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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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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.

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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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	http://www.dicerna.com/
Country	US
Number of Drugs in Active Development	7
Number of Inactive Drugs	12
Number of Patents as Owner	21
Number of Patents as Third Party	1
Number of Deals	4
Key Indications	Cancer,Alpha-1 antitrypsin deficiency,Blood clotting disorder,Hyperoxaluria,Lung tumor,Breast tumor,Ovary tumor,Pancreas tumor,Renal tumor,Uterine cervix tumor
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,Lipid,Intravenous formulation,PEGylated formulation,Infusion,Liposome formulation,Nanoparticle formulation,Nanoparticle formulation injectable

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 apatmer 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 in 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.

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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.

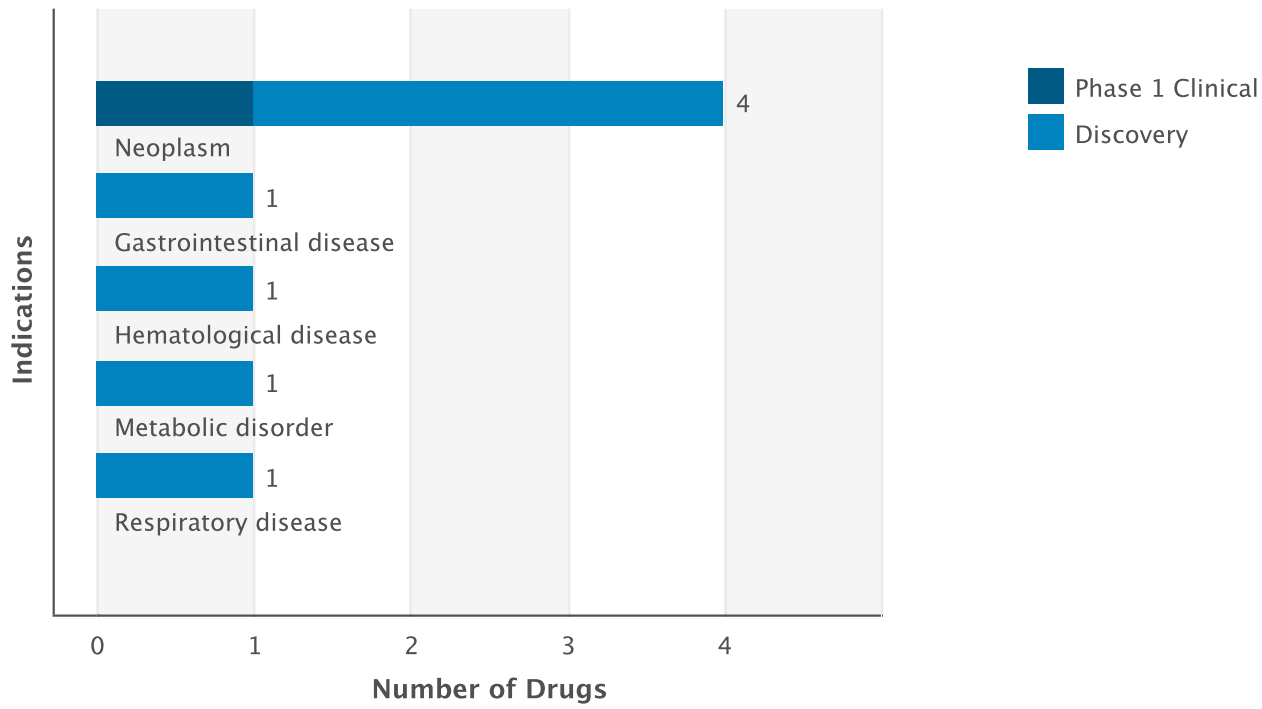
In July 2008, the company raised $8.4 million in series A financing. By that time, Dicerna had raised $21.4 million in series A financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



