

# **Intrexon Corp**

# **CORTELLIS COMPANY DETAILED PIPELINE REPORT**

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

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# ABOUT CORTELLIS COMPANY DETAILED PIPELINE 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 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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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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# **Intrexon Corp**

### **COMPANY OVERVIEW**

Company Name	Intrexon Corp
Parent Company Name	Intrexon Corp
Website	http://www.dna.com/
Country	US
Number of Drugs in Active Development	11
Number of Inactive Drugs	5
Number of Patents as Owner	20
Number of Patents as Third Party	1
Number of Deals	22
Key Indications	Cancer,Melioidosis,Autoimmune disease,Infectious disease,Melanoma,Alzheimers disease,Diabetes mellitus,Lung tumor,Respiratory disease,Solid tumor,Spinal muscular atrophy,Stage III
Key Target-based Actions	IL-12 agonist,Alpha 1 antitrypsin stimulator,DDC gene stimulator,SMN1 gene modulator,Ecdysone receptor modulator,ATP2A2 gene stimulator,Sarco endoplasmic calcium ATPase 2 modulator,Adiponectin receptor agonist,B-lymphocyte cell adhesion molecule stimulator,Beta defensin stimulator,Beta-galactosidase modulator,Bradykinin B2 receptor antagonist,C-type lectin domain protein 12A stimulator,CFTR stimulator,Casein modulator,Cyclooxygenase stimulator,Elongation factor 2 inhibitor,Erbb2 tyrosine kinase receptor inhibitor,Erythropoietin ligand,Glucagon-like peptide 1 agonist,Glucagon-like peptide 2 agonist,IL agonist,IL-10 agonist,IL-2 receptor alpha subunit stimulator,Interferon agonist,Interferon alpha ligand,Interferon beta ligand,Interferon gamma ligand modulator,Kallikrein inhibitor,Leptin agonist,Mesothelin stimulator,Myelin basic protein stimulator,Neural cell adhesion molecule stimulator,Plasma protease C1 inhibitor inhibitor,Plasminogen activator inhibitor 1 modulator,RANTES ligand,Signal transducer CD24 stimulator,TNF alpha ligand,TNF gene modulator,Trophoblast glycoprotein stimulator
Key Technologies	Biological therapeutic,Parenteral formulation unspecified,Monoclonal antibody human,Protein recombinant,Nanoparticle formulation,Gene transfer system viral,Cell therapy,Peptide,Antibody conjugated,Antibody polyclonal,Autologous stem cell therapy,Intratumoral formulation,Oral formulation,Subcutaneous formulation

# **COMPANY PROFILE**

#### **SUMMARY**

Intrexon Corp (formerly Genomatix) is a privately-held life science company which researches and develops DNA control systems for the delivery, targeting, activation, regulation and location of biotherapeutics. The company changed its corporate name to Intrexon in August 2005.

#### **COMPANY LOCATION**

The company has its headquarters in Blacksburg, VA, with additional R&D operations in Valley Forge, PA.

In October 2011, Intrexon launched a cell engineering unit in San Diego, CA.

In February 2011, Intrexon launched its Agricultural Biotechnology Division in Research Triangle Park, North Carolina.

In April 2010, Intrexon relocated its Protein Production Division from San Francisco, CA to Foster City, CA.

#### **ACQUISITIONS AND SPIN-OFFS**



In December 2013, Intrexon entered into a definitive agreement to acquire Medistem, for approximately \$26 million. Medistem stockholders would receive \$0.27 in cash in exchange for each share of Medistem common stock and \$1.08 worth of Intrexon common stock, based on the 20-day volume-weighted average price of Intrexon's common stock immediately prior to closing. The acquisition was subject to Intrexon's satisfactory completion of its due diligence of Medistem and its technology, customary closing conditions and Medistem stockholder approval.

In October 2012, Intrexon entered into a definitive agreement to acquire an approximately 48% interest in Aqua Bounty Technologies by purchasing 48,631,444 shares for \$6 million in cash. Following the close of the transaction, Intrexon would be required to commence a tender offer for any and all of the other outstanding shares of Aqua Bounty. In January 2013, Intrexon chose not to extend the tender offer to acquire outstanding shares in Aqua Bounty.

In October 2011, Intrexon acquired Immunologix, a company focused on the production of fully human monoclonal antibodies.

In October 2011, Intrexon announced the acquisition of GT Life Sciences, and the purchase of assets comprising the LEAP cell-processing platform from Cyntellect. Terms of the transactions were not disclosed.

In April 2011, the company acquired Neugenesis.

In February 2011, Intrexon acquired Agarigen a company which developed a mushroom-based expression platform for rapid, high-yield production of recombinant proteins.

In September 2009, Intrexon acquired certain assets and operations from Clinical Data's subsidiary Avalon Pharmaceutical, including a bioassay facility in Germantown, MD, for \$1.5 million in cash.

In December 2006, RheoGene and Intrexon agreed to merge. The combined company, Intrexon Corp, would be based in Blacksburg, VA, and the UPMC would have an ownership stake in the new company.

