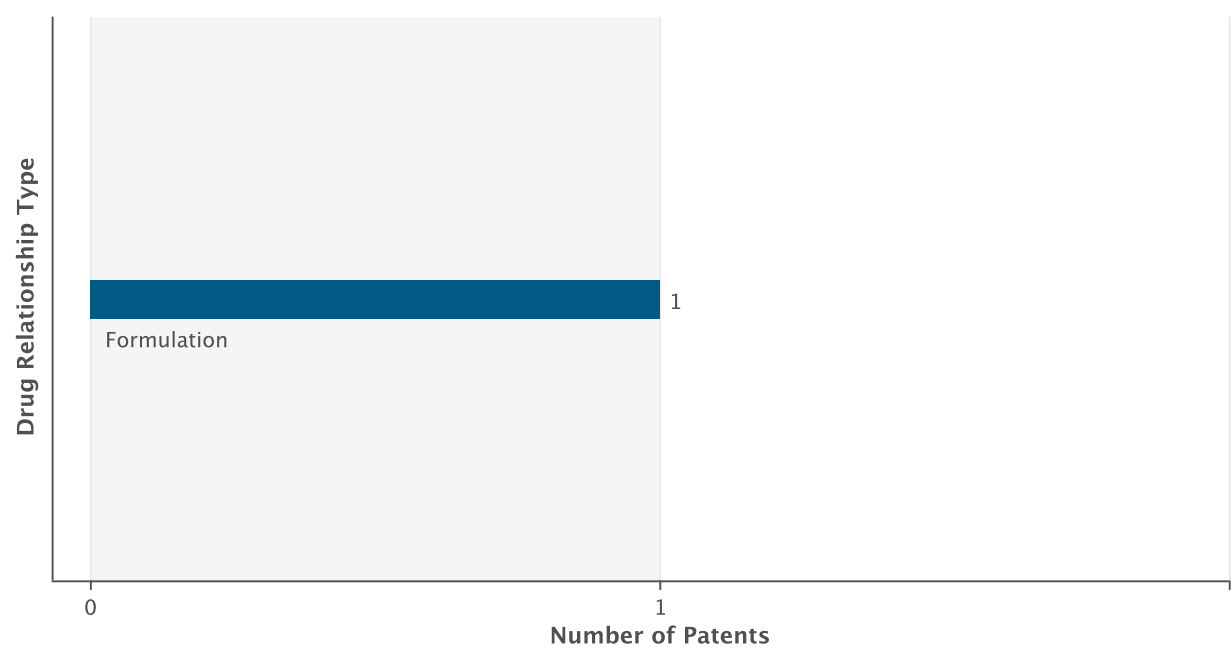


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Dicerna Pharmaceuticals Inc	1	0	1

[Return to Table of Contents](#)

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Formulation	1

[Return to Table of Contents](#)



## KHK-3

### KHK-3 SNAPSHOT

Drug Name	KHK-3
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Kyowa Hakko Kirin Co Ltd;Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Unspecified drug target;Anticancer;siRNA agent
Technologies	Oligonucleotide;Biological therapeutic
Last Change Date	04-Feb-2014

### KHK-3 DEVELOPMENT PROFILE

#### SUMMARY

Dicerna and Kyowa Hakko Kirin are investigating KHK-3, formulated using Dicerna's proprietary Dicer Substrate Technology and Kyowa's proprietary delivery system, for the potential treatment of multiple cancers,. In December 2012, preclinical lead development was ongoing ; in January 2014, developed was presumed to be ongoing.

Kyowa Hakko Kirin and Dicerna are also investigating KHK-1 and KHK-2 for the potential treatment of multiple cancers.

