

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

Company Name	Dicerna Pharmaceuticals Inc
Parent Company Name	Dicerna Pharmaceuticals Inc
Website	http://www.dicerna.com/
Country	US
Number of Drugs in Active Development	9
Number of Inactive Drugs	11
Number of Patents as Owner	23
Number of Patents as Third Party	1
Number of Deals	5
Key Indications	Cancer, Hepatocellular carcinoma, Liver disease, Alpha-1 antitrypsin deficiency, Blood clotting disorder, Hyperoxaluria, Lung tumor, Breast tumor, Ovary tumor, Pancreas tumor, Renal tumor, Uterine cervix tumor, Viral
Key Target-based Actions	Androgen receptor modulator, Epidermal growth factor receptor modulator, Alpha 1 antitrypsin modulator, TNF gene inhibitor, 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, Liposome formulation, PEGylated formulation, Infusion, 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 December 2014, Dicerna had granted stock options to purchase 230,000 shares of common stock of the company to Dr Ashburn, its newly appointed Snr VP for product strategy and operations and the grant was approved by the board of directors of the company on December 09, 2014 as an inducement material. The exercise price of the stock options was \$13.08 per share.

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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.

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.

COMPANY FINANCIALS

SHARE INFORMATION

Close Price (USD)	20.23
Change (USD)	0.82 (4.22%)
Previous Close Price (USD)	19.41
Volume	97,044
52-Week High (USD)	46.00
52-Week Low (USD)	8.00
Date	16-Jan-2015
Exchange	NASDAQ(COMPOSITE)
Current Number of Shares Outstanding	17,773,287
Current Market Capitalization	359,553,596
EPS (Date Reported)	-1.19076

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STOCK PERFORMANCE



MAJOR SHAREHOLDERS

Shareholder	Type	Country	Shares held	% of shares held	Change in holding	% changes in shares held	Date
RTW Investments, LLC	Investment Advisor/Hedge Fund	United States	1,115,981	6.28	1,115,981	1	16-Dec-2014
Fidelity Growth Company Fund	Mutual Fund	United States	824,350	4.64	-21,700	-0.03	30-Nov-2014
Fidelity Puritan Fund	Mutual Fund	United States	384,600	2.16	0	0	30-Nov-2014
Franklin Templeton Invest-Biotechnology Discovery	Mutual Fund	United States	370,900	2.09	-138,900	-0.27	31-Oct-2014
Fidelity Select Biotechnology Portfolio	Mutual Fund	United States	293,221	1.65	0	0	30-Nov-2014
Fidelity OTC Portfolio	Mutual Fund	United States	253,207	1.42	0	0	30-Nov-2014
Fidelity Series Growth Company Fund	Mutual Fund	United States	197,728	1.11	0	0	30-Nov-2014
Fidelity Blue Chip Growth Fund	Mutual Fund	United States	161,260	0.91	0	0	30-Nov-2014