#### **FINANCIAL**

In July 2013, Intrexon filed a registration statement with the SEC for an IPO of its common stock. In August 2013, the company priced its IPO of 9,999,999 common stock shares at \$16 each. The underwriters were also granted a 30-day option to buy additional 1,499,999 common stock shares. At that time, trading of shares, under the symbol 'XON' on the New York Stock Exchange was expected to begin on August 8, 2013; later that month, the IPO was closed and a total of 11,499,998 common stock shares were issued, including the complete exercise of the underwriter's over-allotment option. By September 2013, the company raised a total net proceeds of approximately \$168.3 million by the closing of the offering including the complete exercise of the underwriter's over-allotment option.

In May 2013, the company raised \$150 million from a series F preferred investment round.

In May 2011, Intrexon raised \$100 million from a series E preferred financing round.

In May 2008, Intrexon raised \$25 million from a series C-2 financing. In June 2009, the company raised an additional \$10 million from the series C-2 financing round.

In May 2007, Intrexon raised \$25 million from a series C financing round.

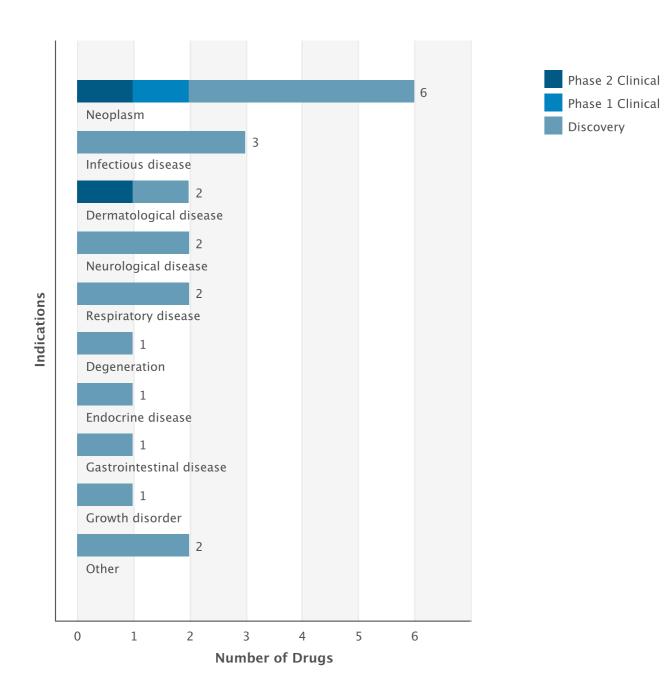


# PRODUCT PORTFOLIO SUMMARY

# **DRUGS**

# Drugs by Indication

Active Drugs by Indication Chart



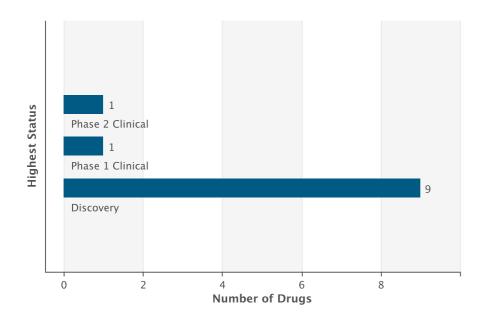


# Drugs by Indication Table

Indication	Active	Inactive	Total
Infectious disease	3	4	7
Neoplasm	6	0	6
Respiratory disease	2	3	5
Neurological disease	2	1	3
Dermatological disease	2	0	2
Degeneration	1	0	1
Immune disorder	1	0	1
Growth disorder	1	0	1
Gastrointestinal disease	1	0	1
Endocrine disease	1	0	1
Metabolic disorder	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
Phase 1 Clinical	1
Discovery	9
No Development Reported	5

#### **DEALS**

Deal Type	Prin	cipal	Par	tner	Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	13	0	0	0	13
Drug - Funding	2	0	0	0	2
Drug - Early Research/Development	2	0	0	0	2
Drug - Development/Commercialization License	1	0	0	0	1
Drug - Commercialization License	1	0	0	0	1
Drug - Development Services	1	0	0	0	1
Technology - Delivery/Formulation	0	0	2	0	2

### **CLINICAL TRIALS**

### Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	0	1

# Trials by Phase

Phase	Ongoing	All
Phase 1	0	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



### **PATENTS** \*

Indication	As Owner	As Third Party	Total
Cardiovascular disease	7	0	7
Endocrine disease	1	0	1
Gastrointestinal disease	1	0	1
Genitourinary disease	2	0	2
Hematological disease	2	0	2
Degeneration	1	0	1
Immune disorder	6	0	6
Musculoskeletal disease	2	0	2
Neoplasm	14	0	14
Ocular disease	1	0	1
Genetic disorder	3	0	3
Metabolic disorder	4	0	4
Neurological disease	4	0	4
Respiratory disease	4	0	4
Infectious disease	5	0	5
Injury	1	0	1
Toxicity and intoxication	1	0	1
Unidentified indication	3	0	3
Inflammatory disease	4	0	4
Dermatological disease	4	0	4
Surgical procedure	1	0	1