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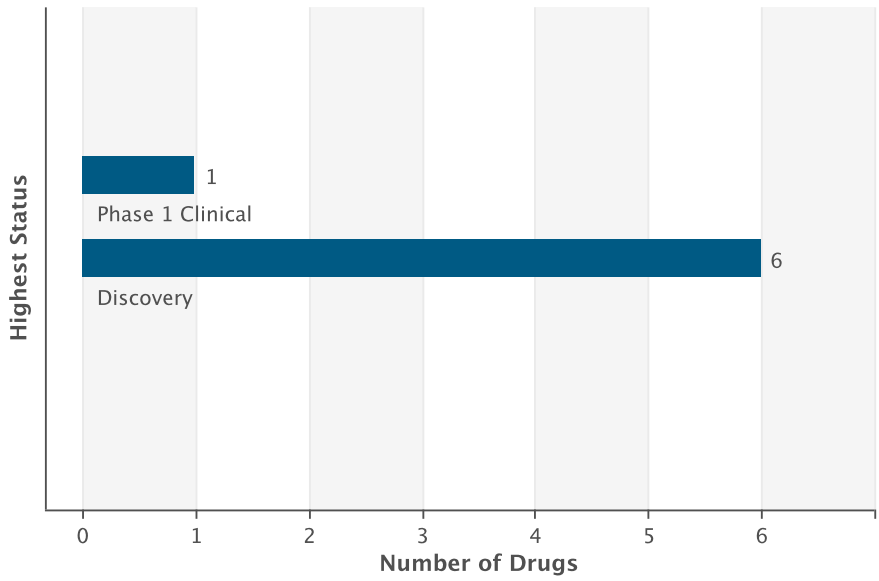
Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	4	8	12
Gastrointestinal disease	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



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Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 1 Clinical	1
Discovery	6
No Development Reported	12

DEALS

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

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

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PATENTS *

Indication	As Owner	As Third Party	Total
Cardiovascular disease	2	0	2
Endocrine disease	5	0	5
Gastrointestinal disease	11	1	12
Genitourinary disease	6	0	6
Hematological disease	2	0	2
Andrology	3	0	3
Immune disorder	3	0	3
Musculoskeletal disease	2	0	2
Neoplasm	21	1	22
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1
Respiratory disease	6	0	6
Infectious disease	6	1	7
Inflammatory disease	2	1	3
Otorhinolaryngological disease	1	0	1
Gynecology and obstetrics	4	0	4

* 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

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	Phase 1 Clinical
Active Indications	Cancer
Target-based Actions	Myc proto-oncogene protein inhibitor
Other Actions	Anticancer;siRNA agent
Technologies	Oligonucleotide;Nanoparticle formulation injectable;Intravenous formulation;Infusion;Biological therapeutic
Last Change Date	18-Apr-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, multiple myeloma, lymphoma and other solid tumors,,. in August 2013, development was ongoing. In April 2014, a phase I trial was initiated.

DCR-M1711 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

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

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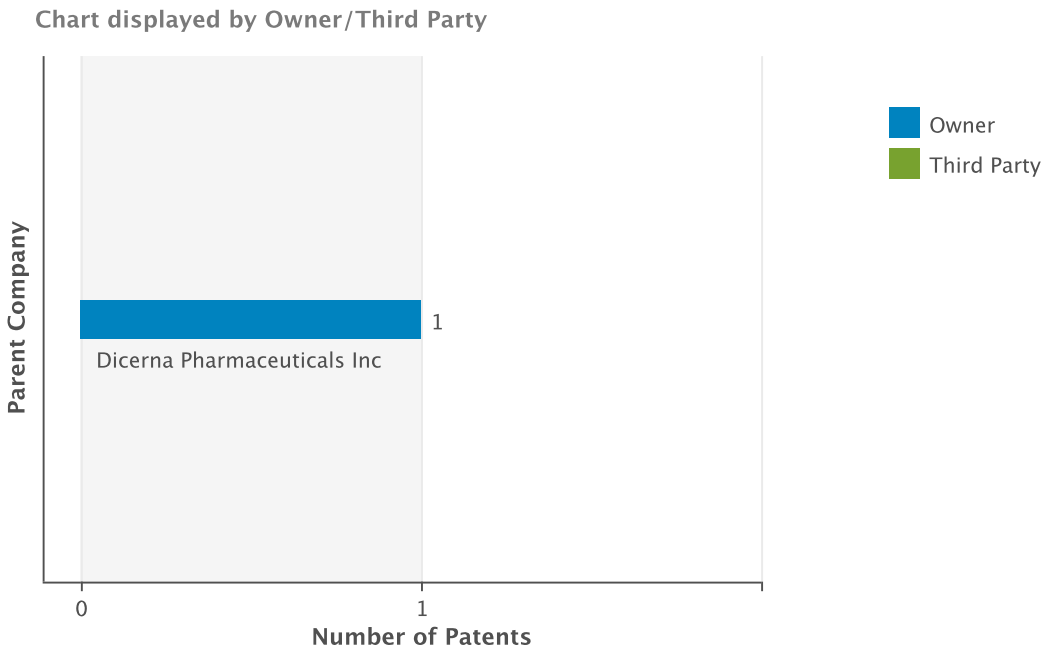
DCR-M1711 DRUG NAMES

