

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

## COMPANY OVERVIEW

Company Name	Dicerna Pharmaceuticals Inc
Parent Company Name	Dicerna Pharmaceuticals Inc
Website	<a href="http://www.dicerna.com/">http://www.dicerna.com/</a>
Country	US
Number of Drugs in Active Development	7
Number of Inactive Drugs	12
Number of Patents as Owner	19
Number of Patents as Third Party	1
Number of Deals	4
Key Indications	Cancer,Hepatocellular carcinoma,Alpha-1 antitrypsin deficiency,Blood clotting disorder,Hyperoxaluria,Solid tumor,Lung tumor,Breast tumor,Ovary tumor,Pancreas tumor,Prostate tumor,Renal tumor,Uterine cervix tumor,Viral infection
Key Target-based Actions	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
Key Technologies	Oligonucleotide,Biological therapeutic,Parenteral formulation unspecified,Small molecule therapeutic,PEGylated formulation,Liposome formulation,Nanoparticle formulation,Gene expression regulation,Lipid,Oligonucleotide antisense,Oligonucleotide

## 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 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.

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

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.

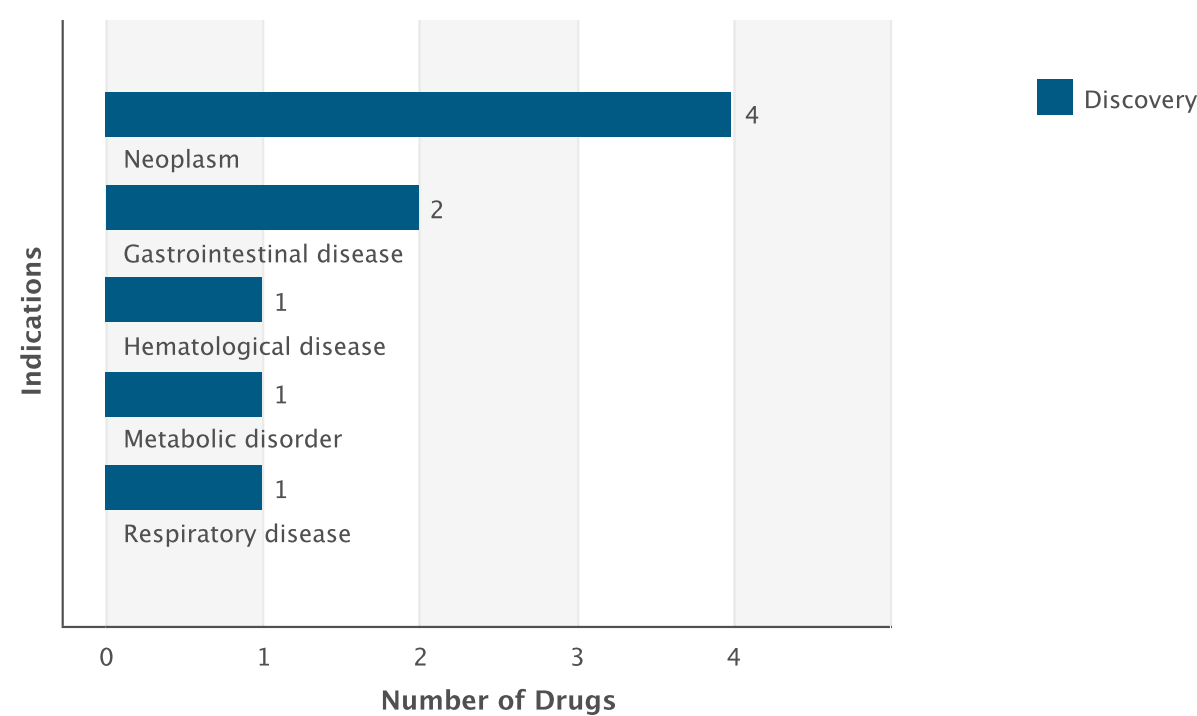
In July 2008, the company raised \$8.4 million in series A financing. By that time, Dicerna had raised \$21.4 million in series A financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



