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Bluebird Bio

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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ABOUT CORTELLIS COMPANY DETAILED PIPELINE 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.

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

COMPANY OVERVIEW

Company Name	Bluebird Bio
Parent Company Name	Bluebird Bio
Website	http://www.bluebirdbio.com/
Country	US
Number of Drugs in Active Development	3
Number of Inactive Drugs	4
Number of Patents as Owner	13
Number of Patents as Third Party	0
Number of Deals	12
Key Indications	Beta thalassemia,Sickle cell anemia,Adrenoleukodystrophy,Hematological neoplasm,Solid tumor,Adrenomyeloneuropathy,Hemoglobinopathy,Alpha thalassemia,Hematological disease,Stem cell transplantation,Thalassemia intermedia,Thalassemia major
Key Target-based Actions	HBB gene stimulator,ATP binding cassette transporter D1 stimulator,Hemoglobin beta subunit stimulator,Caspase stimulator,Erythropoietin ligand,Hemoglobin delta subunit stimulator,Hemoglobin gamma subunit stimulator,PPAR alpha agonist,PPAR delta agonist,PPAR gamma agonist,TK gene stimulator,Thymidine kinase
Key Technologies	Biological therapeutic,Gene transfer system viral,Haematopoietic stem cell therapy,Autologous stem cell therapy,Intravenous formulation,Peripheral blood stem cell therapy,T-lymphocyte,Tumor antigen therapeutic,Virus recombinant,Adult stem cell therapy,Cell culture technique,Cell therapy,Embryonic stem cell therapy,Gene transfer

COMPANY PROFILE

SUMMARY

bluebird bio (formerly Genetix Pharmaceuticals Inc), based in Cambridge, MA, was established in 1992. It is a private company that develops genetically engineered autologous cell therapies for the treatment of blood disorders and neural degenerative disorders. In September 2010, Genetix changed its name to bluebird bio.

LICENSING AGREEMENTS

In January 1999, Genetix signed an agreement with Cordis Corp to develop gene therapy products to treat heart disease.

In July 2001, it was reported that a collaboration between Genetix and Theratechnologies Inc was still ongoing. The two companies entered the partnership in April 1998 when Genetic was granted an exclusive license to incorporate Theratechnologies' vpr technology into its therapeutic gene delivery system.

FINANCIAL

In December 2013, bluebird was added to the NASDAQ Biotechnology Index.

In September 2013, bluebird was added to the Russell 2000, Russell 3000 and Russell Global Indexes.

In June 2013, bluebird announced the pricing of its initial public offering of 5,941,176 shares of common stock at a public offering price of $17.00 per share, before underwriting discounts. The underwriters had a 30 day option to purchase up to an additional 891,176 shares. Trading was expected to begin on Wednesday, June 19, 2013 under the trading symbol "BLUE"; later that month, the offering of 6,832,352 common-stock shares was closed, with full exercise of underwriters' option.

In July 2012, bluebird raised $60 million from a series D financing round.

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In March 2010, Genetix raised $35 million from a series B financing round. In April 2011, bluebird bio secured an additional $30 million in a series C financing round.

In October 2004, Genetix raised $12 million in a private equity investment round to fund a phase I/II trial of LentiGlobin.

