

## **Karyopharm Therapeutics Inc**

## **CORTELLIS COMPANY DETAILED PIPELINE REPORT**

A comprehensive coverage of the the company's drug pipeline portfolio including detailed product records.

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#### **THOMSON REUTERS**

3 Times Square New York, New York 10036 United States

Tel: +1 646 223 4000

thomsonreuters.com



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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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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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## **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	7
Number of Patents as Third Party	0
Number of Deals	9
Key Indications	Cancer,Inflammatory disease,Systemic lupus erythematosus,Viral infection,Autoimmune disease,Acute myelogenous leukemia,Endometrioid carcinoma,Female genital tract tumor,Glioblastoma,Hematological neoplasm,Hormone refractory prostate cancer,Multiple sclerosis,Ovary tumor,Rectal tumor,Sarcoma,Solid tumor,Uterine cervix tumor
Key Target-based Actions	Exportin 1 inhibitor,PAK-4 protein kinase inhibitor,Abl tyrosine kinase inhibitor,Bcr protein inhibitor,Bromodomain containing protein inhibitor,Cyclindependent kinase inhibitor 1B stimulator,Nuclear factor kappa B inhibitor,Nuclear pore complex protein modulator,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 June 2014, Karyopharm commenced an underwritten public offering of 2,200,000 shares of its common stock, including 200,000 shares to be offered by existing stockholders. At that time, the company intended to grant underwriters a 30-day option to purchase up to an additional 330,000 shares of common stock. Later that month, the company priced the underwritten public offering of 2,647,247 common stock shares at \$42.50 per share. The underwriters were granted a 30-day option to buy upto an additional 397,087 common stock shares. At that time, the offering was expected to close on or about July 02, 2014. Again later that month, the underwriters have exercised their option to purchase the additional shares and the company expected to raise approximately \$120.9 million through the underwritten public offering after the option exercise.

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.



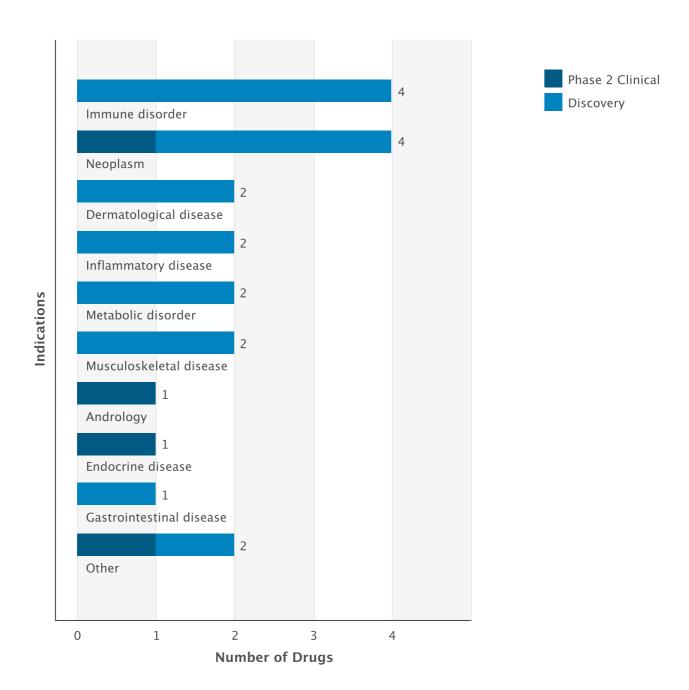
In November 2010, Karyopharm raised \$20 million from a series A financing round. In November 2011, Karyopharm raised \$10 million from a series A2 financing round and by that time, the company had raised \$32 million from both series A and series A2 financing rounds.