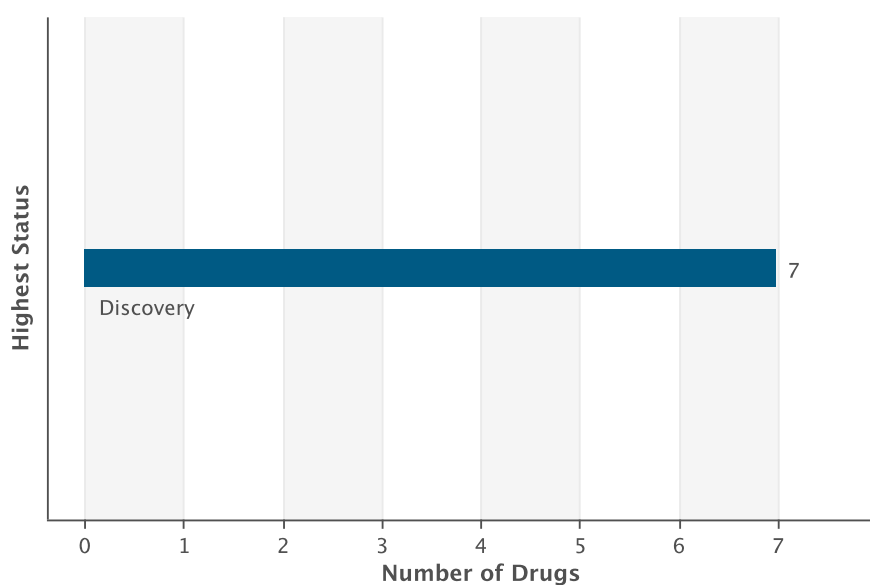
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## Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	4	8	12
Gastrointestinal disease	2	1	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



## Drugs by Highest Status Table

Development Status	Number of Drugs
Discovery	7
No Development Reported	12

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## CLINICAL TRIALS

### 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	2	0	2
Endocrine disease	4	0	4
Gastrointestinal disease	9	1	10
Genitourinary disease	5	0	5
Hematological disease	2	0	2
Andrology	3	0	3
Immune disorder	3	0	3
Musculoskeletal disease	2	0	2
Neoplasm	19	1	20
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1
Respiratory disease	5	0	5
Infectious disease	6	1	7
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.

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## PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

### 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	Biological therapeutic;Oligonucleotide
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

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Company	Indication	Country	Development Status	Date
Kyowa Hakko Kirin Co Ltd	Cancer	Japan	Discovery	04-Jan-2010