### KHK-3 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Cancer	US	Discovery	05-Dec-2012
Kyowa Hakko Kirin Co Ltd	Cancer	Japan	Discovery	05-Dec-2012

[Return to Table of Contents](#)

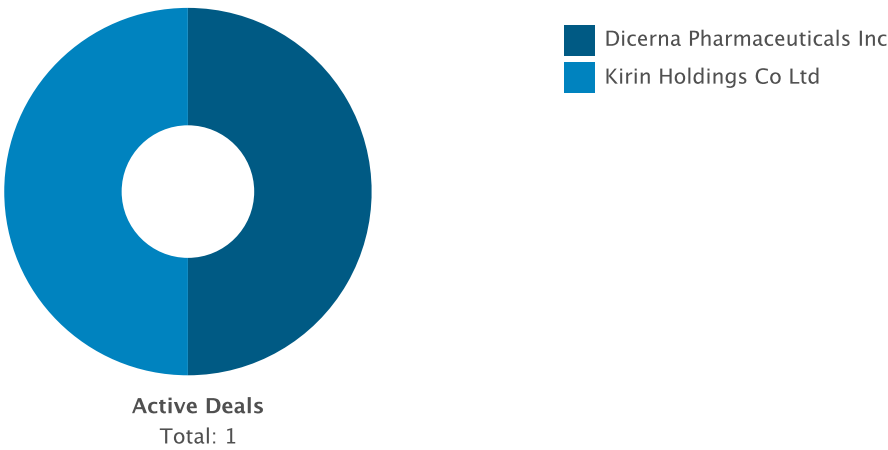
### KHK-3 DRUG NAMES

Names	Type
Dicer-substrate small interfering RNAs (cancer), Dicerna/Kyowa Hakko Kirin	
DsiRNAs (cancer), Dicerna	
Dicer-substrate small interfering RNAs (cancer), Dicerna	
DsiRNAs program (cancer), Dicerna/Kyowa Hakko Kirin	
KHK-3	Research Code

### KHK-3 DEALS AND PATENTS

#### DEALS

#### Deals by Parent Company Chart

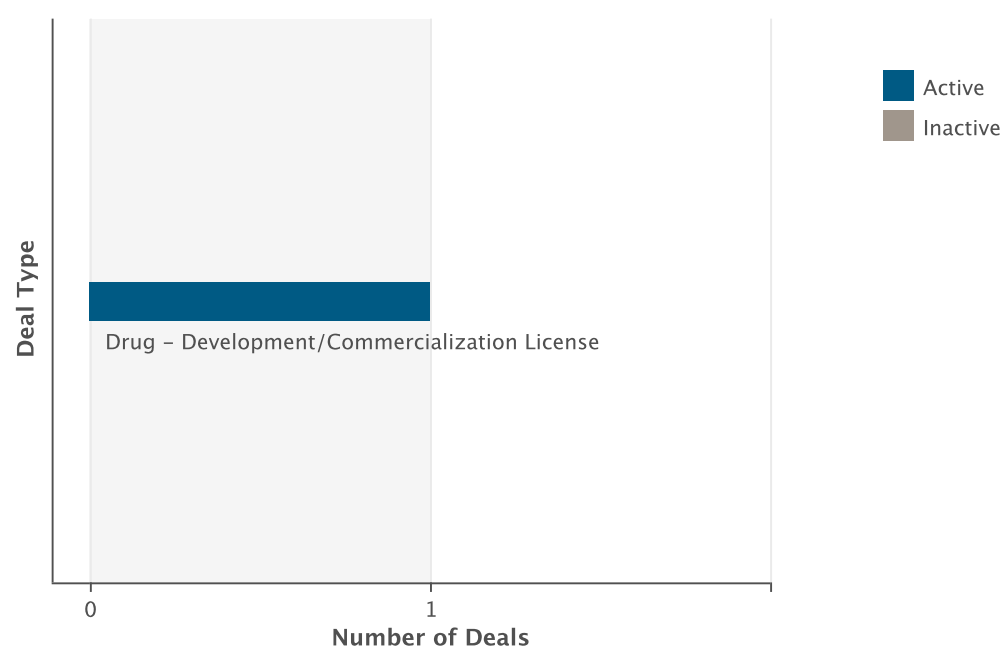


#### Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Dicerna Pharmaceuticals Inc	1	0	0	0	1
Kirin Holdings Co Ltd	0	0	1	0	1

[Return to Table of Contents](#)

Deals by Type Chart



Deals by Type Table

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

[Return to Table of Contents](#)