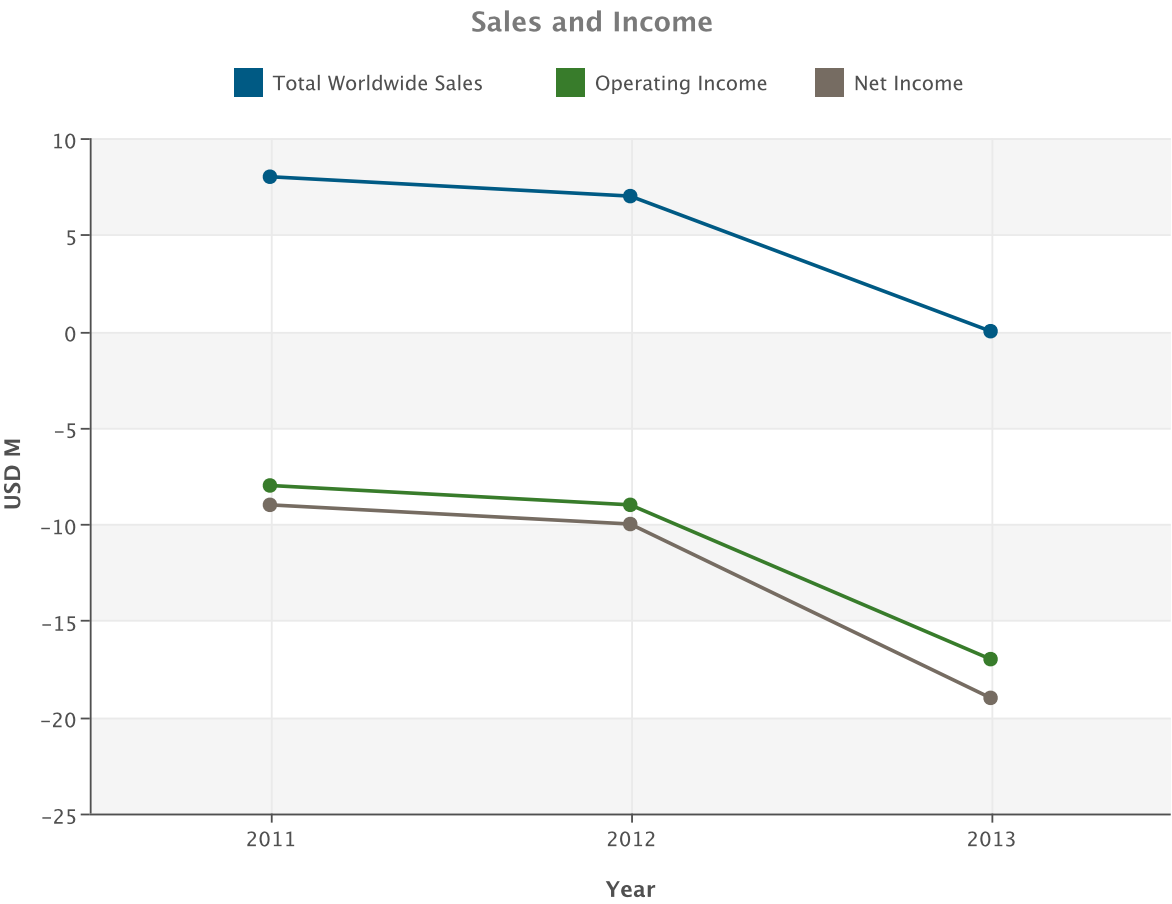
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Fidelity Series Small Cap Opportunities Fund	Mutual Fund	United States	198,493	1.12	658	0	31-Oct-2014
Vanguard Total Stock Market Index Fund	Mutual Fund	United States	138,743	0.78	23,800	0.21	30-Nov-2014
iShares Russell 2000 ETF	Mutual Fund	United States	64,262	0.36	8,333	0.15	31-Dec-2014
Fidelity Series Blue Chip Growth Fund	Mutual Fund	United States	78,600	0.44	0	0	30-Nov-2014
Vanguard Extended Market Index Fund	Mutual Fund	United States	62,479	0.35	3,400	0.06	30-Nov-2014
JP Morgan Small Cap Core Fund	Mutual Fund	United States	74,000	0.42	-2,900	-0.04	31-Oct-2014
JPMorgan Small Cap Value Fund	Mutual Fund	United States	50,900	0.29	1,200	0.02	30-Nov-2014
Fidelity Stock Selector Small Cap Fund	Mutual Fund	United States	60,228	0.34	200	0	31-Oct-2014
Fidelity Advisor Biotechnology Fund	Mutual Fund	United States	45,606	0.26	0	0	30-Nov-2014
Fidelity Spartan Extended Market Index Fund	Mutual Fund	United States	41,857	0.24	21,009	1.01	30-Nov-2014
iShares Russell 2000 Growth ETF	Mutual Fund	United States	26,297	0.15	2,044	0.08	31-Dec-2014
Fambrough (Douglas M III)	Individual Investor	United States	19,800	0.11	2,500	0.14	17-Dec-2014

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FINANCIAL PERFORMANCE



	2011	2012	2013		
Sales (USD M)	7.908	7.015	0		
Operating Income (USD M)	-7.613	-9.25	-17.378		
Net Income (USD M)	-8.556	-10.121	-18.518		