## PRODUCT PORTFOLIO SUMMARY

#### **DRUGS**

#### **Drugs by Indication**

Active Drugs by Indication Chart





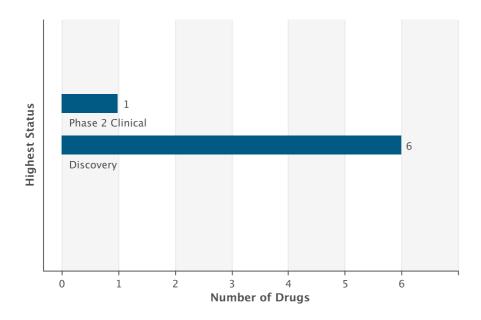
## Drugs by Indication Table

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
Unidentified indication	0	1	1
Hematological disease	1	0	1
Genitourinary disease	1	0	1
Endocrine disease	1	0	1
Neurological disease	1	0	1
Gastrointestinal disease	1	0	1
Gynecology and obstetrics	1	0	1
Andrology	1	0	1



## **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	6
Discontinued	1
No Development Reported	2

#### **DEALS**

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Discovery/Design	0	0	1	0	1
Drug - Funding	3	0	0	0	3
Drug - Early Research/Development	0	0	1	0	1
Drug - Development Services	0	0	4	0	4



#### **CLINICAL TRIALS**

#### Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	13	14
Hematological disease	5	6
Genitourinary disease	2	2
Immune disorder	1	2
Gastrointestinal disease	1	1
Andrology	1	1
Gynecology and obstetrics	1	1
Endocrine disease	1	1
Dermatological disease	1	1

## Trials by Phase

Phase	Ongoing	All
Phase 2	5	5
Phase 1	8	9

#### **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
Gastrointestinal disease	2	0	2
Genitourinary disease	4	0	4
Hematological disease	1	0	1
Immune disorder	3	0	3
Psychiatric disorder	1	0	1



Neoplasm	6	0	6
Ocular disease	2	0	2
Neurological disease	3	0	3
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.



## PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

#### selinexor

## selinexor SNAPSHOT

Drug Name	selinexor
Key Synonyms	selinexor
Originator Company	Karyopharm Therapeutics Inc
Active Companies	Karyopharm Therapeutics Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Endometrioid carcinoma;Hematological neoplasm;Glioblastoma;Cancer;Solid tumor;Rectal tumor;Acute myelogenous leukemia;Hormone refractory prostate cancer;Uterine cervix tumor;Systemic lupus erythematosus;Viral infection;Female genital tract tumor;Ovary tumor;Sarcoma
Target-based Actions	Exportin 1 inhibitor;Abl tyrosine kinase inhibitor;Cyclin-dependent kinase inhibitor 1B stimulator;Bcr protein inhibitor
Other Actions	DNA synthesis inhibitor;Anti-inflammatory;Anticancer protein kinase inhibitor;Synergist;Cell cycle inhibitor;Antiviral;Apoptosis stimulator
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	24-Jul-2014

#### selinexor DEVELOPMENT PROFILE

#### **SUMMARY**

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Karyopharm Therapeutics is developing selinexor (KPT-330; structure shown), a lead from a program of small-molecule nuclear transport inhibitors including KPT-276 (selective inhibitor of nuclear export; SINE; KPT-SINE), which targets nuclear pore complex machinery controlling the import and export of proteins between the nucleus and cytoplasm, including exportin 1 (CRM1), for the potential oral treatment of cancer, including blast crisis chronic myelogenous leukemia and Philadelphia-positive acute lymphoblastic leukemia (ALL), acute myeloid leukemia (AML), and T-cell ALL (T-ALL)prostate tumor, melanoma, osteosarcoma, NSCLC, neuroblastoma, glioblastoma, gynecologic malignancies and diffuse large B-cell lymphoma (DLBCL) .....; the company is also investigating KPT-330, for the potential treatment of systemic lupus erythematosus, HIV infection and rectal cancer. In June 2012, two phase I trials for cancer began; in June 2013, data were presented. In July 2013, a phase Ib trial was initiated in the US and Canada for sarcoma. By February 2014, phase I trials for solid tumors were ongoing in North America and Europe; at that time, a phase I trial was initiated in Singapore for solid tumors. In March 2014, a phase II trial in gynecological malignancies was initiated in Denmark; in April 2014, the trial was initiated in Belgium. In April 2014, a phase II trial for recurrent glioblastoma was initiated in the US and Denmark. In May 2014, a phase II trial for metastatic hormone-refractory prostate cancer was initiated in the US. In May 2013, the company expected to initiate pivotal trials in at least two indications in the first half of 2014. In July 2013, the company planned to initiate phase II/III trials in the first half of 2014. In March 2014, the company planned to initiate two registration-directed trials in relapsed hematological malignancies in 2014. At that time, the company also planned to initiate additional clinical trials in patients with Richter's syndrome, gynecological malignancies, advanced head and neck and lung cancer, hormone and/or chemotherapy refractory metastatic prostate cancer and relapsed glioblastoma multiforme; in April 2014, a phase II AML study was initiated. In May 2014, a phase I trial was planned in rectal cancer patients.

