

## Dicerna Pharmaceuticals Inc

### COMPANY AND PIPELINE OVERVIEW REPORT

A comprehensive coverage of the company and a summary of the drug pipeline portfolio.

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## ABOUT COMPANY AND PIPELINE OVERVIEW REPORT

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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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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**PLEASE NOTE:** the financials section where present in the report includes: Share Information, Stock Performance chart (including the consensus recommendation), and Major Shareholders. Financial Performance presents graphical and tabular data on Worldwide Sales, Operating Income and Net Income over time, together with a Quarterly earnings update. Balance Sheet lists Assets, Liabilities and Stockholders Equity, and Forecasts includes: EPS Forecast and Other Forecasts. The data reported in all sections (except share information and major stock holders) are correct as of the publication date of the report (and not the download date). For share information and major stock holders the data is correct for the date shown with these sections

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# 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>	7
<b>Number of Inactive Drugs</b>	12
<b>Number of Patents as Owner</b>	19
<b>Number of Patents as Third Party</b>	1
<b>Number of Deals</b>	4
<b>Key Indications</b>	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
<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, 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 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 6.9 million 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.

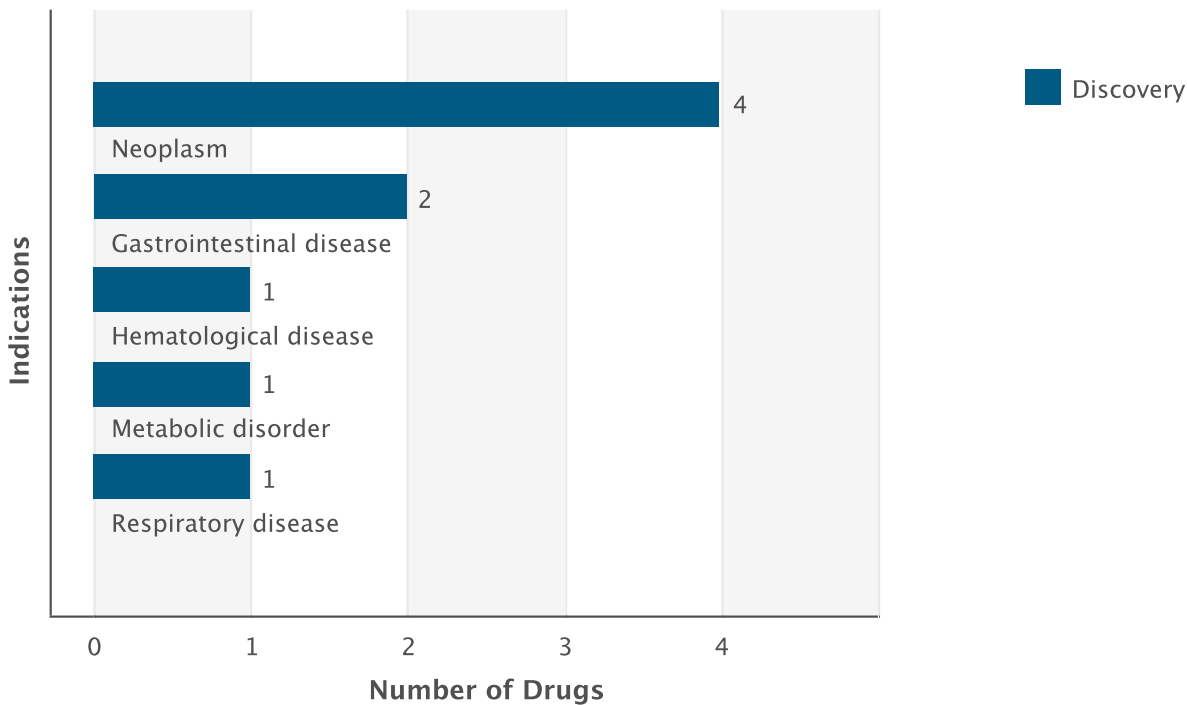
In July 2008, the company raised \$8.4 million in series A financing. By that time, Dicerna had raised \$21.4 million in series A financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



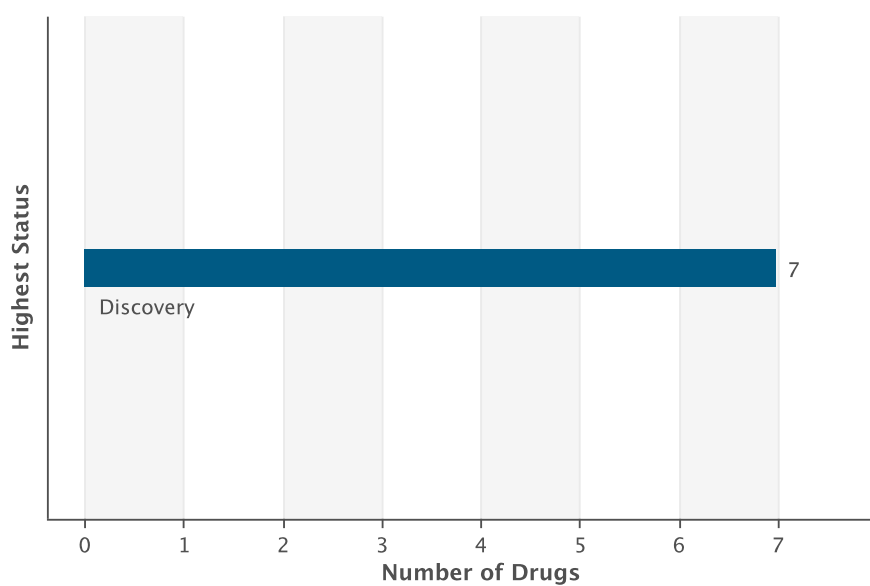
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## Drugs by Indication Table

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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## 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	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 DRUGS

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

### 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
<b>Last Change Date</b>	06-Feb-2014

### 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>	Solid tumor, 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, Parenteral formulation unspecified
<b>Last Change Date</b>	06-Jan-2014

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## KHK-2

<b>Drug Name</b>	KHK-2
<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>	
<b>Other Actions</b>	siRNA agent, Unspecified drug target, Anticancer
<b>Technologies</b>	Oligonucleotide, Biological therapeutic
<b>Last Change Date</b>	06-Feb-2014

## KHK-3

<b>Drug Name</b>	KHK-3
<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>	
<b>Other Actions</b>	siRNA agent, Anticancer, Unspecified drug target
<b>Technologies</b>	Oligonucleotide, Biological therapeutic
<b>Last Change Date</b>	04-Feb-2014

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## DCR-PH1

<b>Drug Name</b>	DCR-PH1
<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>	Hyperoxaluria
<b>Target-based Actions</b>	Oxidase inhibitor
<b>Other Actions</b>	
<b>Technologies</b>	Small molecule therapeutic
<b>Last Change Date</b>	07-Jan-2014

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

<b>Drug Name</b>	DsiRNAs targeting thrombin (clotting disorder), 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>	Blood clotting disorder
<b>Target-based Actions</b>	Factor IIa antagonist
<b>Other Actions</b>	siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	07-Jan-2014

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## DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals

<b>Drug Name</b>	DsiRNAs-targeting alpha 1 antitrypsin, 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>	Alpha-1 antitrypsin deficiency
<b>Target-based Actions</b>	Alpha 1 antitrypsin modulator
<b>Other Actions</b>	siRNA agent
<b>Technologies</b>	Oligonucleotide, Biological therapeutic, Parenteral formulation unspecified
<b>Last Change Date</b>	07-Jan-2014

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