## KHK-1

### KHK-1 SNAPSHOT

Drug Name	KHK-1
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc;Kyowa Hakko Kirin Co Ltd
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	K-Ras GTPase inhibitor
Other Actions	Oncogene inhibitor;siRNA agent
Technologies	Oligonucleotide;Biological therapeutic
Last Change Date	06-Feb-2014

### KHK-1 DEVELOPMENT PROFILE

#### SUMMARY

Kyowa Hakko Kirin and Dicerna are investigating KHK-1, presumed to be the lead from Dicer-substrate siRNAs (DsiRNAs) program, formulated using Dicerna's proprietary Dicer Substrate Technology and Kyowa's proprietary delivery system, which target K-ras, for the potential treatment of cancers including hepatocellular carcinoma,. In May 2009, preclinical data were presented ; in December 2011, a candidate was selected for advanced development ; in December 2012, IND-enabling studies were underway ; in January 2014, development was presumed to be ongoing.

Kyowa Hakko Kirin and Dicerna are also investigating KHK-2 and KHK-3 for the potential treatment of multiple cancers.

The company is also investigating PEGylated-liposome formulations of DsiRNAs, which target the HPV oncogenes E6 and E7, for the potential treatment of cancer, and DsiRNAs for the potential treatment of HIV and hepatitis C virus infection, and inflammation.

### KHK-1 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Cancer	US	Discovery	13-May-2009
Kyowa Hakko Kirin Co Ltd	Cancer	Japan	Discovery	04-Jan-2010

[Return to Table of Contents](#)

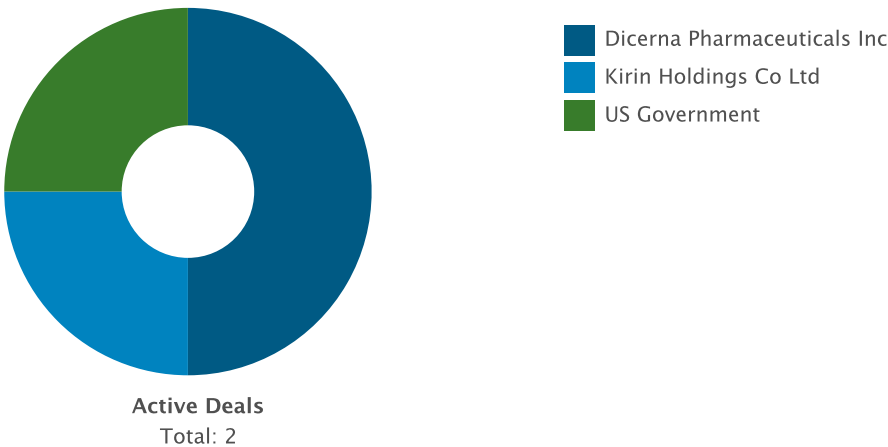
KHK-1 DRUG NAMES

Names	Type
DsiRNAs program (cancer), Dicerna/Kyowa Hakko Kirin	
Dicer-substrate small interfering RNAs (cancer), Dicerna	
Dicer-substrate small interfering RNAs (cancer), Dicerna/Kyowa Hakko Kirin	
KHK-1	Research Code
DsiRNAs (cancer), Dicerna	

