

## Dicerna Pharmaceuticals Inc

### COMPANY AND PIPELINE OVERVIEW REPORT

Coverage of the company and a summary of the drug pipeline portfolio.

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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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	14
Number of Inactive Drugs	2
Number of Patents as Owner	17
Number of Patents as Third Party	1
Number of Deals	4
Key Indications	Cancer, Prostate tumor, Hepatocellular carcinoma, Inflammatory disease, Lung tumor, Viral infection, Breast tumor, Cardiovascular disease, Colorectal tumor, Esophagus tumor, Hematological disease, Hepatitis C virus infection, Immune disorder, Liver disease, Metabolic disorder, Ovary tumor, Pancreas tumor, Renal tumor, Uterine cervix tumor
Key Target-based Actions	Androgen receptor modulator, Epidermal growth factor receptor modulator, TNF gene inhibitor, Beta-catenin modulator, E6 gene inhibitor, E7 gene inhibitor, Hypoxia inducible factor-1 alpha modulator, Hypoxia inducible factor-2 alpha inhibitor, K-Ras GTPase inhibitor, MYC gene inhibitor, Myc proto-oncogene protein inhibitor
Key Technologies	Oligonucleotide, Biological therapeutic, Small molecule therapeutic, Parenteral formulation unspecified, PEGylated formulation, Liposome formulation, Nanoparticle formulation, Gene expression regulation, Lipid, Oligonucleotide conjugated, Peptide, Polynucleotide

## 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 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

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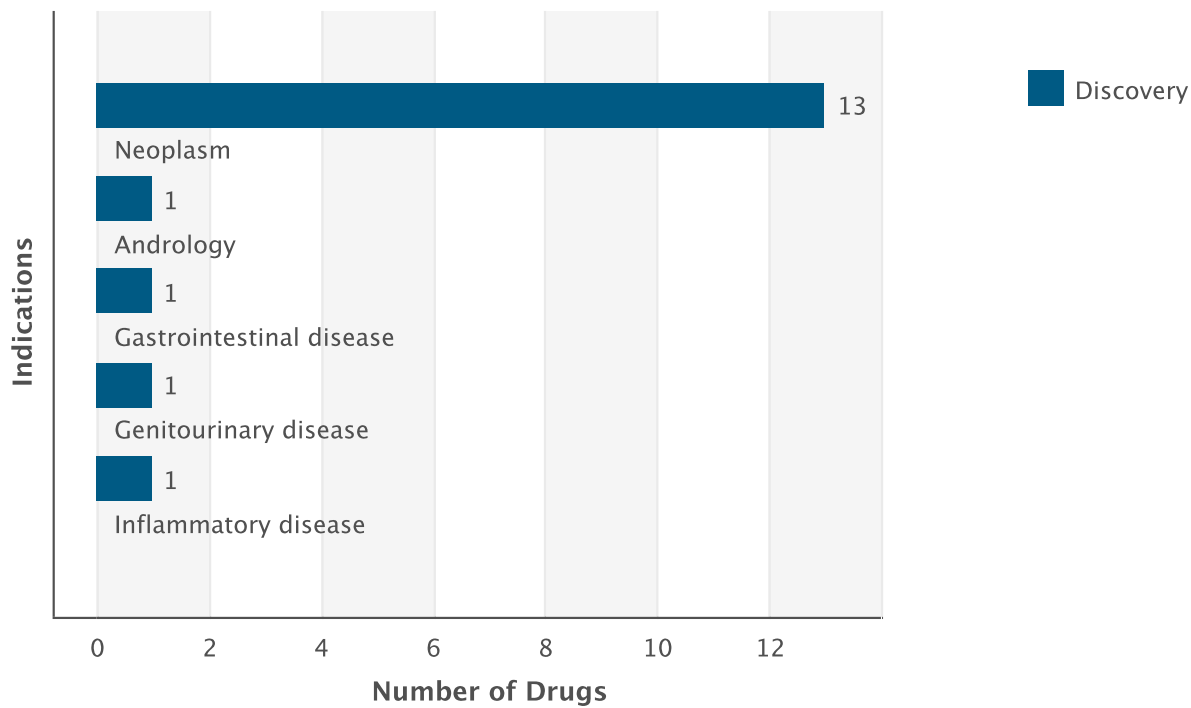
series A financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



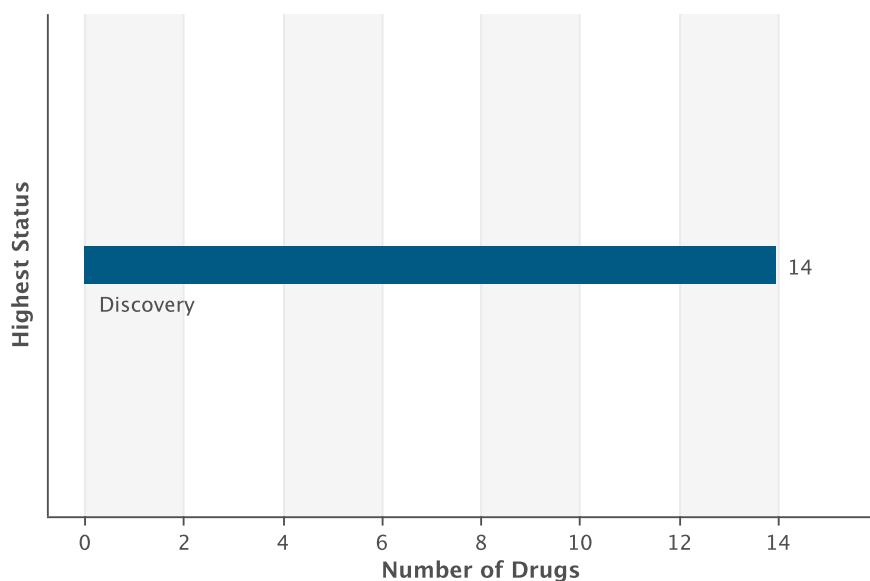
Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	13	1	14
Gastrointestinal disease	1	1	2
Inflammatory disease	1	1	2
Andrology	1	0	1
Genitourinary disease	1	0	1
Infectious disease	0	1	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
Discovery	14
No Development Reported	2