#### selinexor DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Acute myelogenous leukemia	US	Phase 2 Clinical	14-Apr-2014
Karyopharm Therapeutics Inc	Endometrioid carcinoma	Belgium	Phase 2 Clinical	24-Apr-2014
Karyopharm Therapeutics Inc	Endometrioid carcinoma	Denmark	Phase 2 Clinical	21-Mar-2014
Karyopharm Therapeutics Inc	Female genital tract tumor	Belgium	Phase 2 Clinical	24-Apr-2014
Karyopharm Therapeutics Inc	Glioblastoma	Denmark	Phase 2 Clinical	29-Apr-2014
Karyopharm Therapeutics Inc	Glioblastoma	US	Phase 2 Clinical	29-Apr-2014
Karyopharm Therapeutics Inc	Hormone refractory prostate cancer	US	Phase 2 Clinical	23-May-2014
Karyopharm Therapeutics Inc	Ovary tumor	Belgium	Phase 2 Clinical	24-Apr-2014
Karyopharm Therapeutics Inc	Ovary tumor	Denmark	Phase 2 Clinical	21-Mar-2014
Karyopharm Therapeutics Inc	Uterine cervix tumor	Belgium	Phase 2 Clinical	24-Apr-2014
Karyopharm Therapeutics Inc	Uterine cervix tumor	Denmark	Phase 2 Clinical	21-Mar-2014
Karyopharm Therapeutics Inc	Hematological neoplasm	Denmark	Phase 1 Clinical	25-Jul-2012
Karyopharm Therapeutics Inc	Hematological neoplasm	US	Phase 1 Clinical	18-Jun-2012
Karyopharm	Sarcoma	Canada	Phase 1 Clinical	31-Jul-2013



Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Sarcoma	US	Phase 1 Clinical	31-Jul-2013
Karyopharm Therapeutics Inc	Solid tumor	Canada	Phase 1 Clinical	08-Jun-2012
Karyopharm Therapeutics Inc	Solid tumor	Denmark	Phase 1 Clinical	25-Jul-2012
Karyopharm Therapeutics Inc	Solid tumor	Europe	Phase 1 Clinical	27-Feb-2014
Karyopharm Therapeutics Inc	Solid tumor	North America	Phase 1 Clinical	27-Feb-2014
Karyopharm Therapeutics Inc	Solid tumor	Singapore	Phase 1 Clinical	27-Feb-2014
Karyopharm Therapeutics Inc	Acute myelogenous leukemia	Canada	Discovery	24-Jun-2014
Karyopharm Therapeutics Inc	Acute myelogenous leukemia	Europe	Discovery	24-Jun-2014
Karyopharm Therapeutics Inc	Acute myelogenous leukemia	Israel	Discovery	24-Jun-2014
Karyopharm Therapeutics Inc	Cancer	US	Discovery	07-Jun-2010
Karyopharm Therapeutics Inc	Rectal tumor	Israel	Discovery	01-May-2014
Karyopharm Therapeutics Inc	Systemic lupus erythematosus	US	Discovery	14-Nov-2012
Karyopharm Therapeutics Inc	Viral infection	US	Discovery	12-May-2013

## selinexor CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:	
1393477-72-9	2	
F F N N	O H N N N N N N N N N N N N N N N N N N	
Name	Туре	
selinexor	PINN	
KPT-330	Research Code	

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CAS Registry Number:	Confidence Level:
	·
F F	
Name	Type
KPT-185	Research Code