KHK-1 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

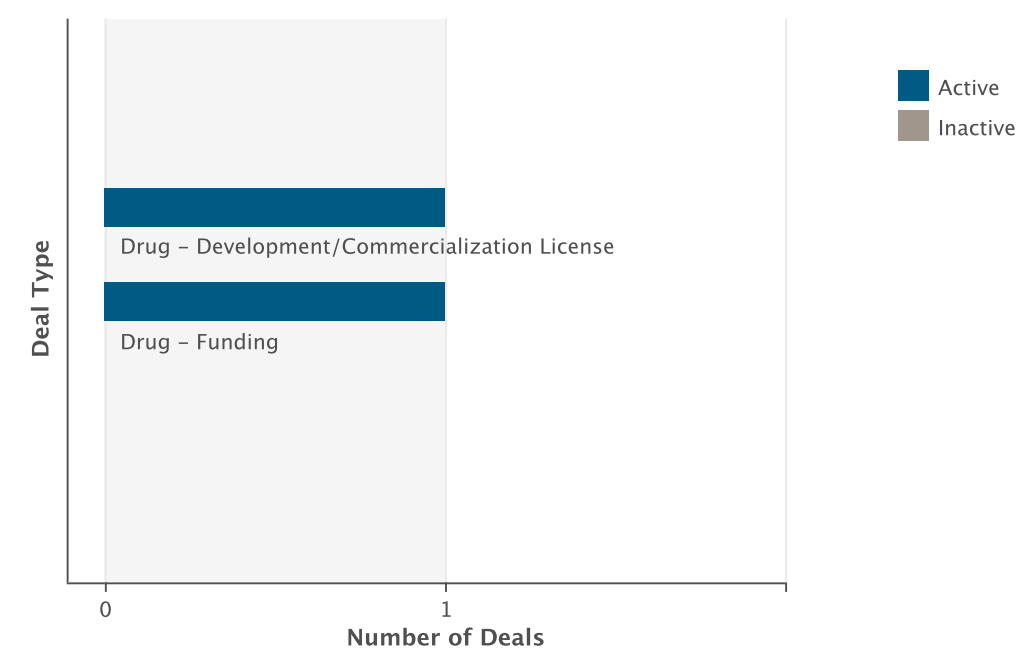


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Dicerna Pharmaceuticals Inc	2	0	0	0	2
Kirin Holdings Co Ltd	0	0	1	0	1
US Government	0	0	1	0	1

[Return to Table of Contents](#)

Deals by Type Chart



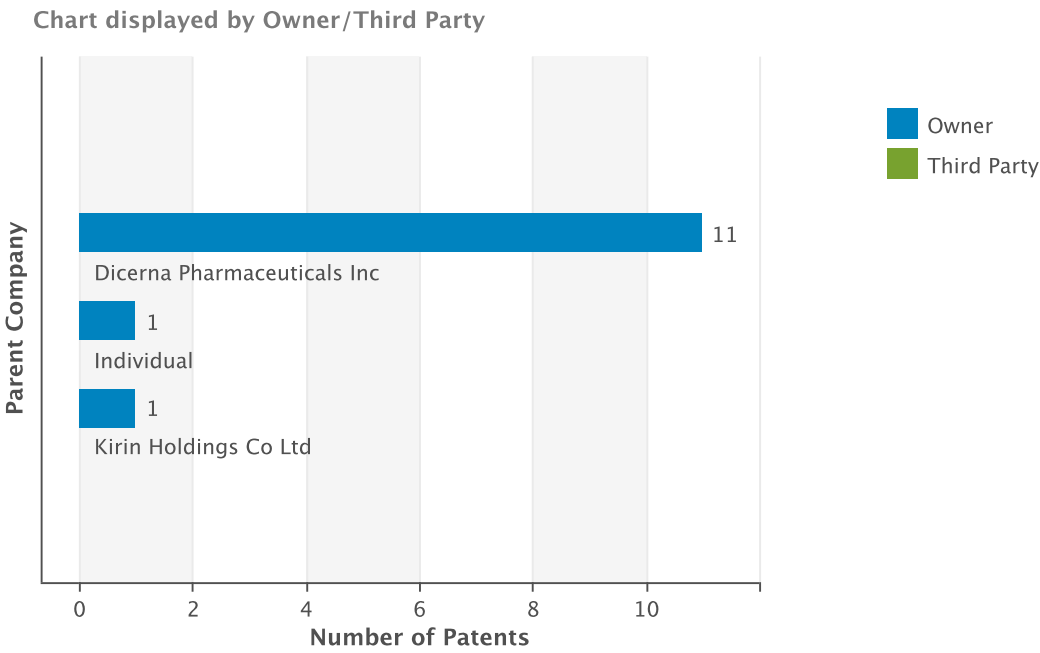
Deals by Type Table

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

[Return to Table of Contents](#)