 $<sup>^{\</sup>star}$  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

#### Ad-RTS-IL-12

### **Ad-RTS-IL-12 SNAPSHOT**

Drug Name	Ad-RTS-IL-12
Key Synonyms	
Originator Company	ZIOPHARM Oncology Inc
Active Companies	ZIOPHARM Oncology Inc;Intrexon Corp
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Stage IV melanoma;Stage III melanoma;Glioblastoma;Metastatic breast cancer
Target-based Actions	IL-12 agonist
Other Actions	Anticancer;Immunostimulant;Adenovirus based gene therapy
Technologies	Oral formulation;Intratumoral formulation;Biological therapeutic;Virus recombinant
Last Change Date	08-Jan-2014

#### Ad-RTS-IL-12 DEVELOPMENT PROFILE

#### **SUMMARY**

ZIOPHARM Oncology and Intrexon are developing INXN-2001 plus INXN-1001 combination regimen, comprising intratumorally injected Ad-RTS-IL-12 (ZIN-ATI-001, IL-12 DNA; Ad-RTS-mIL-12), an adenovirus that inducibly expresses human IL-12 (hIL-12) when activated by an oral small-molecule agent (veledimex, INXN-1001, AD-1001), using the Intrexon's RheoSwitch and UltraVector technologies, for the potential treatment of cancer, .. In October 2012, phase II portion of phase I/II trial was initiated for stage III or IV melanoma. In December 2013, further positive interim results were presented. In January 2013, the trial would start in 2Q 2013, with data expected in 2014, if not by the end of 2013. In June 2013, a phase I trial in patients with recurring glioblastoma was expected to begin in the first half of 2014; in December 2013, recombinant DNA advisory committee (RAC) approved to initiate the study. Also in December 2013, interim data were presented from a phase II study in recurrent/metastatic breast cancer patients.

ZIOPHARM and Intrexon are also developing ZIN-CTI-001, INXN-2001 plus INXN-1001 combination regimen, for the potential treatment of solid tumors.

#### Ad-RTS-IL-12 DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	Development Status	Date
Intrexon Corp	Stage III melanoma	US	Phase 2 Clinical	14-Jul-2011



Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Stage IV melanoma	US	Phase 2 Clinical	14-Jul-2011
ZIOPHARM Oncology Inc	Metastatic breast cancer	US	Phase 2 Clinical	11-Mar-2013
ZIOPHARM Oncology Inc	Stage III melanoma	US	Phase 2 Clinical	14-Jul-2011
ZIOPHARM Oncology Inc	Stage IV melanoma	US	Phase 2 Clinical	14-Jul-2011
ZIOPHARM Oncology Inc	Glioblastoma	US	Discovery	30-Jun-2013

# **Ad-RTS-IL-12 CHEMICAL STRUCTURES**

CAS Registry Number:	Confidence Level:
1093130-72-3	2
	MINN NH
Name	Туре
veledimex	PINN; USAN

# **Ad-RTS-IL-12 DRUG NAMES**

Names	Туре
Ad-RTS-mIL-12	Research Code
IL-12 adenoviral gene therapy (intratumoral)/activator small-molecule (oral) (RheoSwitch/UltraVector, cancer), Intrexon/ZIOPHARM	
INXN-2001/INXN-1001 regimen	
IL-12 gene therapy (UltraVector, cancer), Ziopharm/Intrexon	
INXN-2001	Research Code
INXN-2001/AD-1001 regimen	
IL-12 DNA	
ZIN-ATI-001	Research Code
Ad-RTS-IL-12	Research Code



#### **Ad-RTS-IL-12 CLINICAL TRIALS**

### Trials by Phase and Condition Studied

Phase 4 Phase 3 Clinical Clinical				se 2 nical	Phase 1 Clinical		Phase Unspecified		Total		
On- going	All	On- going	All	On- going	All	On- going	All	On- going	All	On- going	All
Stage IV	melanoma	a									
0	0	0	0	0	0	0	2	0	0	0	2
Stage III	melanoma	ì									
0	0	0	0	0	0	0	2	0	0	0	2
Glioblast	oma										
0	0	0	0	0	0	1	1	0	0	1	1
Glioma											
0	0	0	0	0	0	1	1	0	0	1	1

# Total Trials by Phase and Status

Phase 4 Phase 3 Clinical Clinical			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 an	d Status									
0	0	0	0	0	0	1	3	0	0	1	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)

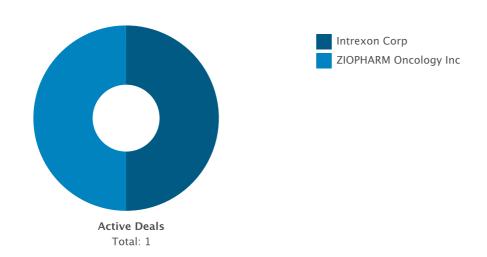
#### Phase 1 Clinical

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

### **Ad-RTS-IL-12 DEALS AND PATENTS**

DEALS

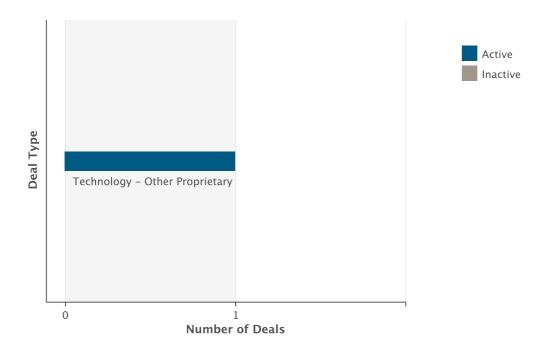
Deals by Parent Company Chart



# **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive	Par Active	Total	
ZIOPHARM Oncology Inc	0	0	1	0	1
Intrexon Corp	1	0	0	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Technology - Other Proprietary	1	0	1