R&D GRANTS

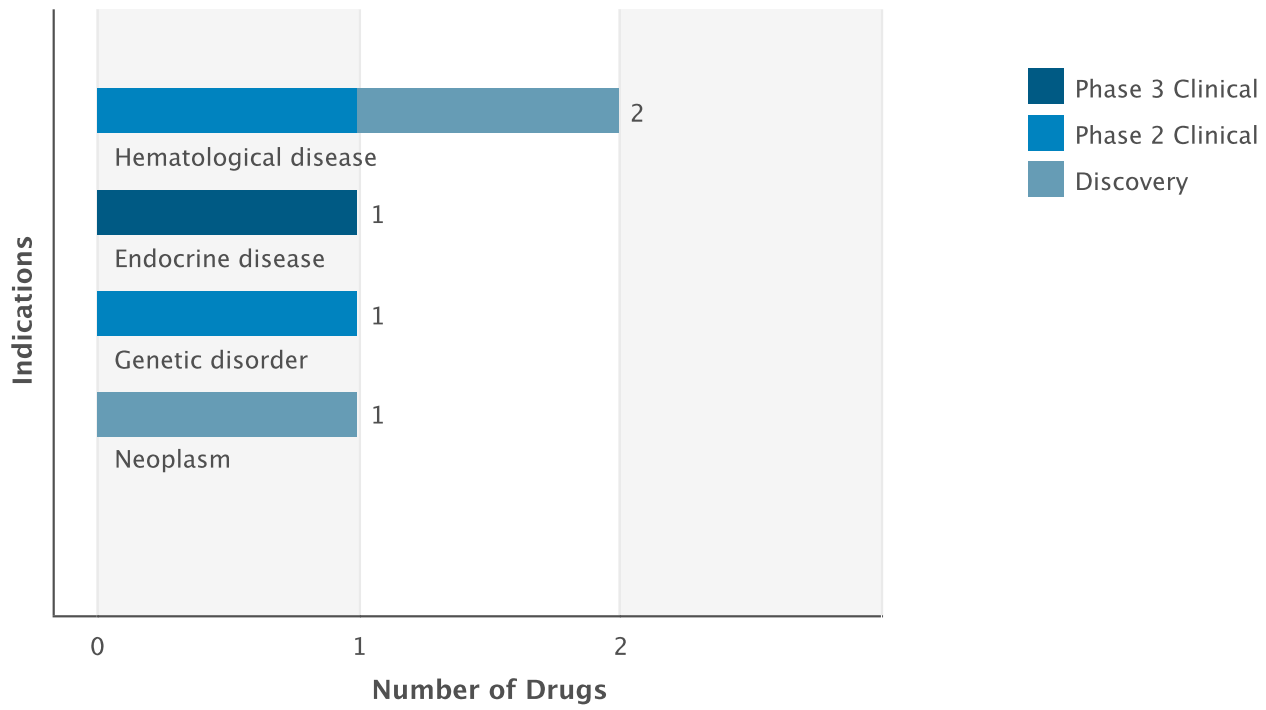
In August 1998, a Phase I SBIR grant was awarded by the NCI to support preclinical development of antiangiogenic gene therapy treatments for cancer.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



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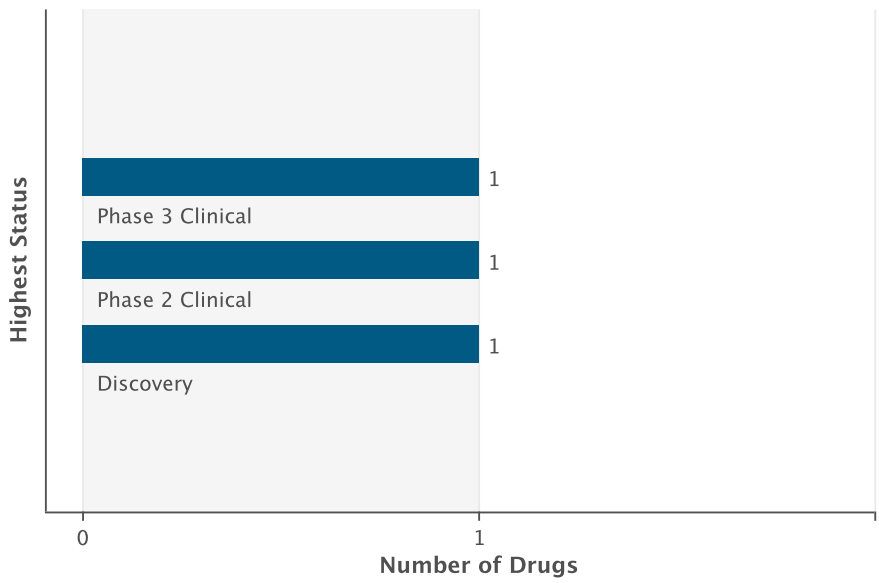
Drugs by Indication Table

Indication	Active	Inactive	Total
Neoplasm	1	3	4
Endocrine disease	1	1	2
Hematological disease	2	0	2
Neurological disease	0	1	1
Musculoskeletal disease	0	1	1
Gynecology and obstetrics	0	1	1
Ocular disease	0	1	1
Genitourinary disease	0	1	1
Cardiovascular disease	0	1	1
Genetic disorder	1	0	1
Inflammatory disease	0	1	1

Drugs by Highest Status

Active Drugs by Highest Status Chart



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Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 3 Clinical	1
Phase 2 Clinical	1
Discovery	1
No Development Reported	4