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Quarterly earnings update

Period	Sales (USD M)	Operating Income (USD M)	Net Income (USD M)
2014 Q3	0	-11.214	-11.193
2014 Q2	0	-11.178	-11.355
2014 Q1	0	-8.092	-11.008
2013 Q4	0	-6.437	-6.728
2013 Q3	0	-3.732	-4.54
2013 Q1	0	-3.554	-4.83
2012 Q4	6.253	1.697	0.484
2012 Q3	0.254	-3.693	-4.935

BALANCE SHEET

Assets (USD M)

	2011	2012	2013		
Cash & Short Term Investments	22.49	3.67	46.595		
Inventories - Total	0	0	0		
Current Assets - Total	23.023	9.032	48.653		
Property, Plant And Equipment - Net	1.314	0.883	0.877		
Other Assets - Total	0.036	0.012	0		
Total Assets	24.637	10.191	49.794		

Liabilities (USD M)

	2011	2012	2013		
Accounts Payable	1.414	1.198	1.7		
Short Term Debt & Current Portion Of Long Term Debt	2.834	4.14	4.587		
Current Liabilities - Total	5.299	5.671	7.678		
Long Term Debt	8.735	4.66	0.26		
Other Liabilities	0.901	0.331	0.529		
Total Liabilities	15.119	10.662	8.467		

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Stockholders Equity (USD M)

	2011	2012	2013		
Minority Interest	0	0	0		
Preferred Stock	60.151	64.248	110.246		
Common Stock	0.001	0.001	0.001		
Treasury Stock		0			
Total Liabilities & Shareholders' Equity	24.637	10.191	49.794		

FORECASTS

EPS Forecast

Year ending	Current Forecast	1 month ago	3 months ago
31-Dec-2015	-3.09	-3.09	-3.08
31-Dec-2016	-3.07	-3.07	-3.61
31-Dec-2017	-3.67	-3.67	-4.15

Other Forecasts

Year ending	DPS	CPS	Pretax	Sales
31-Dec-2015			-55.56	2.15
31-Dec-2016			-72.97	13.9
31-Dec-2017			-87.83	38.37

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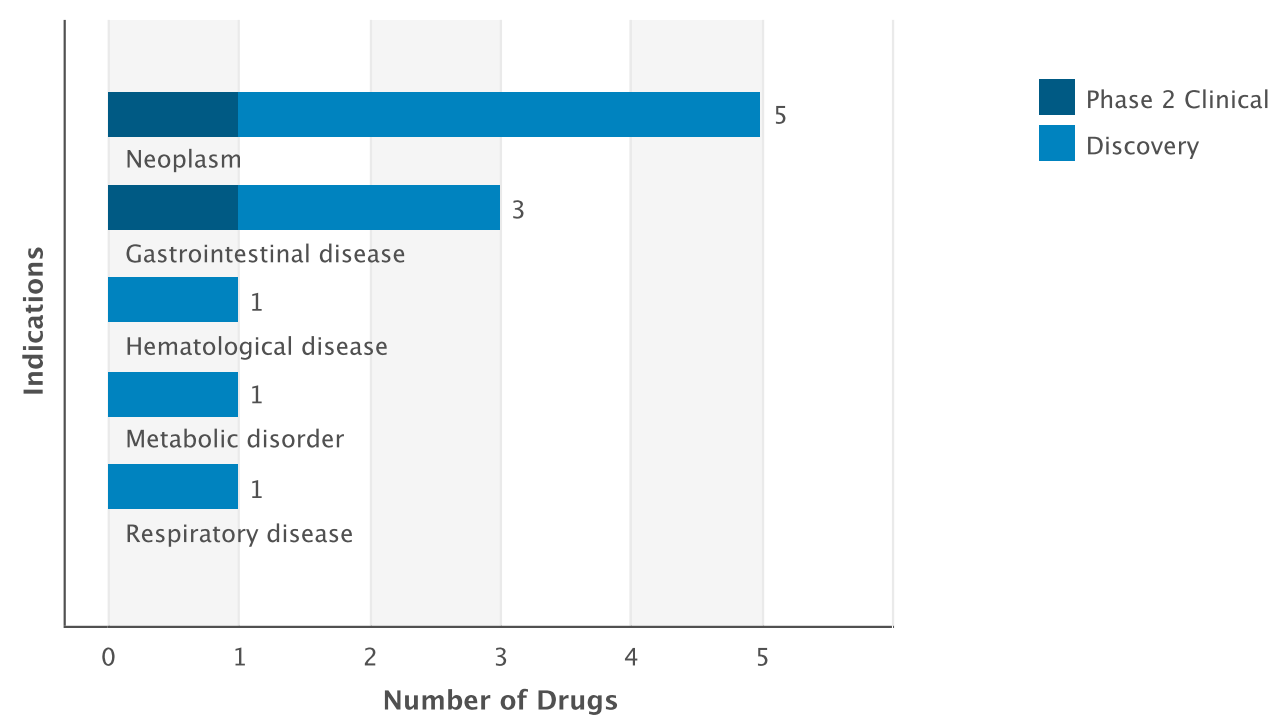