# **SGX-94**

#### **SGX-94 SNAPSHOT**

Drug Name	SGX-94
Key Synonyms	
Originator Company	University of British Columbia
Active Companies	SciClone Pharmaceuticals Inc;Intrexon Corp;Soligenix Inc
Inactive Companies	University of British Columbia
Highest Status	Phase 2 Clinical
Active Indications	Radiation sickness;Oral mucositis;Melioidosis
Target-based Actions	Sequestosome 1 inhibitor
Other Actions	Antibacterial;Anti-inflammatory
Technologies	Biological therapeutic;Systemic formulation unspecified;Peptide
Last Change Date	17-Feb-2014

#### **SGX-94 DEVELOPMENT PROFILE**

#### **SUMMARY**

Soligenix, from an asset acquisition from University of British Columbia, is developing the active ingredient SGX-94 (structure shown), a short synthetic peptide innate defense regulator—that binds to regulatory protein p62 (sequestosome-1), for the potential treatment of oral mucositis (as SGX-942)—,.. Soligenix is also investigating SGX-94, in collaboration with Intrexon, for melioidosis (as SGX-943), and investigating the compound for acute radiation syndrome. In December 2013, a phase II trial for oral mucositis was initiated in the US. In February 2014, further preclinical trials in melioidosis were planned.

#### **SGX-94 DEVELOPMENT STATUS**

#### **CURRENT DEVELOPMENT STATUS**

CONNEINT DEVELOP	WENT STATES			
Company	Indication	Country	<b>Development Status</b>	Date
Soligenix Inc	Oral mucositis	US	Phase 2 Clinical	05-Dec-2013
Intrexon Corp	Melioidosis	US	Discovery	01-May-2013
SciClone Pharmaceuticals Inc	Oral mucositis	China	Discovery	08-Jul-2013
Soligenix Inc	Melioidosis	US	Discovery	18-Dec-2012
Soligenix Inc	Radiation sickness	US	Discovery	27-Mar-2013
University of British Columbia	Melioidosis	Canada	Discontinued	18-Dec-2012



Company	Indication	Country	<b>Development Status</b>	Date
University of British Columbia	Oral mucositis	Canada	Discontinued	18-Dec-2012

# **SGX-94 CHEMICAL STRUCTURES**

CAS Registry Number:	Confidence Level:
	4
HN NH <sub>2</sub> NH	N N N N H <sub>2</sub>
Name	Туре
SGX-94	Research Code

# **SGX-94 DRUG NAMES**

Names	Туре
SGX-942	
SGX-94	Research Code
SGX-943	Research Code

### **SGX-94 CLINICAL TRIALS**

# Trials by Phase and Condition Studied

	Phase 4 Phase 3 Clinical Clinical			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
Oral muc	cositis										
0	0	0	0	1	1	0	0	0	0	1	1



#### Total Trials by Phase and Status

	ase 4 Phase 3 Clinical			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 an	d Status									
0	0	0	0	1	1	0	0	0	0	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

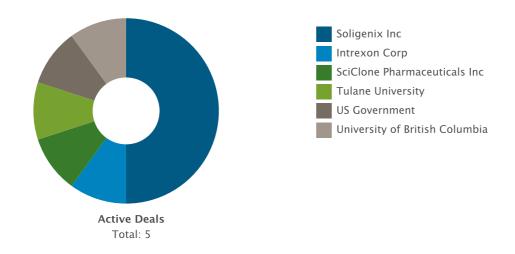
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

#### Phase 1 Clinical

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

# **SGX-94 DEALS AND PATENTS**

# DEALS Deals by Parent Company Chart

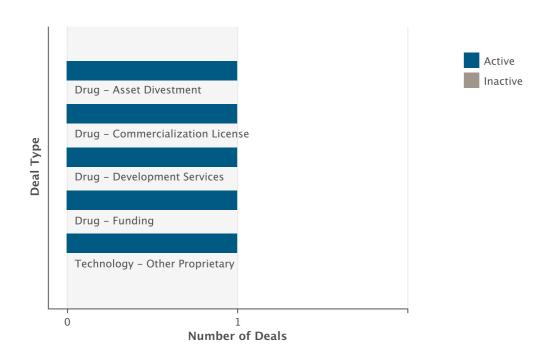




# **Deals by Parent Company Table**

Company Name	Principal Active Inactive		Partner Active Inactive		Total
Soligenix Inc	2	0	3	0	5
SciClone Pharmaceuticals Inc	0	0	1	0	1
Tulane University	1	0	0	0	1
Intrexon Corp	1	0	0	0	1
US Government	0	0	1	0	1
University of British Columbia	1	0	0	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Development Services	1	0	1
Drug - Funding	1	0	1
Drug - Asset Divestment	1	0	1
Drug - Commercialization License	1	0	1
Technology - Other Proprietary	1	0	1



