

# **Karyopharm Therapeutics Inc**

# **COMPANY AND PIPELINE OVERVIEW REPORT**

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

Publication Date: 18-Feb-2014

### **THOMSON REUTERS**

3 Times Square New York, New York 10036 United States

Tel: +1 646 223 4000

thomsonreuters.com



# 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.



#### **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 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.

THOMSON REUTERS

**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



# **TABLE OF CONTENTS**

Company Overview	7
Company Profile	7
Subsidiary Companies	7
Product Portfolio Summary	8
Product Portfolio Drugs	1.



# **Karyopharm Therapeutics Inc**

#### **COMPANY OVERVIEW**

Company Name	Karyopharm Therapeutics Inc
Parent Company Name	Karyopharm Therapeutics Inc
Website	http://karyopharm.com/
Country	US
Number of Drugs in Active Development	7
Number of Inactive Drugs	3
Number of Patents as Owner	6
Number of Patents as Third Party	0
Number of Deals	5
Key Indications	Cancer,Inflammatory disease,Systemic lupus erythematosus,Viral infection,Autoimmune disease,Endometrioid carcinoma,Female genital tract tumor,Glioblastoma,Hematological neoplasm,Multiple sclerosis,Ovary tumor,Sarcoma,Solid tumor,Uterine cervix tumor
Key Target-based Actions	Exportin 1 inhibitor,Abl tyrosine kinase inhibitor,Bcr protein inhibitor,Bromodomain containing protein inhibitor,Cyclin-dependent kinase inhibitor 1B stimulator,Nuclear factor kappa B inhibitor,Nuclear pore complex protein modulator,PAK-4 protein kinase inhibitor,XPO1 gene modulator,p53 tumor suppressor protein stimulator
Key Technologies	Small molecule therapeutic, Oral formulation, Dermatological formulation

#### **COMPANY PROFILE**

#### **SUMMARY**

Karyopharm, headquartered in Newton, MA, is a clinical-stage pharmaceutical company involved in the discovery and development of novel first-in-class drugs directed against nuclear transport targets for the treatment of cancer and other major diseases.

#### **FINANCIAL**

In December 2013, Karyopharm was added to the Russell 3000 and Russell 2000 Indexes as part of Russell Investments.

In October 2013, the company planned an IPO of its common stock; at that time a Form S-1 registration statement had been filed with the US Securities and Exchange Commission; in November 2013, the IPO of of 6.8 million shares were priced at \$16 per share. The underwriters were granted a 30-day option to purchase up to an additional 1.02 million shares of common stock. The shares were to be traded on the NASDAQ Global Select Market under the ticker symbol 'KPTI'. At that time, the offering was expected to close on November 12, 2013. In December 2013, the underwriters fully exercised their option to purchase an additional 1.02 million shares of common stock at the public offering price of \$16 each. Proceeds to Karyopharm from the exercise of the option were approximately \$15.2 million, including which gross proceeds to Karyopharm from the initial public offering were \$125.1 million.

In May 2013, Karyopharm raised \$48.2 million from a series B financing round. In July 2013, the company raised \$19 million in a series B1 financing.

In November 2010, Karyopharm raised \$20 million from a series A financing round. In November 2011, Karyopharm raised \$10 million from a series A2 financing round and by that time, the company had raised \$32 million from both series A and series A2 financing rounds.



### **SUBSIDIARY COMPANIES**

#### **Names**

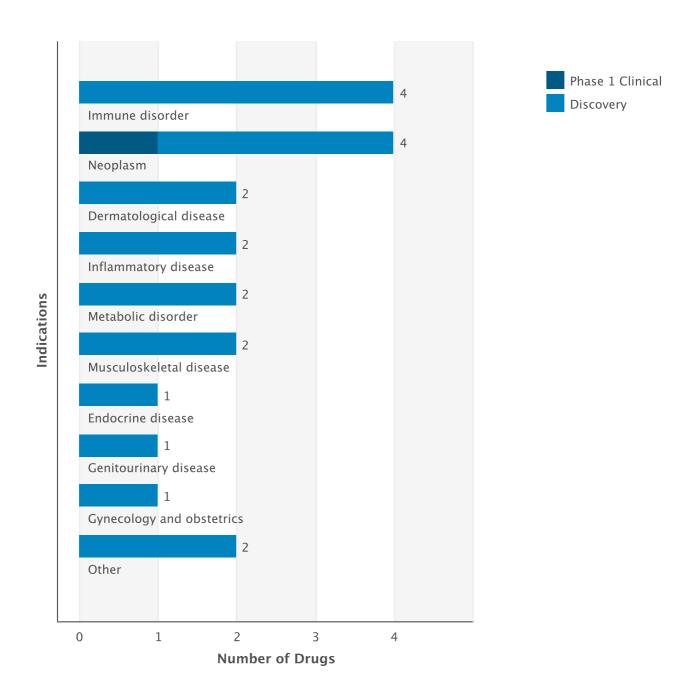
NPM Pharma Inc

#### PRODUCT PORTFOLIO SUMMARY

### **DRUGS**

### Drugs by Indication

Active Drugs by Indication Chart



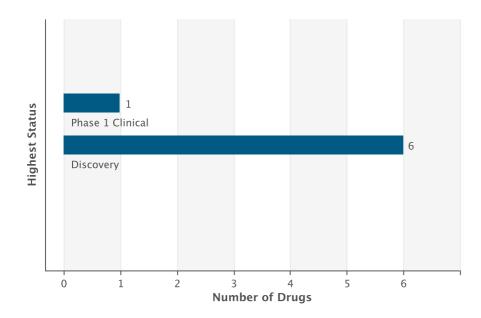


# Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	4	2	6
Immune disorder	4	1	5
Dermatological disease	2	1	3
Infectious disease	1	2	3
Musculoskeletal disease	2	0	2
Metabolic disorder	2	0	2
Inflammatory disease	2	0	2
Neurological disease	1	0	1
Genitourinary disease	1	0	1
Endocrine disease	1	0	1
Gynecology and obstetrics	1	0	1
Hematological disease	1	0	1
Unidentified indication	0	1	1