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



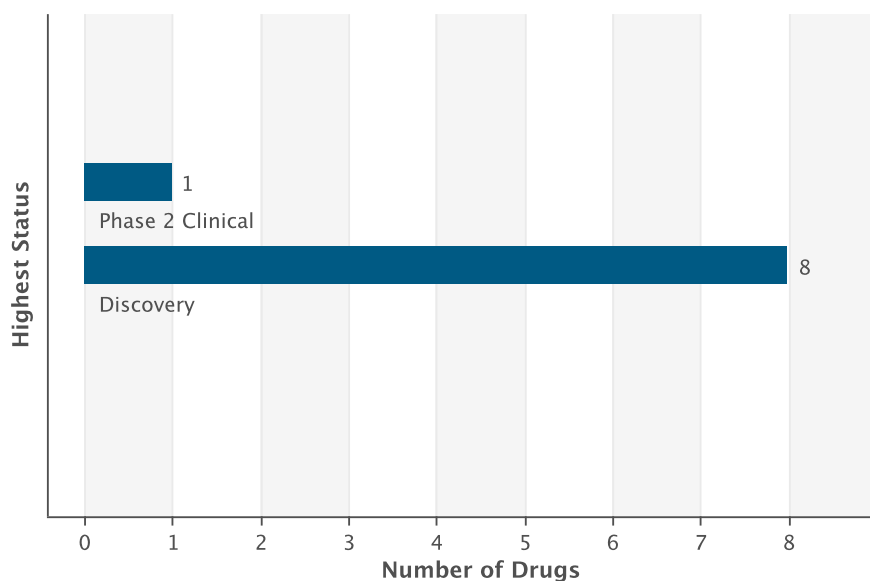
Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	5	7	12
Gastrointestinal disease	3	1	4
Unidentified indication	0	2	2
Inflammatory disease	0	2	2
Metabolic disorder	1	0	1
Hematological disease	1	0	1
Infectious disease	0	1	1
Respiratory disease	1	0	1
Andrology	0	1	1
Genitourinary 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
Phase 2 Clinical	1
Discovery	8
No Development Reported	11

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
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Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	2	2
Immune disorder	1	1
Gastrointestinal disease	1	1
Hematological disease	1	1

Trials by Phase

Phase	Ongoing	All
Phase 1	2	2

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	5	0	5
Gastrointestinal disease	13	1	14
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	24	1	25
Metabolic disorder	2	0	2
Mouth disease	1	0	1
Neurological disease	1	0	1

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Respiratory disease	7	0	7
Infectious disease	7	1	8
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 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 2 Clinical
Active Indications	Cancer, Hepatocellular carcinoma
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	13-Jan-2015

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	siRNA agent, Oncogene inhibitor
Technologies	Oligonucleotide, Biological therapeutic
Last Change Date	06-Feb-2014

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

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DsiRNAs-targeting beta catenin (cancer), Dicerna

Drug Name	DsiRNAs-targeting beta catenin (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	Beta-catenin modulator
Other Actions	siRNA agent, Anticancer
Technologies	Oligonucleotide, Biological therapeutic
Last Change Date	06-Dec-2014

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

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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

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

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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, Unspecified drug target
Technologies	Oligonucleotide, Oligonucleotide conjugated, Biological therapeutic, Parenteral formulation unspecified
Last Change Date	18-Nov-2014

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