PATENTS

Patents by Parent Company Chart

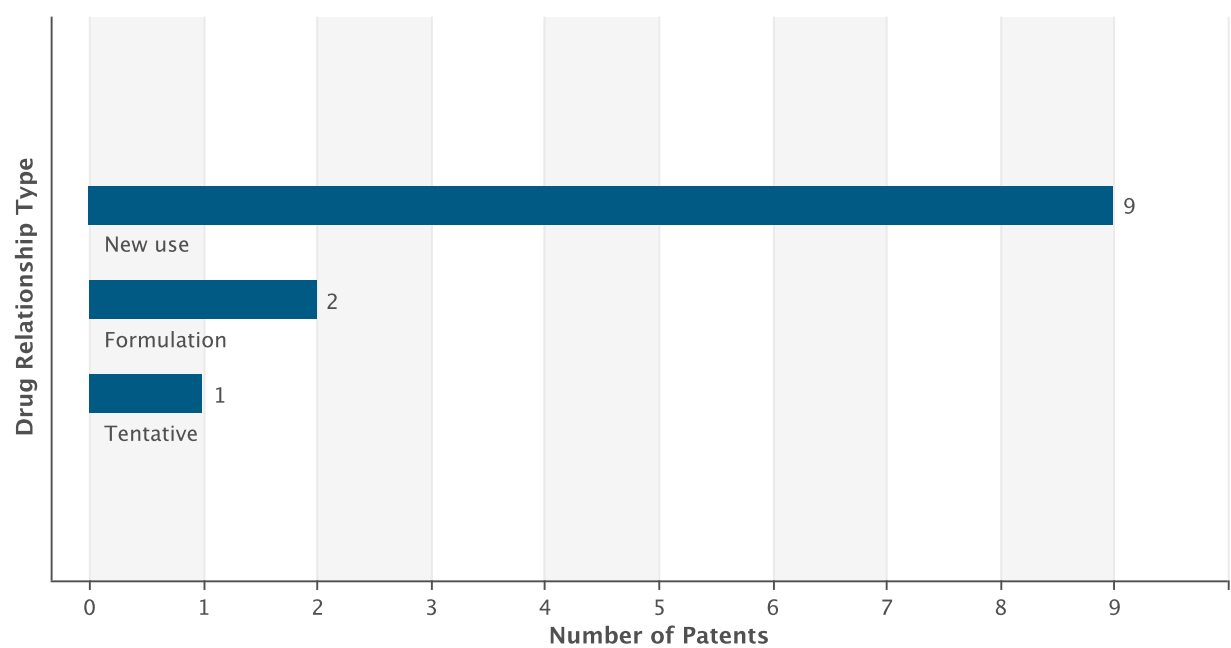


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Dicerna Pharmaceuticals Inc	11	0	11
Kirin Holdings Co Ltd	1	0	1
Individual	1	0	1

[Return to Table of Contents](#)

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	9
Formulation	2
Tentative	1



## KHK-2

### KHK-2 SNAPSHOT

Drug Name	KHK-2
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Kyowa Hakko Kirin Co Ltd;Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Unspecified drug target;siRNA agent;Anticancer
Technologies	Oligonucleotide;Biological therapeutic
Last Change Date	06-Feb-2014

### KHK-2 DEVELOPMENT PROFILE

#### SUMMARY

Dicerna and Kyowa Hakko Kirin are investigating KHK-2, formulated using Dicerna's proprietary Dicer Substrate Technology and Kyowa's proprietary delivery system, for the potential treatment of multiple cancers,. In December 2012, preclinical lead development was underway ; in January 2014, development was presumed to be ongoing.

Kyowa Hakko Kirin and Dicerna are also investigating KHK-1 and KHK-3 for the potential treatment of multiple cancers.

### KHK-2 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Cancer	US	Discovery	08-Aug-2012
Kyowa Hakko Kirin Co Ltd	Cancer	Japan	Discovery	08-Aug-2012

[Return to Table of Contents](#)