#### DC-RTS-IL-12

#### **DC-RTS-IL-12 SNAPSHOT**

Drug Name	DC-RTS-IL-12
Key Synonyms	
Originator Company	Intrexon Corp
Active Companies	ZIOPHARM Oncology Inc;Intrexon Corp
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Solid tumor
Target-based Actions	IL-12 agonist
Other Actions	Genetically engineered autologous cell therapy;Anticancer;Immunostimulant
Technologies	Oral formulation;Intratumoral formulation;Biological therapeutic;Cell therapy
Last Change Date	18-Nov-2013

#### **DC-RTS-IL-12 DEVELOPMENT PROFILE**

#### **SUMMARY**

ZIOPHARM, in collaboration with Intrexon, is developing the INXN-3001 plus INXN-1001 combination regimen, DC-RTS-IL-12 (ZIN-CTI-001), comprising intratumorally injected autologous dendritic cells (INXN-3001, INcell-1001, IL-12 DNA) adenovirally transduced to inducibly express human IL-12 (hIL-12) when activated by an oral small-molecule agent (veledimex, INXN-1001, AD-1001), using the company's RheoSwitch technology, for the potential treatment of solid tumors,. By May 2009, positive data had been obtained from a phase Ia trial of the INXN-1001 component and at that time, phase Ib trial began; in June 2011, data from a phase I trial in stage III/IV melanoma were presented. In May 2012, ZIOPHARM planned to progress the program into phase II development.

#### **DC-RTS-IL-12 DEVELOPMENT STATUS**

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Solid tumor	US	Phase 1 Clinical	11-May-2009
ZIOPHARM Oncology Inc	Solid tumor	US	Phase 1 Clinical	06-Jan-2011

#### **DC-RTS-IL-12 CHEMICAL STRUCTURES**

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CAS Registry Number:	Confidence Level:
1093130-72-3	2
	N H
Name	Туре
veledimex	PINN; USAN

# **DC-RTS-IL-12 DRUG NAMES**

Names	Туре
INcell-1001/AD-1001	Research Code
ZIN-CTI-001	Research Code
INcell-1001 + AD-1001 (solid tumor, RheoSwitch), Intrexon	
INXN-3001 + INXN-1001, Intrexon	
INXN-3001	Research Code
IL-12 DNA	
AD-1001	Research Code
human IL-12 (hIL-12)-expressing autologous dendritic cells (adenovirally transduced, intratumorally injected) + activator small-molecule (oral) (solid tumor, RheoSwitch), Intrexon/ZIOPHARM	
DC-RTS-IL-12	Research Code
INXN-1001	Research Code
INcell-1001	Research Code

# **DC-RTS-IL-12 CLINICAL TRIALS**

Trials by Phase and Condition Studied

	se 4 nical		se 3 nical		se 2 nical		se 1 nical		ase ecified	То	tal
On- going	All	On- going	All								
Solid tum	Solid tumor										
0	0	0	0	0	0	0	1	0	0	0	1
Melanoma											
0	0	0	0	0	0	0	1	0	0	0	1

### Total Trials by Phase and Status

	se 4 nical		se 3 nical		se 2 nical		se 1 nical		ase ecified	То	tal
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	0	0	0	2	0	0	0	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)

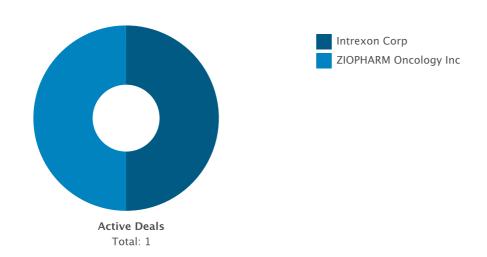
#### Phase 1 Clinical

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

# **DC-RTS-IL-12 DEALS AND PATENTS**

DEALS

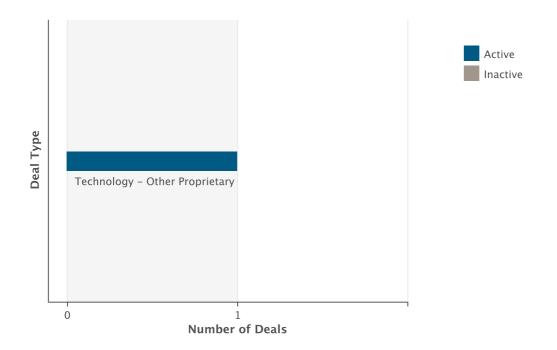
Deals by Parent Company Chart



# **Deals by Parent Company Table**

Company Name		i <b>cipal</b> Inactive	Par Active	Total	
Intrexon Corp	1	0	0	0	1
ZIOPHARM Oncology Inc	0	0	1	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Technology - Other Proprietary	1	0	1

# antibody drug conjugates (cancer), Intrexon

### antibody drug conjugates (cancer), Intrexon SNAPSHOT

Drug Name	antibody drug conjugates (cancer), Intrexon
Key Synonyms	
Originator Company	Intrexon Corp
Active Companies	Intrexon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Cancer
Target-based Actions	
Other Actions	Anticancer
Technologies	Biological therapeutic;Antibody conjugated
Last Change Date	28-Mar-2012

# antibody drug conjugates (cancer), Intrexon DEVELOPMENT PROFILE

### **SUMMARY**

Intrexon is investigating antibody drug conjugates for the potential treatment of cancer. In February 2012, the program was listed as being in lead series; at that time, the company was seeking to outlicense the program.