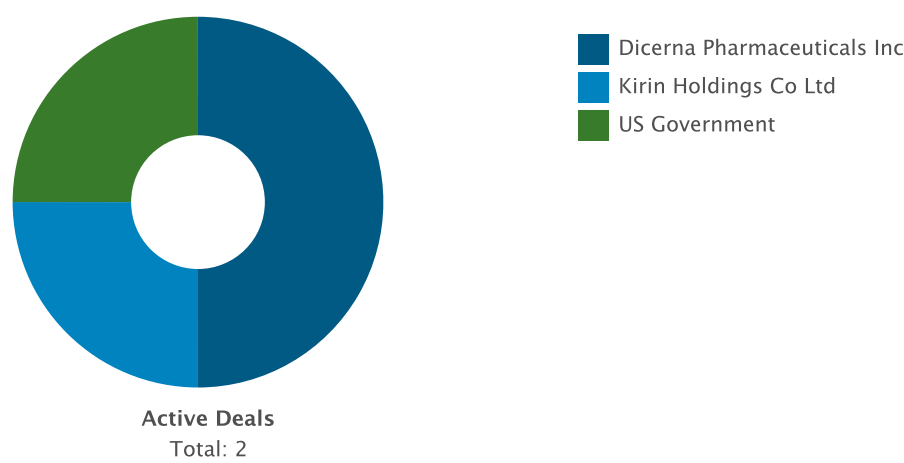
### KHK-1 DRUG NAMES

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

### KHK-1 DEALS AND PATENTS

#### DEALS

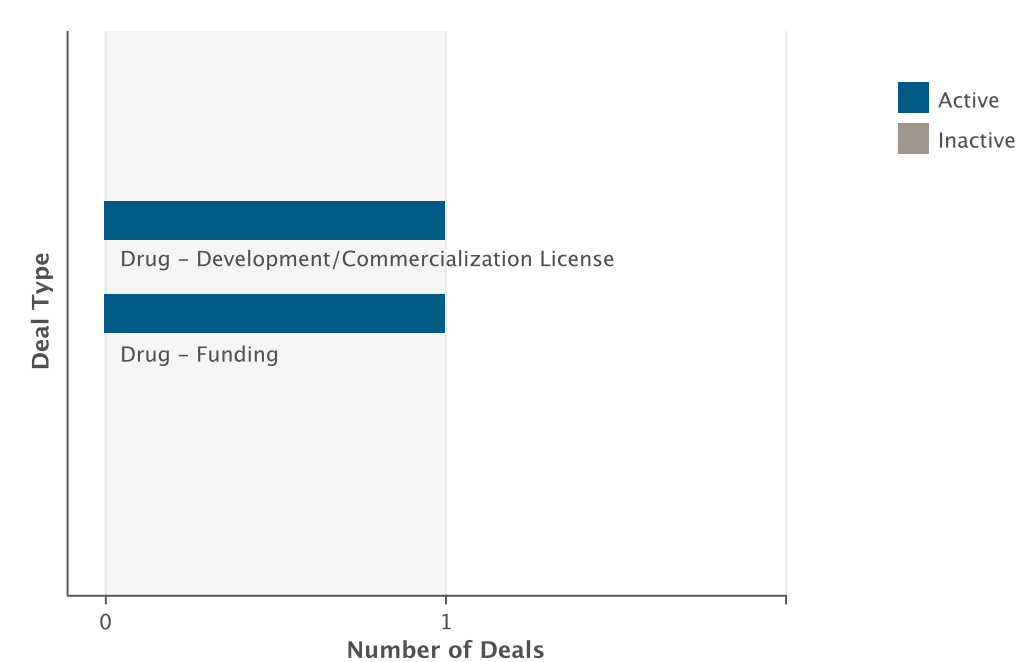
Deals by Parent Company Chart



Deals by Parent Company Table

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

Deals by Type Chart



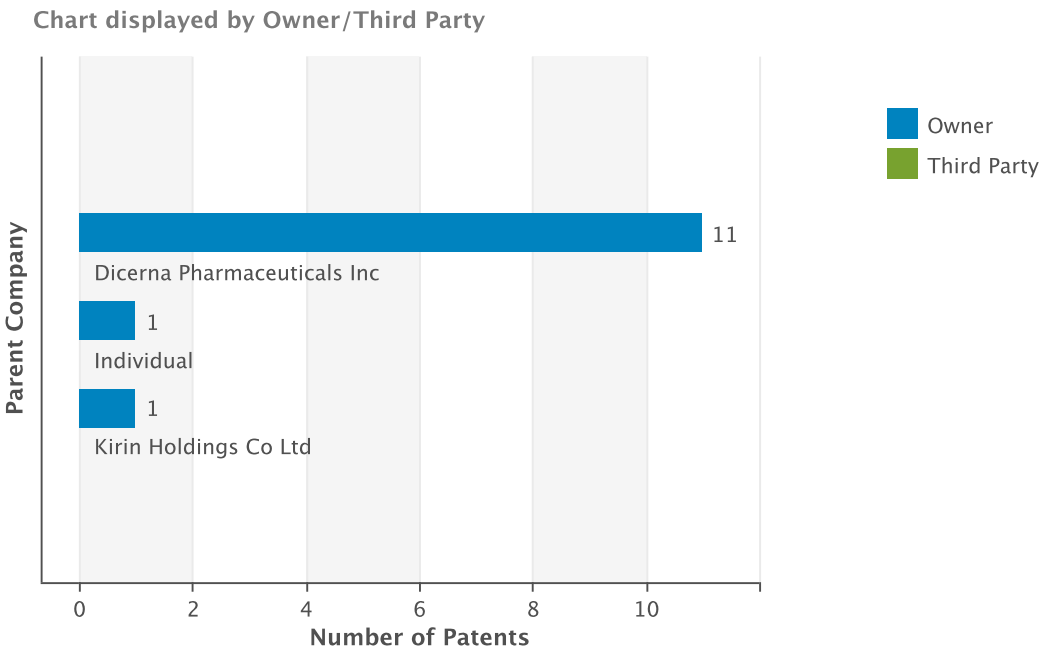
Deals by Type Table

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

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PATENTS

Patents by Parent Company Chart

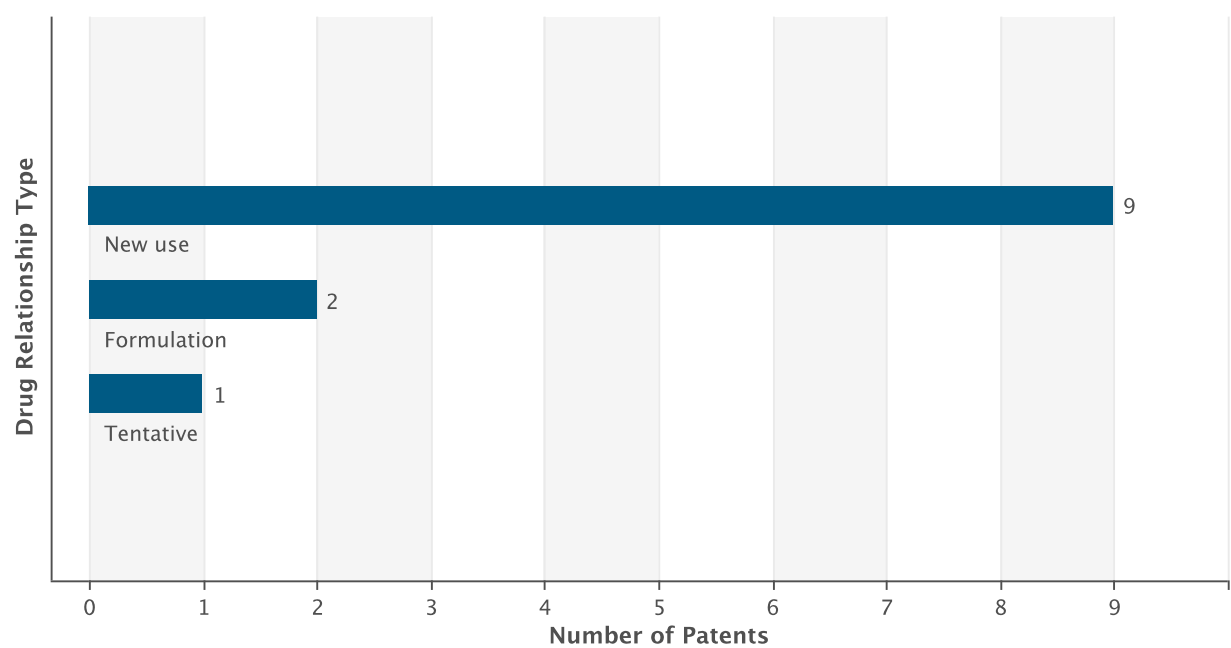


Patents by Parent Company Table

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

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	9
Formulation	2
Tentative	1

## DCR-M1711

### DCR-M1711 SNAPSHOT

Drug Name	DCR-M1711
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Hepatocellular carcinoma;Solid tumor
Target-based Actions	Myc proto-oncogene protein inhibitor
Other Actions	siRNA agent;Anticancer
Technologies	Nanoparticle formulation;Biological therapeutic;Parenteral formulation unspecified;Oligonucleotide
Last Change Date	06-Jan-2014

### DCR-M1711 DEVELOPMENT PROFILE

#### SUMMARY

Dicerna is investigating DCR-M1711, 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 treatment of hepatocellular carcinoma and other solid tumors,. In June 2012, IND-enabling studies were underway ; in August 2013, development was ongoing. In August 2013, the company expected to initiate clinical trials in early 2014 and to establish human proof-of-concept in 2015.