## 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	1	0	1

## PATENTS \*

Indication	As Owner	As Third Party	Total
Cardiovascular disease	2	0	2
Endocrine disease	3	0	3

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Gastrointestinal disease	8	1	9
Genitourinary disease	4	0	4
Hematological disease	2	0	2
Andrology	3	0	3
Immune disorder	3	0	3
Musculoskeletal disease	2	0	2
Neoplasm	17	1	18
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1
Respiratory disease	4	0	4
Infectious disease	6	1	7
Inflammatory disease	2	1	3
Otorhinolaryngological disease	1	0	1
Gynecology and obstetrics	2	0	2

\* 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 DRUGS

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

### DsiRNAs (inflammation), Dicerna/Kyowa Hakko Kirin

<b>Drug Name</b>	DsiRNAs (inflammation), Dicerna/Kyowa Hakko Kirin
<b>Key Synonyms</b>	
<b>Originator Company</b>	Dicerna Pharmaceuticals Inc
<b>Active Companies</b>	Dicerna Pharmaceuticals Inc, Kyowa Hakko Kirin Co Ltd
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Inflammatory disease
<b>Target-based Actions</b>	TNF gene inhibitor
<b>Other Actions</b>	Anti-inflammatory, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	06-Jul-2013

### KHK-1

<b>Drug Name</b>	KHK-1
<b>Key Synonyms</b>	
<b>Originator Company</b>	Dicerna Pharmaceuticals Inc
<b>Active Companies</b>	Kyowa Hakko Kirin Co Ltd, Dicerna Pharmaceuticals Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	K-Ras GTPase inhibitor
<b>Other Actions</b>	Oncogene inhibitor, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	15-Jan-2013

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## androgen receptor modulator (castration-resistant prostate cancer), Dicerna Pharmaceuticals

<b>Drug Name</b>	androgen receptor modulator (castration-resistant prostate cancer), Dicerna Pharmaceuticals
<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>	Discovery
<b>Active Indications</b>	Prostate tumor
<b>Target-based Actions</b>	Androgen receptor modulator
<b>Other Actions</b>	Anticancer
<b>Technologies</b>	Small molecule therapeutic
<b>Last Change Date</b>	12-Apr-2012

## DCR-M1711

<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>	Discovery
<b>Active Indications</b>	Hepatocellular carcinoma
<b>Target-based Actions</b>	Myc proto-oncogene protein inhibitor
<b>Other Actions</b>	Anticancer, siRNA agent
<b>Technologies</b>	Oligonucleotide, Nanoparticle formulation, Biological therapeutic, Small molecule therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	30-Jan-2013

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## KHK-1

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	
Other Actions	Anticancer, Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	06-Dec-2012

## KHK-2

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, Small molecule therapeutic
Last Change Date	30-Jan-2013

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### KHK-3

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	siRNA agent, Anticancer, Unspecified drug target
Technologies	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
Last Change Date	30-Jan-2013

### DsiRNAs targeting MYC gene (cancer), Dicerna

Drug Name	DsiRNAs targeting MYC gene (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	MYC gene inhibitor
Other Actions	Oncogene inhibitor, siRNA agent
Technologies	
Last Change Date	06-Dec-2012

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

<b>Drug Name</b>	DsiRNAs-targeting beta catenin (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Beta-catenin modulator
<b>Other Actions</b>	Anticancer, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

### DsiRNAs-targeting HIF1a (cancer), Dicerna

<b>Drug Name</b>	DsiRNAs-targeting HIF1a (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Hypoxia inducible factor-1 alpha modulator
<b>Other Actions</b>	Anticancer, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

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### DsiRNAs-targeting androgen receptor (cancer), Dicerna

<b>Drug Name</b>	DsiRNAs-targeting androgen receptor (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Androgen receptor modulator
<b>Other Actions</b>	siRNA agent, Anticancer
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

### DsiRNAs targeting HIF2a (cancer), Dicerna

<b>Drug Name</b>	DsiRNAs targeting HIF2a (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Hypoxia inducible factor-2 alpha inhibitor
<b>Other Actions</b>	Anticancer, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

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### DsiRNAs targeting HER2 (cancer), Dicerna

<b>Drug Name</b>	DsiRNAs targeting HER2 (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Epidermal growth factor receptor modulator
<b>Other Actions</b>	siRNA agent, Anticancer
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

### DsiRNAs targeting HER3 (cancer), Dicerna

<b>Drug Name</b>	DsiRNAs targeting HER3 (cancer), Dicerna
<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>	Discovery
<b>Active Indications</b>	Cancer
<b>Target-based Actions</b>	Epidermal growth factor receptor modulator
<b>Other Actions</b>	Anticancer, siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Small molecule therapeutic
<b>Last Change Date</b>	30-Jan-2013

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