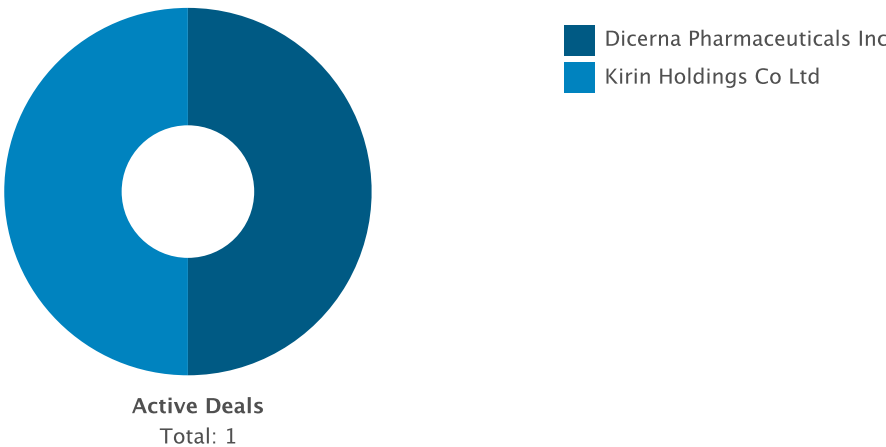
KHK-2 DRUG NAMES

Names	Type
DsiRNAs (cancer), Dicerna	
Dicer-substrate small interfering RNAs (cancer), Dicerna/Kyowa Hakko Kirin	
DsiRNAs program (cancer), Dicerna/Kyowa Hakko Kirin	
KHK-2	Research Code
Dicer-substrate small interfering RNAs (cancer), Dicerna	

KHK-2 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

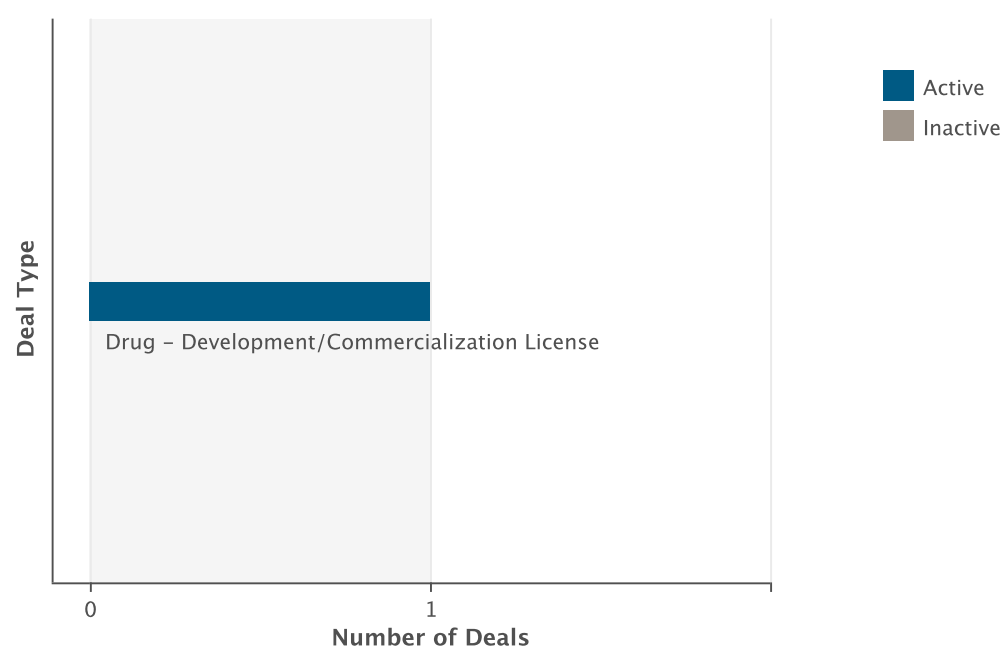


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Kirin Holdings Co Ltd	0	0	1	0	1
Dicerna Pharmaceuticals Inc	1	0	0	0	1

[Return to Table of Contents](#)

Deals by Type Chart



Deals by Type Table

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

[Return to Table of Contents](#)

## DCR-PH1

### DCR-PH1 SNAPSHOT

Drug Name	DCR-PH1
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Hyperoxaluria
Target-based Actions	Oxidase inhibitor
Other Actions	siRNA agent
Technologies	Oligonucleotide;Nanoparticle formulation;Lipid;Biological therapeutic
Last Change Date	18-Nov-2014