### DCR-M1711 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Dicerna Pharmaceuticals Inc	Hepatocellular carcinoma	US	Discovery	21-Jun-2012
Dicerna Pharmaceuticals Inc	Solid tumor	US	Discovery	06-Jan-2014

### DCR-M1711 DRUG NAMES

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

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## 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	
Technologies	Small molecule therapeutic
Last Change Date	07-Jan-2014

### DCR-PH1 DEVELOPMENT PROFILE

#### SUMMARY

Dicerna Pharmaceuticals is investigating DCR-PH1, a glycolate oxidase inhibitor for the potential treatment of primary hyperoxaluria 1. In January 2014, the drug was listed as being in preclinical studies.

### DCR-PH1 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

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

### DCR-PH1 DRUG NAMES

Names	Type
DCR-PH1	Research Code

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## 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	Biological therapeutic;Oligonucleotide
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

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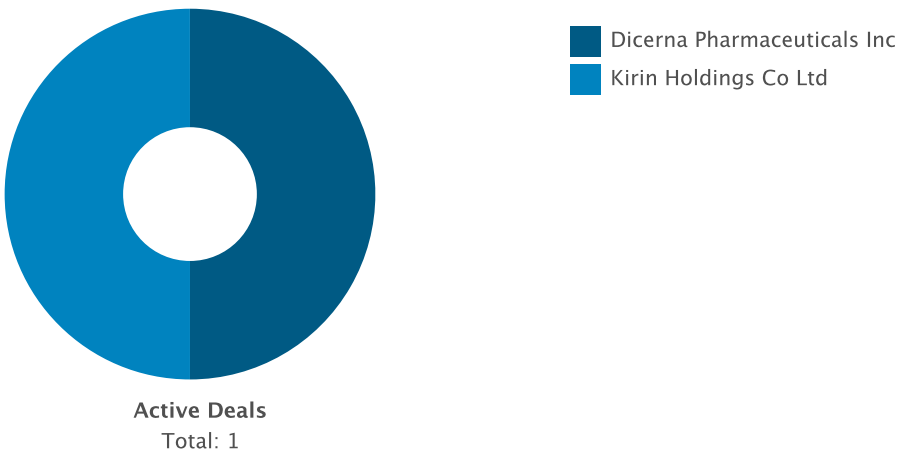
### KHK-3 DRUG NAMES

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

### KHK-3 DEALS AND PATENTS

#### DEALS

#### Deals by Parent Company Chart

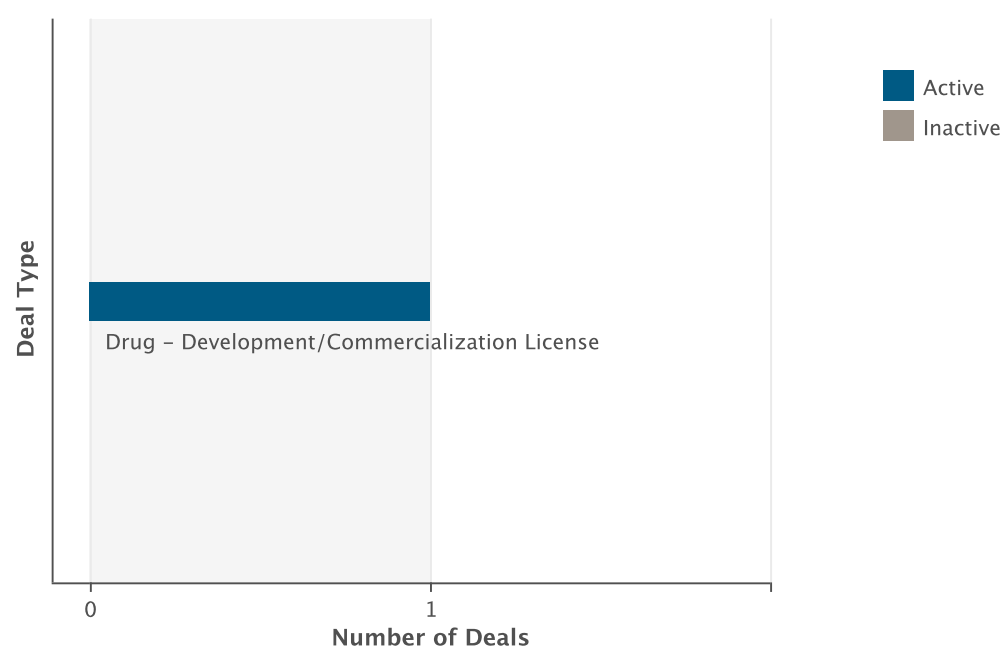


#### Deals by Parent Company Table

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

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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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## 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	Biological therapeutic;Oligonucleotide
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