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

DCR-M1711 DEALS AND PATENTS

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

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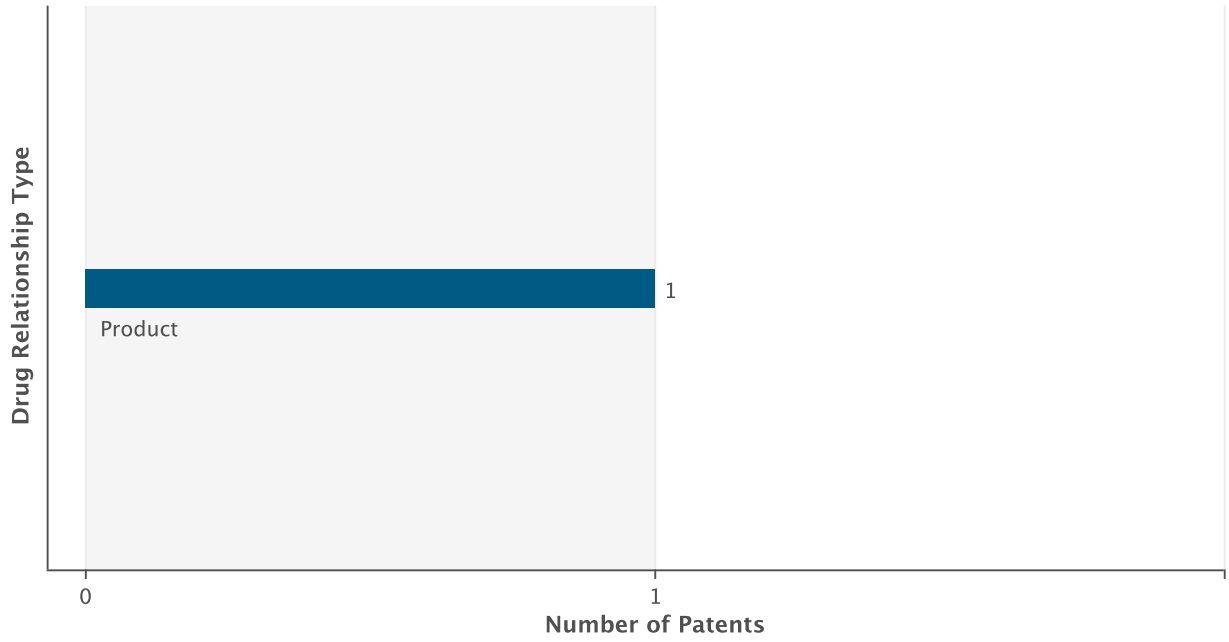
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	1

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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	Nanoparticle formulation;Lipid;Small molecule therapeutic
Last Change Date	01-Jul-2014

DCR-PH1 DEVELOPMENT PROFILE

SUMMARY

Dicerna Pharmaceuticals is investigating DCR-PH1, a Dicer Substrate RNA (DsiRNA) molecule that inhibits glycolate oxidase delivered using EnCore lipid nanoparticle technology, for the potential treatment of primary hyperoxaluria 1,. In January 2014, the drug was listed as being in preclinical studies. In June 2014, preclinical data were reported. In March 2014, the company expected to complete optimization and disclose a clinical candidate for this program in the first half of 2014, with clinical trials anticipated for 2015.