### antibody drug conjugates (cancer), Intrexon DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Cancer	US	Discovery	26-Feb-2012

### antibody drug conjugates (cancer), Intrexon DRUG NAMES

Names Type	
antibody drug conjugates (cancer), Intrexon	

# rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon

### rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon SNAPSHOT

Drug Name	rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon
Key Synonyms	
Originator Company	Intrexon Corp
Active Companies	Intrexon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Respiratory disease; Diabetes mellitus
Target-based Actions	Alpha 1 antitrypsin stimulator
Other Actions	Hypoglycemic agent
Technologies	Biological therapeutic;Subcutaneous formulation;Protein recombinant
Last Change Date	28-Mar-2012

### rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

Intrexon is investigating recombinant human alpha 1-antitrypsin (rHuA1AT), incorporating Halozyme's recombinant human hyaluronidase (rHuPH20)-based Enhanze technology, for the potential subcutaneous injectable treatment of diabetes, and respiratory diseases including cystic fibrosis, pulmonary disease and COPD caused by A1AT deficiency. In June 2011, the program was undergoing the scale-up phase of process development. In February 2012, the drug was listed as being in preclinical development; at that time, the company was seeking to outlicense the drug.

### rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

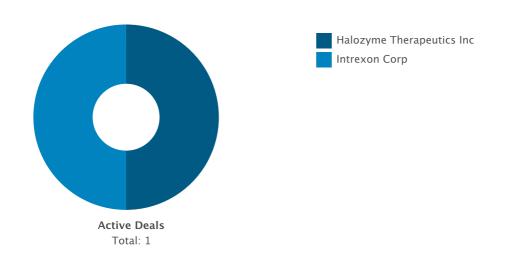
Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Diabetes mellitus	US	Discovery	26-Feb-2012
Intrexon Corp	Respiratory disease	US	Discovery	26-Feb-2012

### rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon DRUG NAMES

Names	Туре
recombinant human alpha 1-antitrypsin (sc Enhanze, A1AT deficiency), Intrexon	
rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon	
ITXN-A1AT	Research Code

# rHuA1AT (sc Enhanze, A1AT deficiency), Intrexon DEALS AND PATENTS

# DEALS Deals by Parent Company Chart

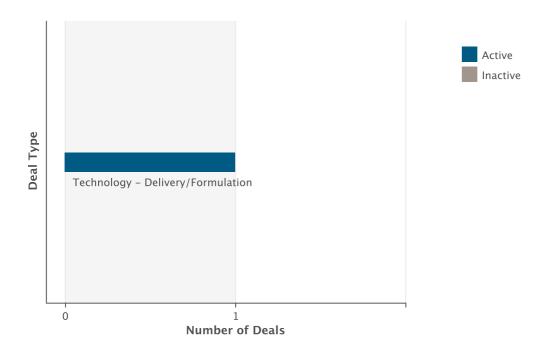


# **Deals by Parent Company Table**

Company Name		cipal Inactive		tner Inactive	Total
Intrexon Corp	0	0	1	0	1
Halozyme Therapeutics Inc	1	0	0	0	1



# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Technology - Delivery/Formulation	1	0	1

# fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon

fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon SNAPSHOT

fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon
Immunologix
Intrexon Corp
Immunologix
Discovery
Cancer;Infectious disease;Autoimmune disease
Anticancer monoclonal antibody;Antimicrobial;Immunomodulator
Monoclonal antibody human;Biological therapeutic;Parenteral formulation unspecified
15-Jun-2012

# fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

Intrexon, following its acquisition of Immunologix, is investigating fully human mAbs for the potential treatment of cancer, infectious disease including west nile and dengue fever, and autoimmune disease including inflammation. In February 2012, the program was listed as being in lead series and at that time, the company was seeking to outlicense the program.

# fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon DEVELOPMENT STATUS

## **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Autoimmune disease	US	Discovery	26-Feb-2012
Intrexon Corp	Cancer	US	Discovery	26-Feb-2012
Intrexon Corp	Infectious disease	US	Discovery	26-Feb-2012

# fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon DRUG NAMES

Names	Туре
fully human monoclonal antibodies (cancer/infection/autoimmune disease), Intrexon	
fully human monoclonal antibodies (cancer/infection), Immunologix	



# fully human mAbs (Alzheimers disease), Intrexon

### fully human mAbs (Alzheimers disease), Intrexon SNAPSHOT

Drug Name	fully human mAbs (Alzheimers disease), Intrexon
Key Synonyms	
Originator Company	Immunologix
Active Companies	Intrexon Corp
Inactive Companies	Immunologix
Highest Status	Discovery
Active Indications	Alzheimers disease
Target-based Actions	
Other Actions	Neuroprotectant
Technologies	Monoclonal antibody human;Biological therapeutic;Parenteral formulation unspecified
Last Change Date	12-Jun-2012

### fully human mAbs (Alzheimers disease), Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

Intrexon, following its acquisition of Immunologix, is investigating fully human mAbs targeting amyloid plaques, for the potential treatment of Alzheimer's disease. By June 2011, the target had been validated.