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	2	0	2
Patent - Exclusive Rights	0	0	1	0	1
Drug - Funding	4	0	0	0	4
Drug - Early Research/Development	1	0	1	0	2
Drug - Development/Commercialization License	1	0	1	0	2
Drug - Manufacturing/Supply	1	0	0	0	1

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Hematological disease	1	3
Genetic disorder	1	3
Endocrine disease	1	2
Neoplasm	0	2
Neurological disease	0	1
Gynecology and obstetrics	0	1
Genitourinary disease	0	1

Trials by Phase

Phase	Ongoing	All
Phase 2	1	1
Phase 1	1	5

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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	6	0	6
Endocrine disease	4	0	4
Genitourinary disease	1	0	1
Hematological disease	8	0	8
Immune disorder	2	0	2
Musculoskeletal disease	1	0	1
Neoplasm	3	0	3
Genetic disorder	7	0	7
Neurological disease	1	0	1
Infectious disease	1	0	1
Inflammatory disease	1	0	1
Gynecology and obstetrics	1	0	1
Dermatological disease	1	0	1
Surgical procedure	1	0	1

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

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PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

Lenti-D

Lenti-D SNAPSHOT

Drug Name	Lenti-D
Key Synonyms	Lenti-D
Originator Company	INSERM
Active Companies	INSERM;Bluebird Bio
Inactive Companies	
Highest Status	Phase 3 Clinical
Active Indications	Adrenoleukodystrophy
Target-based Actions	
Other Actions	Genetically engineered autologous cell therapy;Retrovirus based gene therapy
Technologies	Virus recombinant;Biological therapeutic;Parenteral formulation unspecified;Autologous stem cell therapy;Peripheral blood stem cell therapy;Haematopoietic stem cell therapy
Last Change Date	16-Nov-2013

Lenti-D DEVELOPMENT PROFILE

SUMMARY

bluebird bio (formerly Genetix Pharmaceuticals), in collaboration with INSERM, is developing Lenti-D, a stem cell therapy using lentiviral vectors carrying human ABCD1 cDNA encoding the functional adrenoleukodystrophy protein (ALDP) to transduce patient hematopoietic stem cells, for the potential treatment of adult and childhood cerebral adrenoleukodystrophy (CCALD),. In August 2013, a phase II/III trial was initiated in the US. In November 2009, launch was expected in 2014.

Lenti-D DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Bluebird Bio	Adrenoleukodystrophy	US	Phase 3 Clinical	15-Aug-2013
Bluebird Bio	Adrenoleukodystrophy	France	Phase 2 Clinical	13-Jan-2010
INSERM	Adrenoleukodystrophy	France	Phase 2 Clinical	13-Jan-2010

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Lenti-D DRUG NAMES

Names	Type
CCALD program (cerebral adrenoleukodystrophy), INSERM/bluebird	
adrenoleukodystrophy gene therapy, INSERM/Genetix	
genetically-modified stem cell therapy (adrenoleukodystrophy), INSERM/bluebird	
Lenti-D	Trade Name

Lenti-D CLINICAL TRIALS

Trials by Phase and Condition Studied

Phase 4 Clinical		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
Adrenoleukodystrophy											
0	0	0	0	1	1	0	1	0	0	1	2

Total Trials by Phase and Status

Phase 4 Clinical		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 and Status											
0	0	0	0	1	1	0	1	0	0	1	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

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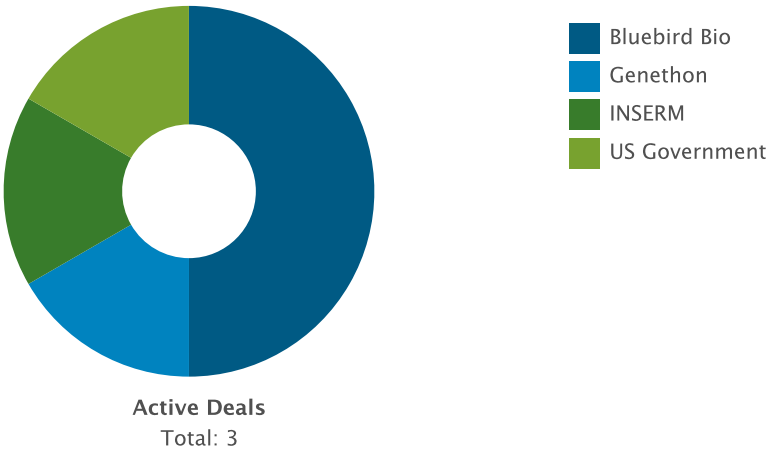
Lenti-D DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Bluebird Bio	2	0	1	0	3
Genethon	0	0	1	0	1
INSERM	1	0	0	0	1
US Government	0	0	1	0	1