# **Drugs by Highest Status**

Active Drugs by Highest Status Chart





### Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 1 Clinical	1
Discovery	6
Discontinued	1
No Development Reported	2

### **DEALS**

Deal Type	Prin	cipal	Par	tner	Total
	Active	Inactive	Active	Inactive	
Drug - Discovery/Design	0	0	1	0	1
Drug - Funding	2	0	0	0	2
Drug - Early Research/Development	0	0	1	0	1
Drug - Development Services	0	0	1	0	1

#### **CLINICAL TRIALS**

### Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	5	5
Genitourinary disease	1	1
Endocrine disease	1	1
Gynecology and obstetrics	1	1
Hematological disease	1	1

### Trials by Phase

Phase	Ongoing	All
Phase 2	2	2
Phase 1	3	3

### **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)



### **PATENTS** \*

Indication	As Owner	As Third Party	Total
Cardiovascular disease	2	0	2
Gastrointestinal disease	2	0	2
Genitourinary disease	4	0	4
Immune disorder	2	0	2
Psychiatric disorder	1	0	1
Neoplasm	5	0	5
Ocular disease	2	0	2
Neurological disease	2	0	2
Nutritional disorder	1	0	1
Respiratory disease	4	0	4
Infectious disease	5	0	5
Inflammatory disease	5	0	5
Dermatological disease	1	0	1

<sup>\*</sup> This table represents a summary of the core patent coverage for this company covering Therapeutic EP, US and WO patents since 1990 only.

THOMSON REUTERS

#### PRODUCT PORTFOLIO DRUGS

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

#### selinexor

Drug Name	selinexor
Key Synonyms	selinexor
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Endometrioid carcinoma, Hematological neoplasm, Glioblastoma, Cancer, Solid tumor, Uterine cervix tumor, Ovary tumor, Female genital tract tumor, Viral infection, Systemic lupus erythematosus, Sarcoma
Target-based Actions	Exportin 1 inhibitor, Abl tyrosine kinase inhibitor, Cyclin-dependent kinase inhibitor 1B stimulator, Bcr protein inhibitor
Other Actions	Dermatological agent, Antiviral, Synergist, Cell cycle inhibitor, Anti- inflammatory, Anticancer, Apoptosis stimulator, Anticancer protein kinase inhibitor
Technologies	Oral formulation, Small molecule therapeutic
Last Change Date	07-Feb-2014

# nuclear import inhibitor (cancer), Karyopharm Therapeutics

Drug Name	nuclear import inhibitor (cancer), Karyopharm Therapeutics
Key Synonyms	
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Unspecified drug target, Anticancer
Technologies	Small molecule therapeutic
Last Change Date	19-Sep-2013



# **KPT-355**

Drug Name	KPT-355
Key Synonyms	
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	Rega Institute for Medical Research
Highest Status	Discovery
Active Indications	Inflammatory disease, Autoimmune disease
Target-based Actions	Exportin 1 inhibitor
Other Actions	Anticancer, Anti-inflammatory, Antiviral
Technologies	Oral formulation, Small molecule therapeutic
Last Change Date	28-Nov-2013

### **KPT-350**

Drug Name	KPT-350
Key Synonyms	
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Inflammatory disease, Systemic lupus erythematosus
Target-based Actions	Nuclear factor kappa B inhibitor, Exportin 1 inhibitor
Other Actions	Immunosuppressant, Anti-inflammatory
Technologies	Oral formulation, Small molecule therapeutic
Last Change Date	28-Nov-2013

THOMSON REUTERS

# anticancer compounds, Karyopharm Therapeutics

Drug Name	anticancer compounds, Karyopharm Therapeutics
Key Synonyms	
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Unspecified drug target, Anticancer
Technologies	Small molecule therapeutic
Last Change Date	04-Jun-2013

# PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics

Drug Name	PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics
Key Synonyms	
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	PAK-4 protein kinase inhibitor
Other Actions	Anticancer protein kinase inhibitor
Technologies	Oral formulation, Small molecule therapeutic
Last Change Date	23-Jan-2014

THOMSON REUTERS

# SINE compounds (multiple sclerosis), Mount Sinai School of Medicine/ Karyopharm Therapeutics

Drug Name	SINE compounds (multiple sclerosis), Mount Sinai School of Medicine/ Karyopharm Therapeutics
Key Synonyms	
Originator Company	Mount Sinai School of Medicine
Active Companies	Karyopharm Therapeutics Inc, Mount Sinai School of Medicine
Inactive Companies	
Highest Status	Discovery
Active Indications	Multiple sclerosis
Target-based Actions	Exportin 1 inhibitor
Other Actions	Anti-inflammatory
Technologies	Small molecule therapeutic
Last Change Date	10-Jan-2014



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.

THOMSON REUTERS