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

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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	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

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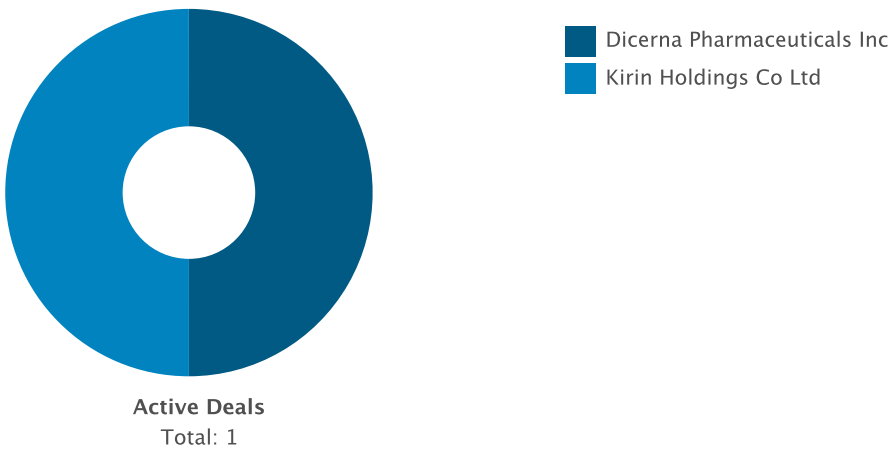
KHK-3 DRUG NAMES

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

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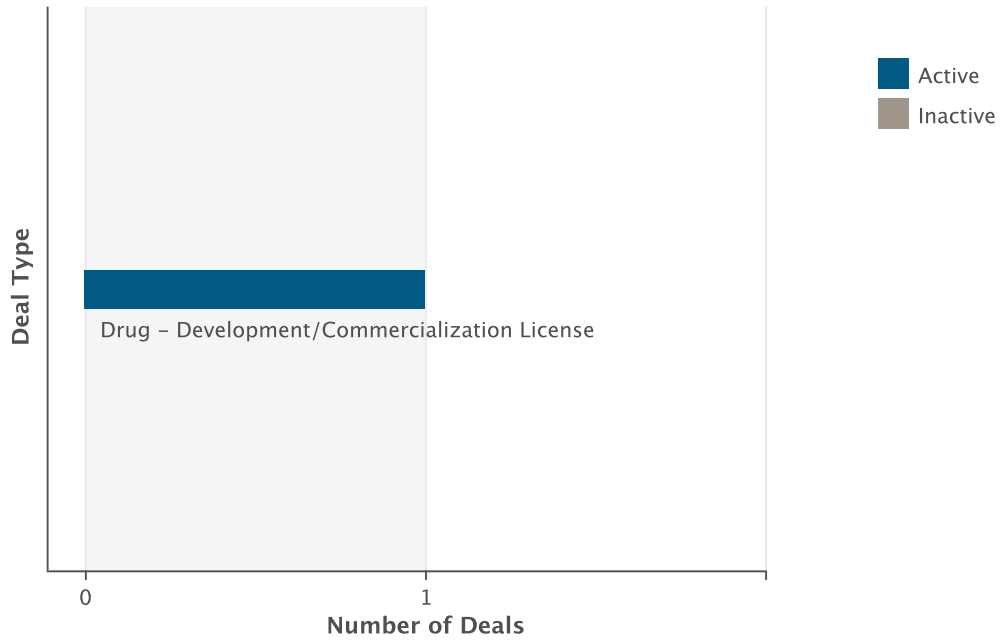
Deals by Type Chart



Deals by Type Table

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

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KHK-1

KHK-1 SNAPSHOT

Drug Name	KHK-1
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	K-Ras GTPase inhibitor
Other Actions	siRNA agent;Oncogene inhibitor
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