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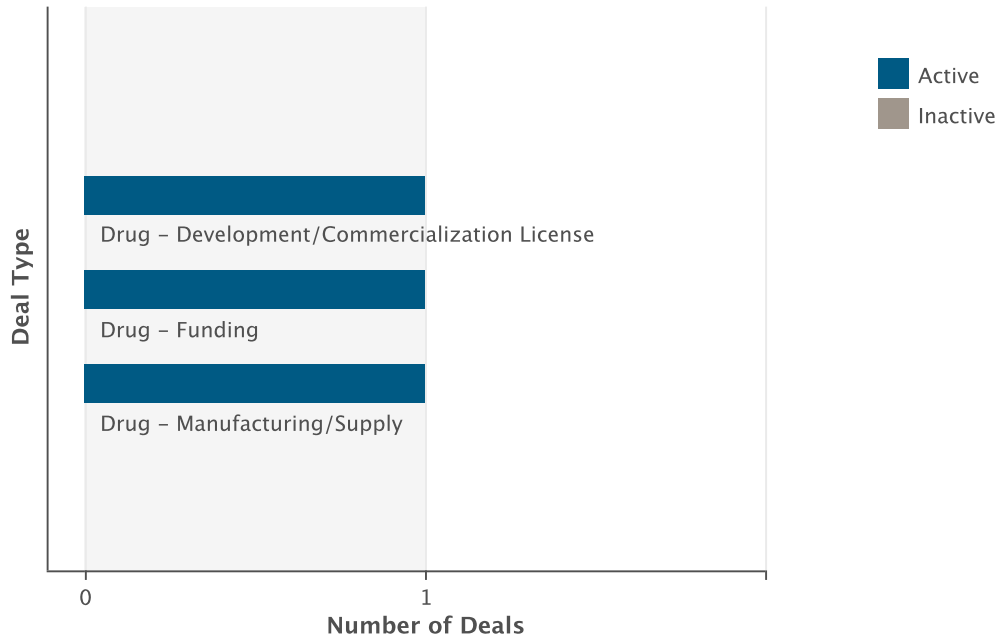
Deals by Type Chart



Deals by Type Table

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

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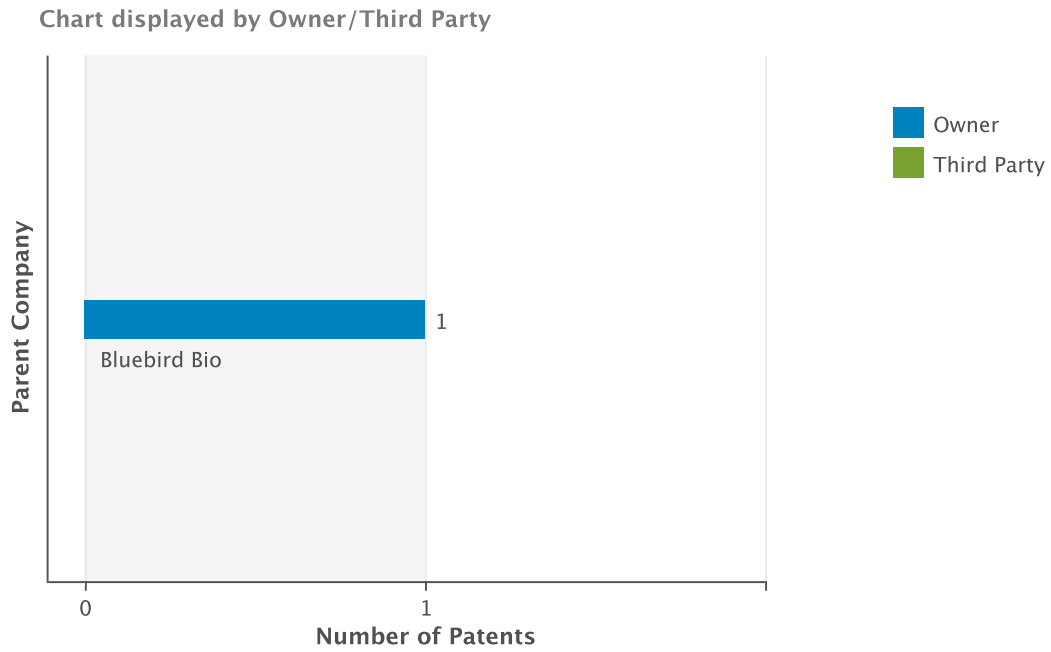
PATENTS