### DCR-PH1 DEVELOPMENT PROFILE

#### SUMMARY

### DCR-PH1 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Hyperoxaluria	US	Discovery	06-Jan-2014

[Return to Table of Contents](#)

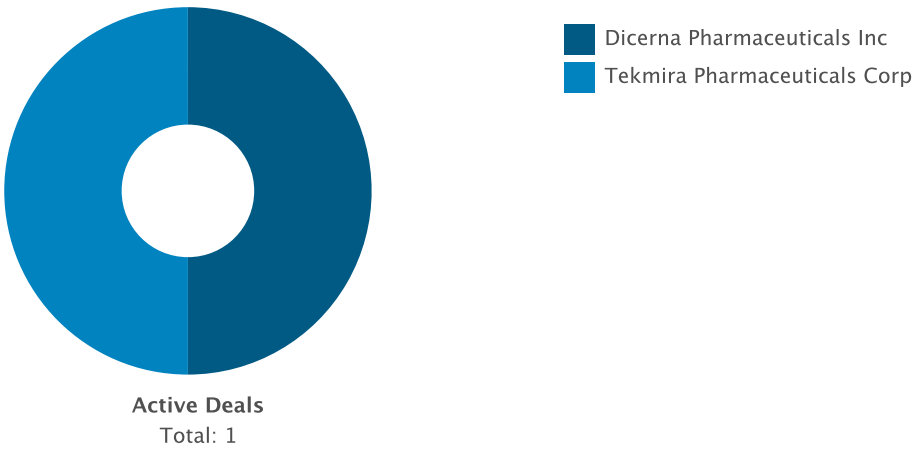
DCR-PH1 DRUG NAMES

Names	Type
Dicer Substrate RNA (EnCore lipid nanoparticle, hyperoxaluria), Dicerna Pharmaceuticals	
DCR-PH1	Research Code
DsiRNA (EnCore lipid nanoparticle, hyperoxaluria), Dicerna Pharmaceuticals	

DCR-PH1 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Dicerna Pharmaceuticals Inc	0	0	1	0	1
Tekmira Pharmaceuticals Corp	1	0	0	0	1

[Return to Table of Contents](#)

Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Technology - Delivery/Formulation	1	0	1

[Return to Table of Contents](#)

## DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals

### DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals SNAPSHOT

Drug Name	DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Blood clotting disorder
Target-based Actions	Factor IIa antagonist
Other Actions	siRNA agent
Technologies	Oligonucleotide;Biological therapeutic;Parenteral formulation unspecified
Last Change Date	07-Jan-2014

### DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals DEVELOPMENT PROFILE

#### SUMMARY

Dicerna Pharmaceuticals is investigating Dicer-substrate siRNAs (DsiRNAs) that target thrombin, formulated using Dicerna's proprietary Dicer Substrate Technology, for the potential treatment of clotting disorder. In January 2014, the program was listed as being in research.

### DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Blood clotting disorder	US	Discovery	06-Jan-2014

### DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals DRUG NAMES

Names	Type
DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals	

[Return to Table of Contents](#)



## DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals

### DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals SNAPSHOT

Drug Name	DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Alpha-1 antitrypsin deficiency
Target-based Actions	Alpha 1 antitrypsin modulator
Other Actions	siRNA agent
Technologies	Oligonucleotide;Biological therapeutic;Parenteral formulation unspecified
Last Change Date	07-Jan-2014

### DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals DEVELOPMENT PROFILE

#### SUMMARY

Dicerna Pharmaceuticals is investigating Dicer-substrate siRNAs (DsiRNAs) targeting alpha 1 antitrypsin (AAT), developed using Dicerna's Dicer Substrate Technology, for the potential treatment of AAT deficiency. In January 2014, the program was listed as being in research.

### DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Alpha-1 antitrypsin deficiency	US	Discovery	06-Jan-2014

### DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals DRUG NAMES

Names	Type
DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals	

[Return to Table of Contents](#)



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](http://cortellis.thomsonreuters.com/cortellis_for_you/?cid=thomsonone).

For subscription information, e-mail [scientific.lifesciences@thomsonreuters.com](mailto: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.

[Return to Table of Contents](#)