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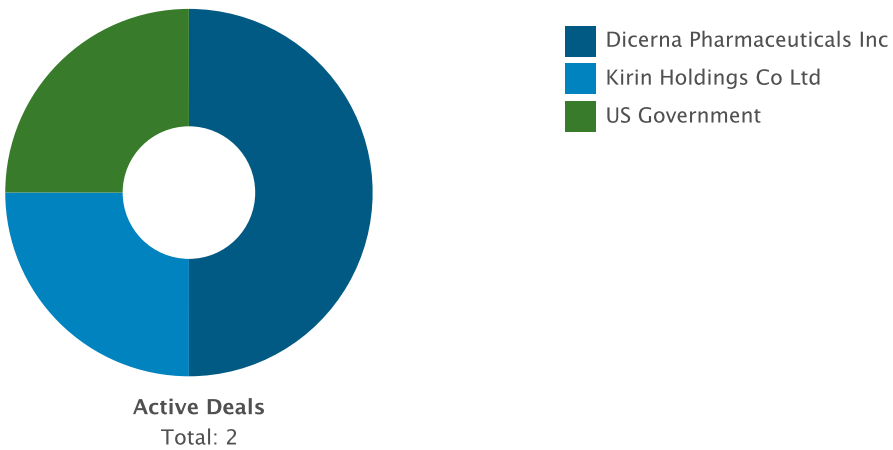
KHK-1 DRUG NAMES

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

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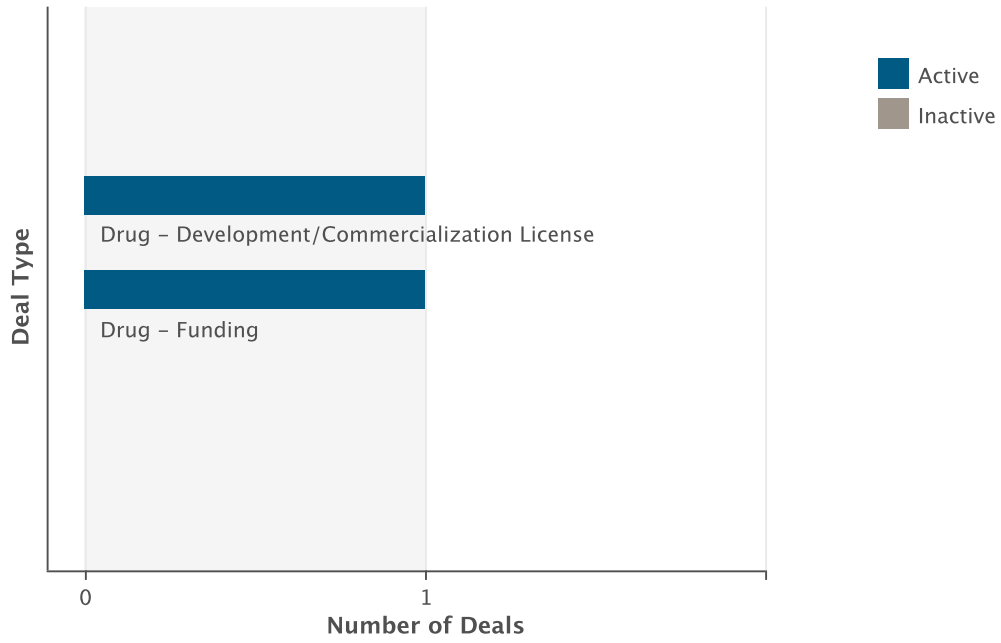
Deals by Type Chart