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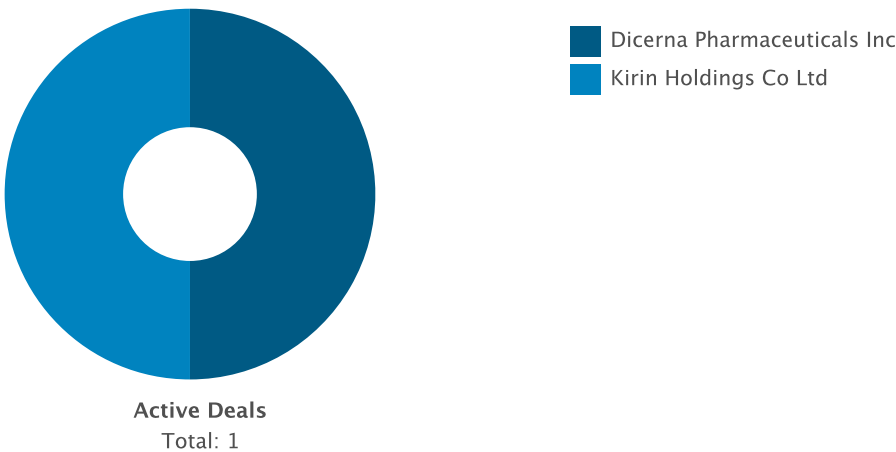
KHK-2 DRUG NAMES

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

KHK-2 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

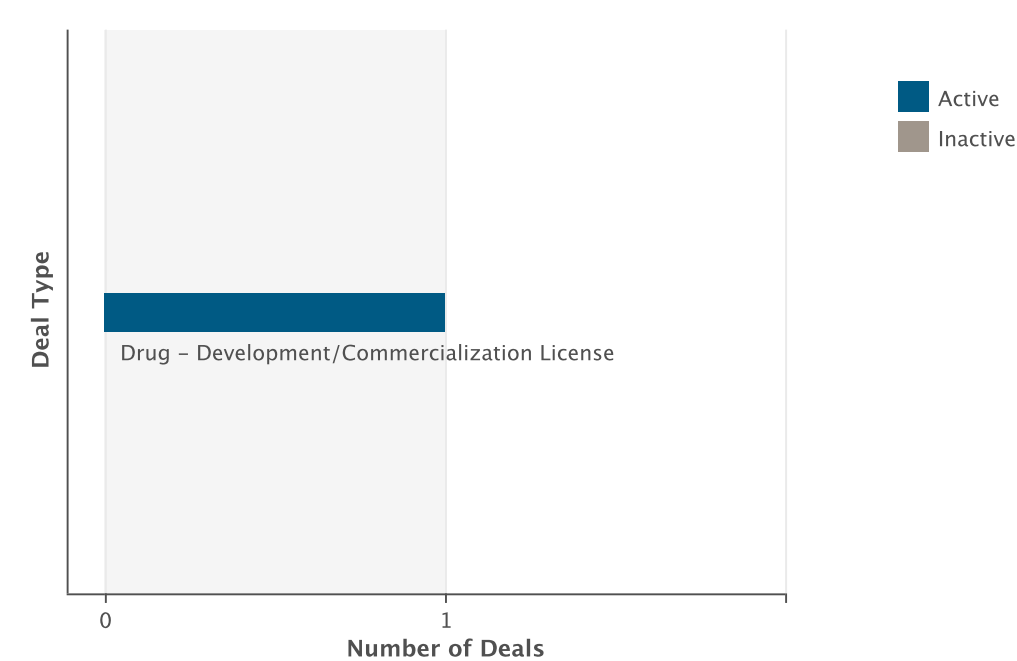


Deals by Parent Company Table

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

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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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## 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	

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## 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	

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