CAS Registry Number:	Confidence Level: 4
F F	N N O O O O O O O O O O O O O O O O O O
Name	Туре
KPT-249	Research Code

CAS Registry Number:	Confidence Level:
	3
t t	
Name	Туре
KPT-251	Research Code



CAS Registry Number:	Confidence Level:
	3
t t t	N F
Name	Туре
KPT-276	Research Code

#### selinexor DRUG NAMES

Names	Туре
KPT-SINE	Research Code
nuclear transport inhibitors (inflammation/viral diseases/cancer), Karyopharm Therapeutics	
KPT-276	Research Code
Exportin-1 inhibitors (inflammation/viral diseases/cancer), Karyopharm Therapeutics	
selective nuclear export inhibitors (autoimmune/inflammation/HIV/cancer/dermatologica I disease), Karyopharm Therapeutics	
KPT-185	Research Code
KPT-251	Research Code
CRM-1 inhibitors (inflammation/viral diseases/cancer), Karyopharm Therapeutics	
KPT-330	Research Code
selective nuclear export inhibitors (autoimmune diseases/HIV/cancer), Karyopharm Therapeutics	
KPT-XXX	Research Code
KPT-225	Research Code
KPT-249	Research Code
KPT-207	Research Code
selinexor	PINN
SINE (autoimmune diseases/HIV/cancer), Karyopharm Therapeutics	



## selinexor CLINICAL TRIALS

## Trials by Phase and Condition Studied

	se 4 nical		se 3 nical		se 2 nical		se 1 nical	Pha Unspe		То	tal
On- going	All	On- going	All	On- going	All	On- going	All	On- going	All	On- going	AII
Acute my	elogenous	s leukemia	l								
0	0	0	0	1	1	2	2	0	0	3	3
Advance	d solid tum	nor									
0	0	0	0	0	0	2	2	0	0	2	2
Multiple r	myeloma										
0	0	0	0	0	0	0	2	0	0	0	2
Metastati	c rectal ca	incer									
0	0	0	0	0	0	1	1	0	0	1	1
Ovary tur	mor										
0	0	0	0	1	1	0	0	0	0	1	1
Acute lyn	nphoblasti	c leukemia	a								
0	0	0	0	0	0	1	1	0	0	1	1
Hematolo	ogical neop	olasm									
0	0	0	0	0	0	1	1	0	0	1	1
Glioblast	oma										
0	0	0	0	1	1	0	0	0	0	1	1
Diffuse la	arge B-cell	lymphoma	a								
0	0	0	0	1	1	0	0	0	0	1	1
Soft tissu	e sarcoma	3									
0	0	0	0	0	0	1	1	0	0	1	1
Melanom	ıa										
0	0	0	0	0	0	1	1	0	0	1	1
Endomet	rioid carcii	noma									
0	0	0	0	1	1	0	0	0	0	1	1



Uterine cervix tumor											
0	0	0	0	1	1	0	0	0	0	1	1
Mouth tumor											
0	0	0	0	0	0	0	0	1	1	1	1
Prostate	tumor										
0	0	0	0	1	1	0	0	0	0	1	1
Metastatic pancreas cancer											
0	0	0	0	0	0	0	1	0	0	0	1

#### Total Trials by Phase and Status

Pha Clir			Phase 2 Clinical		Phase 1 Clinical		Phase Unspecified		Total		
On- going	All	On- going	All	On- going	All	On- going	All	On- going	All	On- going	All
Total by Phase and Status											
0	0	0	0	5	5	8	11	1	1	14	17

#### **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

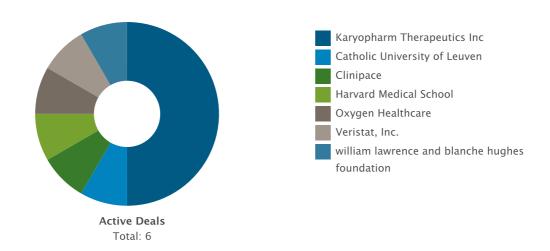
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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#### selinexor DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