Deals by Type Table

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

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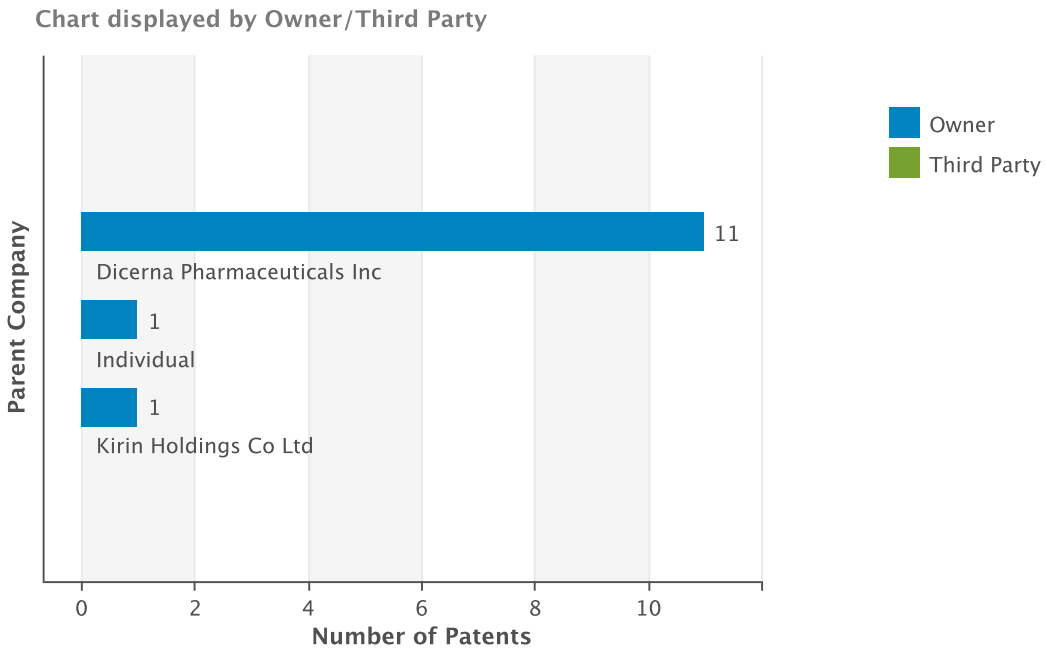
PATENTS

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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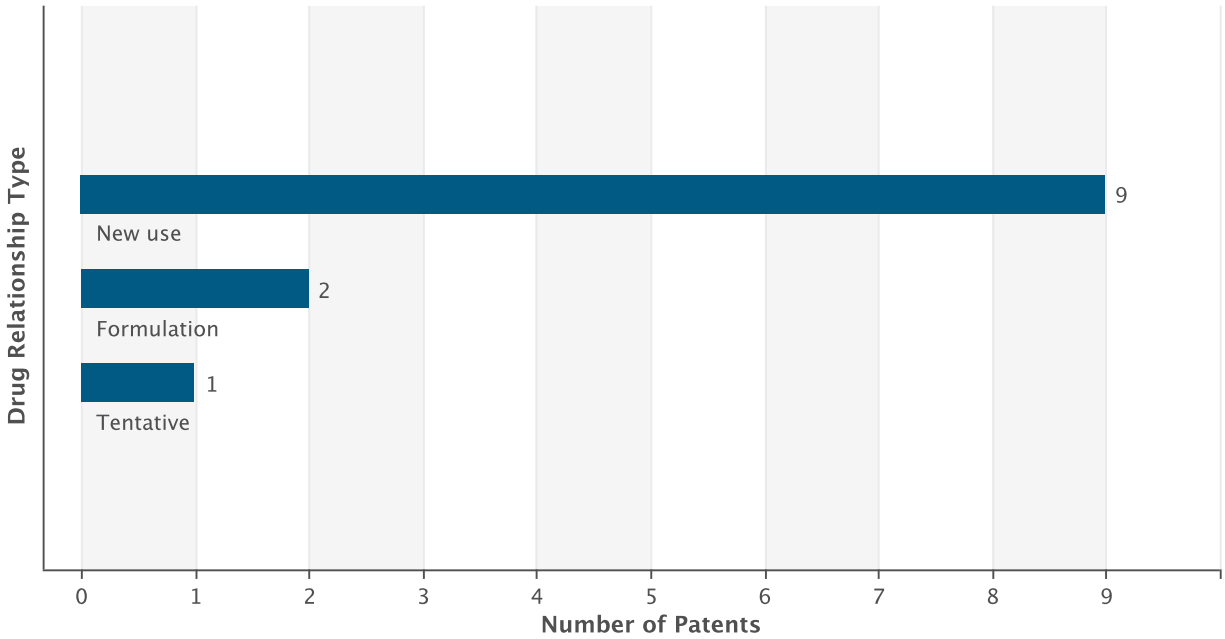
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	9
Formulation	2
Tentative	1

