

Dicerna Pharmaceuticals Inc

COMPANY AND PIPELINE OVERVIEW REPORT

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

Publication Date: 19-Nov-2014

THOMSON REUTERS

3 Times Square
New York, New York 10036
United States

Tel: +1 646 223 4000

thomsonreuters.com

[Return to Table of Contents](#)

ABOUT COMPANY AND PIPELINE OVERVIEW 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 and Pipeline Overview reports are the first in a series of reports that track pharmaceutical and biotechnology companies worldwide. Further report offerings planned to follow include: Company Detailed Pipeline and Company Competitive Landscape reports. 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 a significant number of 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](#)



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

[Return to Table of Contents](#)



TABLE OF CONTENTS

Company Overview..... 7

Company Profile..... 7

Product Portfolio Summary..... 8

Product Portfolio Drugs..... 11

[Return to Table of Contents](#)

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	8
Number of Inactive Drugs	12
Number of Patents as Owner	21
Number of Patents as Third Party	1
Number of Deals	5
Key Indications	Cancer,Liver disease,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,Lipid,Oligonucleotide conjugated,Intravenous formulation,PEGylated formulation,Infusion,Liposome formulation,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.

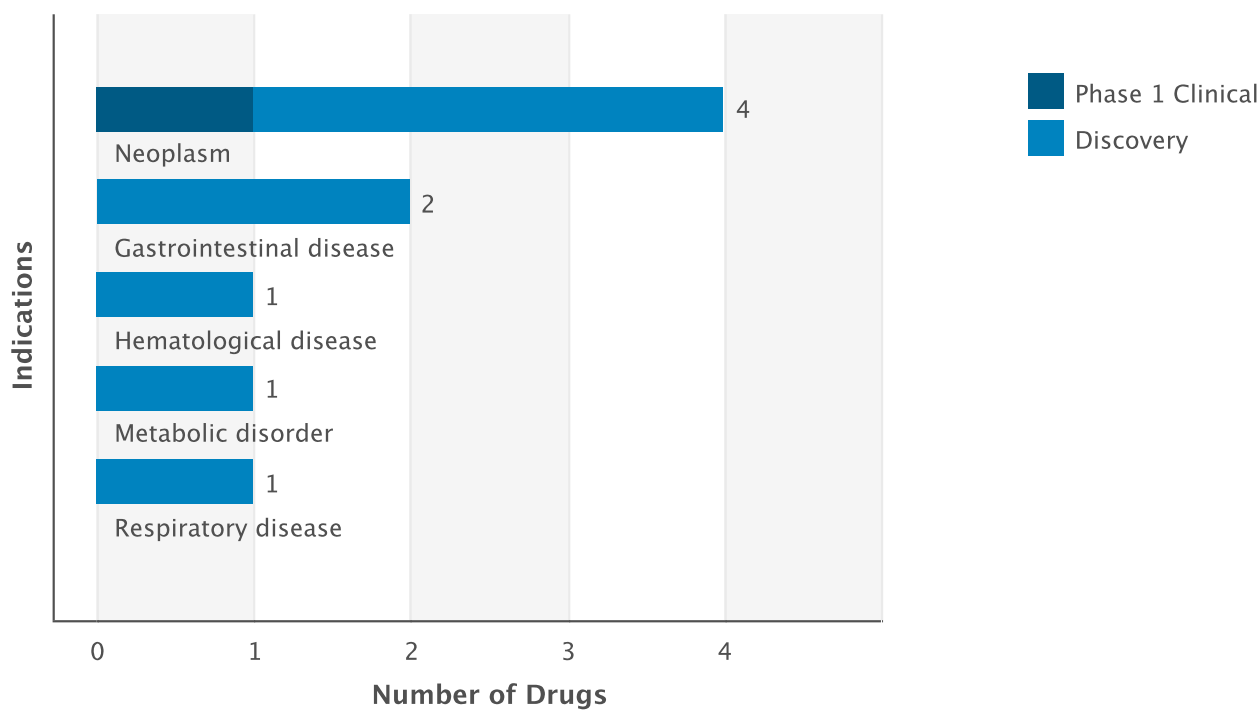
In July 2008, the company raised \$8.4 million in series A financing. By that time, Dicerna had raised \$21.4 million in series A financing.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



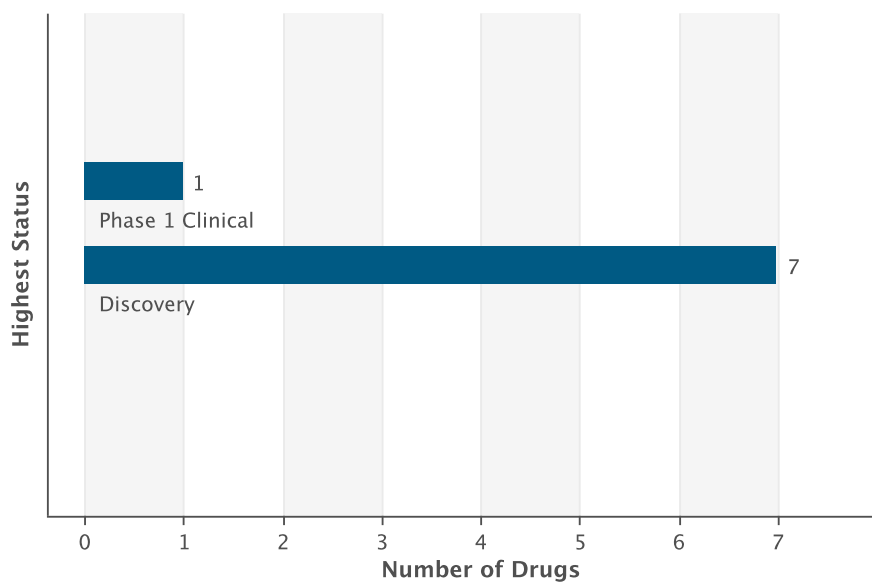
[Return to Table of Contents](#)

Drugs by Indication Table

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

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	5	0	5
Gastrointestinal disease	12	1	13
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	22	1	23
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1
Respiratory disease	7	0	7
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.

[Return to Table of Contents](#)

PRODUCT PORTFOLIO DRUGS

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

DCR-M1711

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	07-Nov-2014

KHK-1

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

[Return to Table of Contents](#)

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	Anticancer, Unspecified drug target, siRNA agent
Technologies	Oligonucleotide, Biological therapeutic
Last Change Date	06-Feb-2014

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	Unspecified drug target, Anticancer, siRNA agent
Technologies	Oligonucleotide, Biological therapeutic
Last Change Date	04-Feb-2014

[Return to Table of Contents](#)

DCR-PH1

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

DsiRNAs targeting thrombin (clotting disorder), Dicerna Pharmaceuticals

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

[Return to Table of Contents](#)

DsiRNAs-targeting alpha 1 antitrypsin, Dicerna Pharmaceuticals

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

DsiRNA-EX conjugates (liver diseases), Dicerna

Drug Name	DsiRNA-EX conjugates (liver diseases), Dicerna
Key Synonyms	
Originator Company	Dicerna Pharmaceuticals Inc
Active Companies	Dicerna Pharmaceuticals Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Liver disease
Target-based Actions	
Other Actions	siRNA agent
Technologies	Oligonucleotide conjugated, Biological therapeutic, Parenteral formulation unspecified
Last Change Date	18-Nov-2014

[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.

For subscription information, e-mail 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](#)