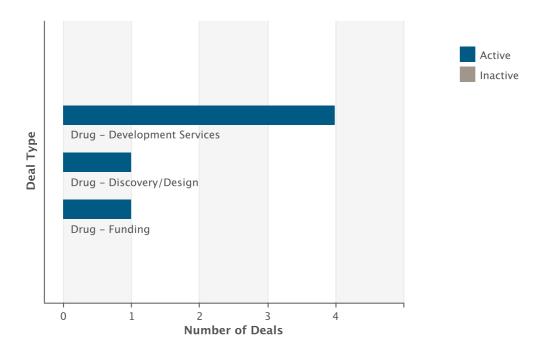


#### **Deals by Parent Company Table**

Company Name	Principal		Par	Total	
	Active	Inactive	Active	Inactive	
Karyopharm Therapeutics Inc	1	0	5	0	6
Oxygen Healthcare	1	0	0	0	1
william lawrence and blanche hughes foundation	0	0	1	0	1
Clinipace	1	0	0	0	1
Harvard Medical School	1	0	0	0	1
Veristat, Inc.	1	0	0	0	1
Catholic University of Leuven	1	0	0	0	1

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## **Deals by Type Chart**



## **Deals by Type Table**

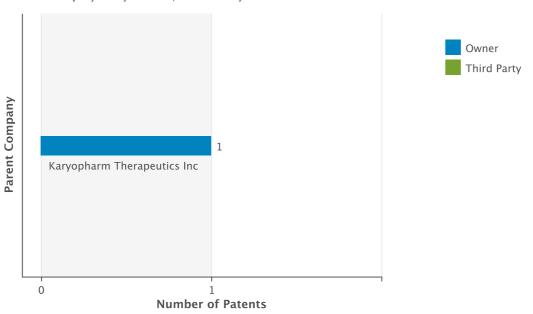
Deal Type	Active	Inactive	Total
Drug - Development Services	4	0	4
Drug - Discovery/Design	1	0	1
Drug - Funding	1	0	1



#### **PATENTS**

#### **Patents by Parent Company Chart**

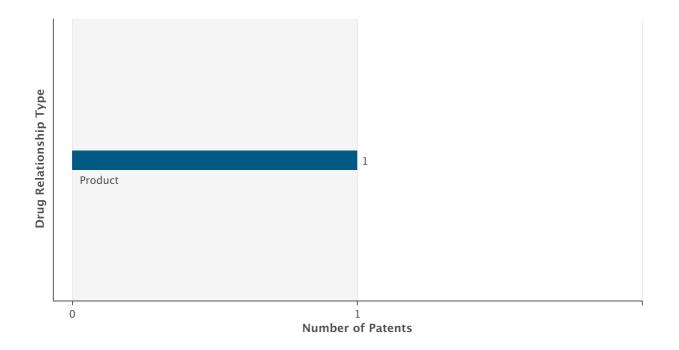
Chart displayed by Owner/Third Party



#### **Patents by Parent Company Table**

Company Name	As Owner	As Third Party	Total
Karyopharm Therapeutics Inc	1	0	1

## **Patents by Drug Relationship Type Chart**





## **Patents by Drug Relationship Type Table**

Drug Relationship	Total
Product	1



## anticancer compounds, Karyopharm Therapeutics

#### anticancer compounds, Karyopharm Therapeutics SNAPSHOT

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	Anticancer;Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	04-Jun-2013

#### anticancer compounds, Karyopharm Therapeutics DEVELOPMENT PROFILE

## **SUMMARY**

Karyopharm Therapeutics is investigating undisclosed therapeutics for the potential treatment of cancer. In June 2013, the program was listed as being in discovery.