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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	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

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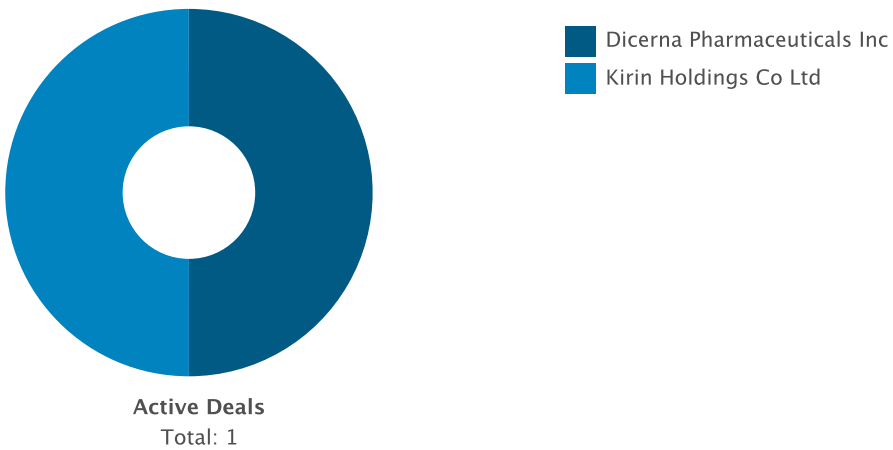
KHK-2 DRUG NAMES

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

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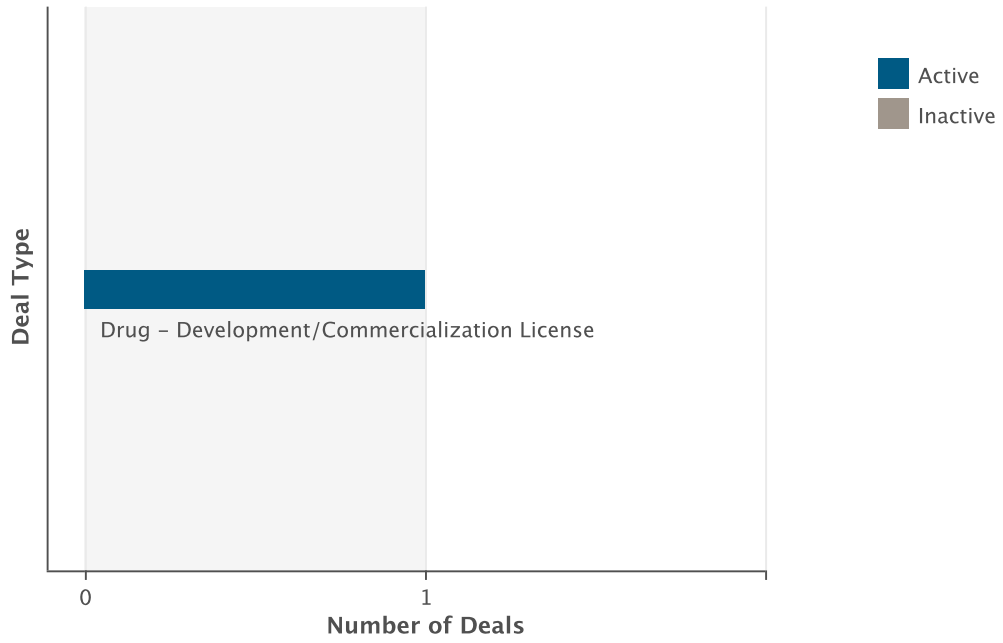
Deals by Type Chart



Deals by Type Table

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

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DsiRNAs targeting 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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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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