### fully human mAbs (Alzheimers disease), Intrexon DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Alzheimers disease	US	Discovery	24-Oct-2011

#### fully human mAbs (Alzheimers disease), Intrexon DRUG NAMES

Names	Туре
fully human monoclonal antibodies (Alzheimers disease), Immunologix	
fully human mAbs (Alzheimers disease), Intrexon	



# **SGX-101**

#### **SGX-101 SNAPSHOT**

Drug Name	SGX-101
Key Synonyms	
Originator Company	Soligenix Inc
Active Companies	Soligenix Inc;Intrexon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Melioidosis
Target-based Actions	
Other Actions	Unspecified drug target;Antibacterial
Technologies	Monoclonal antibody human;Biological therapeutic;Parenteral formulation unspecified
Last Change Date	18-Oct-2013

#### **SGX-101 DEVELOPMENT PROFILE**

### **SUMMARY**

Soligenix and Intrexon are investigating human mAbs for the potential treatment of melioidosis. In May 2013, development was ongoing.

#### **SGX-101 DEVELOPMENT STATUS**

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Melioidosis	US	Discovery	01-May-2013
Soligenix Inc	Melioidosis	US	Discovery	01-May-2013

### **SGX-101 DRUG NAMES**

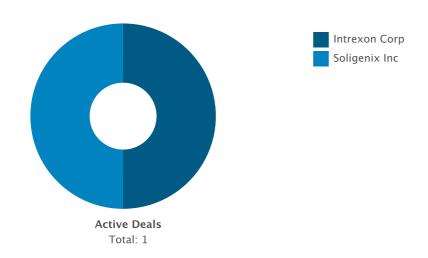
Names	Туре
human mAbs (melioidosis), Soligenix/Intrexon	
SGX-101	Research Code



# **SGX-101 DEALS AND PATENTS**

DEALS

Deals by Parent Company Chart



# **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive		tner Inactive	Total
Soligenix Inc	0	0	1	0	1
Intrexon Corp	1	0	0	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Technology - Other Proprietary	1	0	1

# SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon

# SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon SNAPSHOT

Drug Name	SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon
Key Synonyms	
Originator Company	Intrexon Corp
Active Companies	Intrexon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Spinal muscular atrophy
Target-based Actions	SMN1 gene modulator
Other Actions	Stem cell modulator
Technologies	Parenteral formulation unspecified;Autologous stem cell therapy
Last Change Date	30-Oct-2013

# SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

AveXis, a spin-off from BioLife Cell Bank, in collaboration with Intrexon, is investigating a SMN1-based gene replacement stem cell therapy for the potential treatment of spinal muscular atrophy (SMA). In August 2012, development was ongoing. At that time, phase I clinical trials were planned ,.

# SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Spinal muscular atrophy	US	Discovery	02-Aug-2012

#### SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon DRUG NAMES

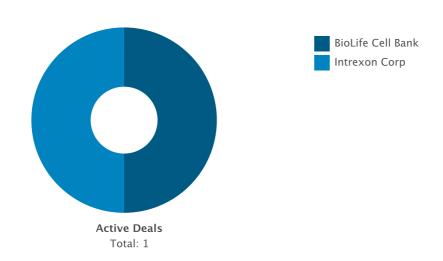
Names	Туре
SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon	



# SMN1-based gene replacement stem cell therapy (spinal muscular atrophy), Intrexon DEALS AND PATENTS

#### **DEALS**

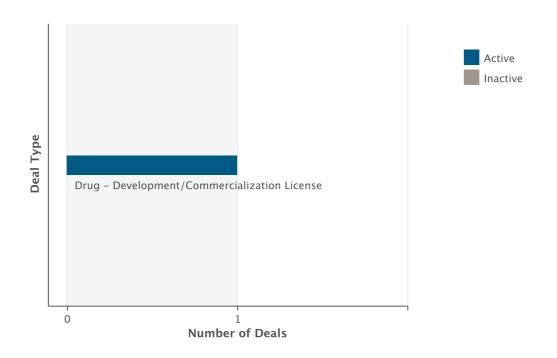
### **Deals by Parent Company Chart**



# **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive		tner Inactive	Total
BioLife Cell Bank	0	0	1	0	1
Intrexon Corp	1	0	0	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	0	1



# interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon

### interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon SNAPSHOT

Drug Name	interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon
Key Synonyms	
Originator Company	ZIOPHARM Oncology Inc
Active Companies	Intrexon Corp;ZIOPHARM Oncology Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Melanoma
Target-based Actions	IFNA gene modulator
Other Actions	Anticancer;Plasmid based gene therapy;Immunostimulant
Technologies	DNA technology;Intramuscular formulation;Biological therapeutic
Last Change Date	19-Mar-2013

# interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

ZIOPHARM Oncology, in collaboration with Intrexon, is investigating pRTS-IFNalpha, an interferon-alpha plasmid transgene therapy, created using RheoSwitch and UltraVector technologies, for the potential treatment of melanoma.

# interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon DEVELOPMENT STATUS

#### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Melanoma	US	Discovery	07-Nov-2012
ZIOPHARM Oncology Inc	Melanoma	US	Discovery	07-Nov-2012

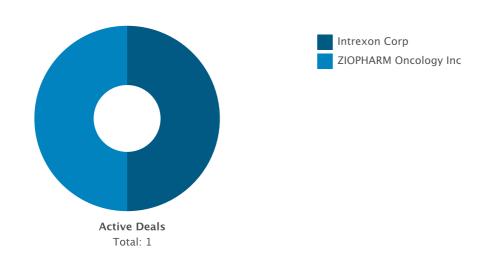
# interferon-alpha plasmid transgene therapy (melanoma), ZIOPHARM/Intrexon DRUG NAMES

Names	Туре
pRTS-IFNalpha	
interferon-alpha plasmid transgene therapy	
(melanoma), ZIOPHARM/Intrexon	



DEALS

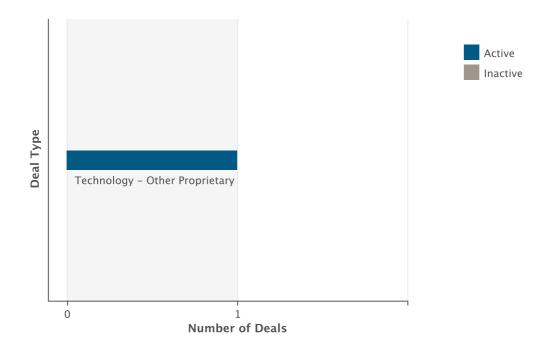
Deals by Parent Company Chart



# **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive		tner Inactive	Total
Intrexon Corp	1	0	0	0	1
ZIOPHARM Oncology Inc	0	0	1	0	1

# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Technology - Other Proprietary	1	0	1

# transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon

transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon SNAPSHOT

Drug Name	transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon
Key Synonyms	
Originator Company	ZIOPHARM Oncology Inc
Active Companies	Mesoblast Ltd;ZIOPHARM Oncology Inc;Intrexon Corp
Inactive Companies	
Highest Status	Discovery
Active Indications	Lung tumor
Target-based Actions	
Other Actions	Anticancer;Gene therapy
Technologies	Biological therapeutic;Mesenchymal stem cell therapy
Last Change Date	02-Nov-2013

transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon DEVELOPMENT PROFILE

#### **SUMMARY**

Mesoblast, Intrexon and ZIOPHARM Oncology are investigating transgene enabled cell-based therapeutics, which combine Mesoblast's Mesenchymal Lineage Cells (MLCs) with Intrexon's RheoSwitch Therapeutic System (RTS), for the potential treatment of lung and other cancers. In October 2013, the companies would form a joint venture to develop therapeutic candidates if their feasibility studies in lung cancer were successful.

transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon DEVELOPMENT STATUS

### **CURRENT DEVELOPMENT STATUS**

Company	Indication	Country	<b>Development Status</b>	Date
Intrexon Corp	Lung tumor	US	Discovery	23-Oct-2013
Mesoblast Ltd	Lung tumor	Australia	Discovery	23-Oct-2013
ZIOPHARM Oncology Inc	Lung tumor	US	Discovery	23-Oct-2013



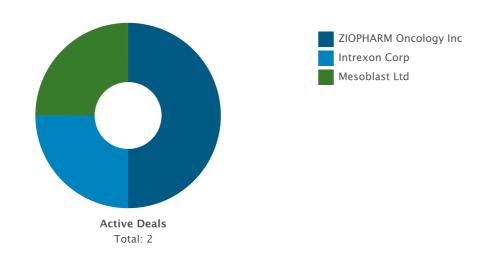
transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon DRUG NAMES

Names	Туре
transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon	

transgene enabled cell-based therapeutics (Mesenchymal Lineage Cells/ RheoSwitch, lung cancer), ZIOPHARM Oncology/ Mesoblast/ Intrexon DEALS AND PATENTS

**DEALS** 

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

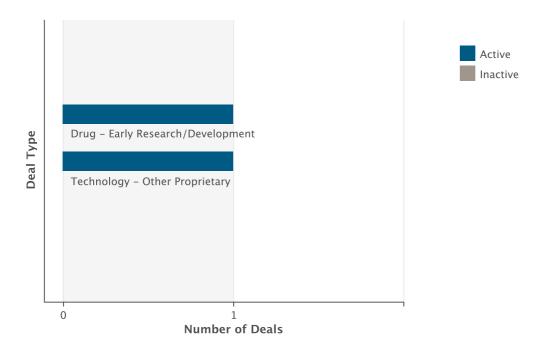


# **Deals by Parent Company Table**

Company Name		<b>cipal</b> Inactive		tner Inactive	Total
ZIOPHARM Oncology Inc	0	0	2	0	2
Intrexon Corp	1	0	0	0	1
Mesoblast Ltd	1	0	0	0	1



# **Deals by Type Chart**



# **Deals by Type Table**

Deal Type	Active	Inactive	Total
Drug - Early Research/Development	1	0	1
Technology - Other Proprietary	1	0	1



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