#### anticancer compounds, Karyopharm Therapeutics DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Cancer	US	Discovery	04-Jun-2013

#### anticancer compounds, Karyopharm Therapeutics DRUG NAMES

Names	Туре
anticancer compounds, Karyopharm Therapeutics	

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## **KPT-350**

#### **KPT-350 SNAPSHOT**

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

#### **KPT-350 DEVELOPMENT PROFILE**

## **SUMMARY**

#### **KPT-350 DEVELOPMENT STATUS**

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	Development Status	Date
Karyopharm Therapeutics Inc	Inflammatory disease	US	Discovery	04-Jun-2013
Karyopharm Therapeutics Inc	Systemic lupus erythematosus	US	Discovery	04-Jun-2013

#### **KPT-350 DRUG NAMES**

Names	Туре
KPT-350	Research Code
oral NF-B inhibitor (inflammatory disease/systemic lupus erythematosus), Karyopharm Therapeutics	



## nuclear import inhibitor (cancer), Karyopharm Therapeutics

#### nuclear import inhibitor (cancer), Karyopharm Therapeutics SNAPSHOT

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	Anticancer;Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	19-Sep-2013

#### nuclear import inhibitor (cancer), Karyopharm Therapeutics DEVELOPMENT PROFILE

#### **SUMMARY**

Karyopharm Therapeutics is investigating nuclear import inhibitor for the potential treatment of cancer. In November 2012, the program was listed as being in discovery. In September 2013, the program was listed as being in preclinical development.

#### nuclear import inhibitor (cancer), Karyopharm Therapeutics DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Cancer	US	Discovery	22-Nov-2012

#### nuclear import inhibitor (cancer), Karyopharm Therapeutics DRUG NAMES

Names	Type
nuclear import inhibitor (cancer), Karyopharm Therapeutics	
nuclear import inhibitor, Karyopharm Therapeutics	



#### **KPT-355**

#### **KPT-355 SNAPSHOT**

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-355 DEVELOPMENT PROFILE**

#### **SUMMARY**

Karyopharm Therapeutics, in collaboration with Rega Institute for Medical Research, is investigating KPT-355, a lead from oral selective inhibitors of nuclear export (SINE) directed against the chromosomal region maintenance 1 receptor (CRM1; exportin 1), for the potential treatment of autoimmune and inflammatory diseases including psoriasis, systemic lupus erythematosus, multiple sclerosis and rheumatoid arthritis; viral infections including influenza, RSV and HIV infections; and cancer,.. In November 2012, the drug was listed as being in preclinical development. In October 2013, preclinical data were presented.

The company was previously investigating KPT-355, for the potential treatment of viral infections including influenza, RSV and HIV infections; and cancer,.. In November 2012, the drug was listed as being in preclinical development. However, in June 2013, the drug was no longer listed on the pipeline as being under investigation for viral infection and cancer

#### **KPT-355 DEVELOPMENT STATUS**

#### **CURRENT DEVELOPMENT STATUS**

CORRENT DEVELOPMENT STATUS				
Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Autoimmune disease	US	Discovery	07-Jun-2010
Karyopharm Therapeutics Inc	Inflammatory disease	US	Discovery	07-Jun-2010
Karyopharm Therapeutics Inc	Cancer	US	No Development Reported	04-Jun-2013
Karyopharm Therapeutics Inc	Viral infection	US	No Development Reported	04-Jun-2013



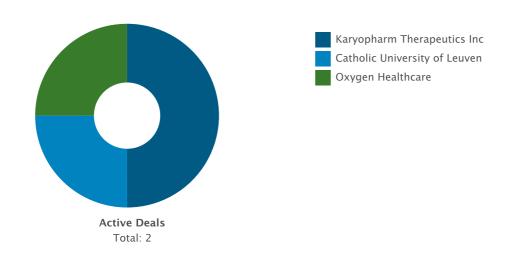
Company	Indication	Country	<b>Development Status</b>	Date
Rega Institute for Medical Research	Autoimmune disease	Belgium	No Development Reported	04-Jun-2013
Rega Institute for Medical Research	Cancer	US	No Development Reported	04-Jun-2013
Rega Institute for Medical Research	Inflammatory disease	Belgium	No Development Reported	04-Jun-2013
Rega Institute for Medical Research	Viral infection	Belgium	No Development Reported	04-Jun-2013

## **KPT-355 DRUG NAMES**

Names	Туре
KPT-355	Research Code

## **KPT-355 DEALS AND PATENTS**

# DEALS Deals by Parent Company Chart

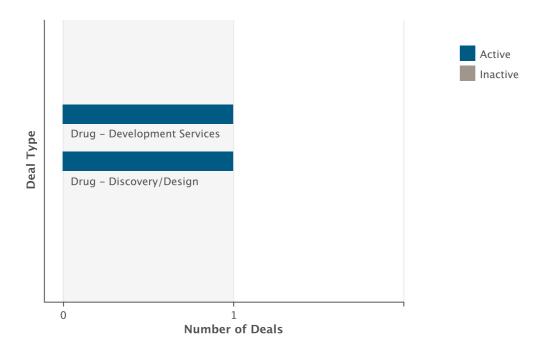


## **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive		tner Inactive	Total
Karyopharm Therapeutics Inc	0	0	2	0	2
Catholic University of Leuven	1	0	0	0	1
Oxygen Healthcare	1	0	0	0	1



## **Deals by Type Chart**



## **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Discovery/Design	1	0	1
Drug - Development Services	1	0	1



## PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics

#### PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics SNAPSHOT

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;Apoptosis stimulator;Cell cycle inhibitor
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	01-May-2014

### PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics DEVELOPMENT PROFILE

#### **SUMMARY**

Karyopharm Therapeutics is investigating small molecule inhibitors of P21-activated kinase 4 (PAK4) including KPT-8752 and KPT-7523, for the potential oral treatment of cancer including hematological and pancreatic cancer. In September 2013, development was ongoing.

#### PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Cancer	US	Discovery	19-Sep-2013

#### PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics DRUG NAMES

Names	Туре
KPT-7651	Research Code
KPT-8752	Research Code
PAK4 inhibitors (oral, cancer), Karyopharm Therapeutics	
KPT-7523	Research Code



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

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

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

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

#### **SUMMARY**

Karyopharm Therapeutics and Mount Sinai School of Medicine are investigating selective inhibitors of nuclear export (SINE) compounds that inhibit Exportin-1 for the potential treatment of multiple sclerosis and other inflammatory diseases. In January 2014, studies were planned in order to potentially select a candidate for early-stage clinical trials in multiple sclerosis.

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

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Karyopharm Therapeutics Inc	Multiple sclerosis	US	Discovery	08-Jan-2014
Mount Sinai School of Medicine	Multiple sclerosis	US	Discovery	08-Jan-2014

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

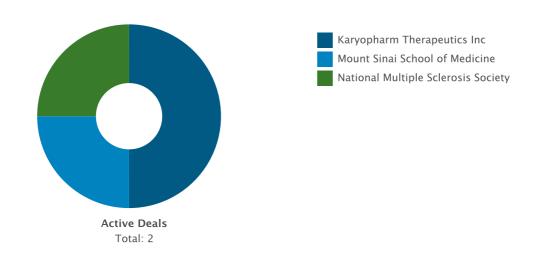
Names	Туре
selective inhibitors of nuclear export (multiple sclerosis), Mount Sinai School of Medicine/ Karyopharm Therapeutics	
SINE compounds (multiple sclerosis), Mount Sinai School of Medicine/ Karyopharm Therapeutics	



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

#### **DEALS**

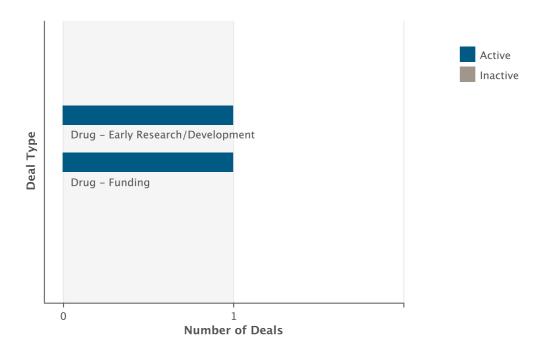
#### **Deals by Parent Company Chart**



## **Deals by Parent Company Table**

Company Name		cipal Inactive		tner Inactive	Total
Karyopharm Therapeutics Inc	1	0	1	0	2
Mount Sinai School of Medicine	1	0	0	0	1
National Multiple Sclerosis Society	0	0	1	0	1

## **Deals by Type Chart**



## **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1
Drug - Early Research/Development	1	0	1



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