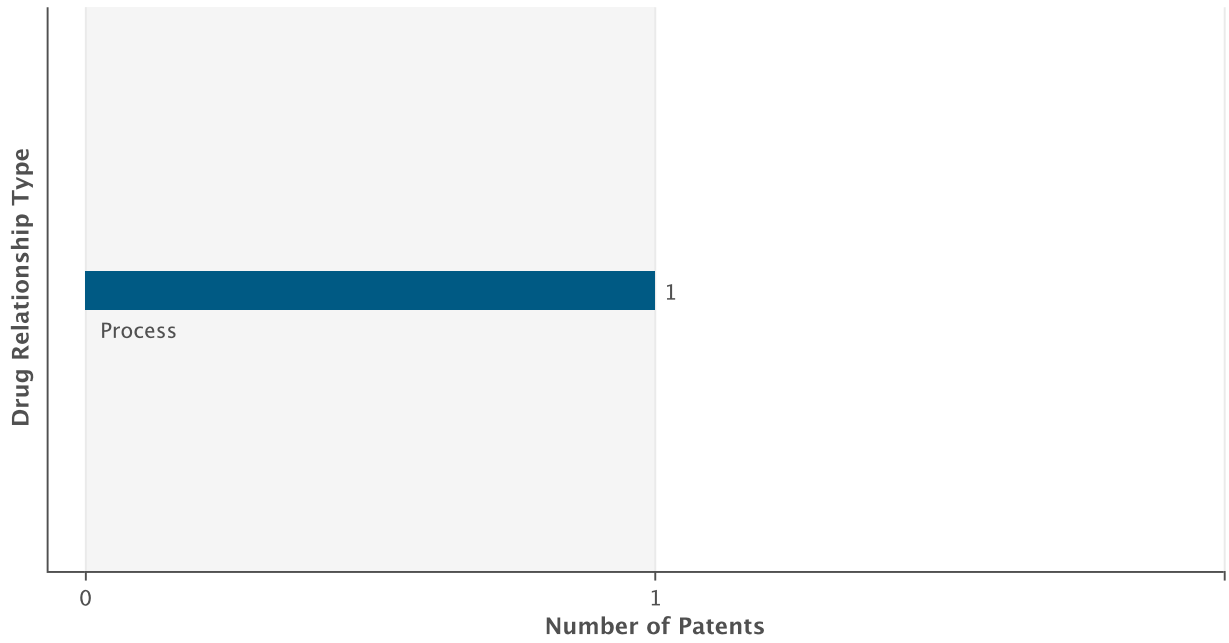
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Bluebird Bio	1	0	1

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
Process	1

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LentiGlobin

LentiGlobin SNAPSHOT

Drug Name	LentiGlobin
Key Synonyms	LentiGlobin
Originator Company	Bluebird Bio
Active Companies	Bluebird Bio
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Beta thalassemia;Sickle cell anemia
Target-based Actions	HBB gene stimulator
Other Actions	Genetically engineered autologous cell therapy;Retrovirus based gene therapy
Technologies	Virus recombinant;Intravenous formulation;Biological therapeutic;Autologous stem cell therapy;Peripheral blood stem cell therapy;Haematopoietic stem cell therapy
Last Change Date	31-Dec-2013

LentiGlobin DEVELOPMENT PROFILE

SUMMARY

bluebird bio (formerly Genetix Pharmaceuticals) is developing LentiGlobin, a gene therapy which utilizes its second-generation lentiviral vector-based gene transfer system LentiPak to transduce autologous hematopoietic stem cells with a functional human beta-globin gene, for the potential treatment of sickle cell disease (SCD) and beta-thalassemia. By January 2010, a phase I/II trial had been initiated in beta-thalassemia ; in February 2012, the trial was nearing completion. In August 2013, a US phase I/II trial for beta-thalassemia was initiated. By November 2006, a phase I trial was reported to be underway for SCD. By August 2012, the therapy was still listed as being in phase I for SCD and the company expected to enroll SCD patients into a phase I/II trial during 2012. In November 2009, drug launch was expected in 2014 or 2015.

LentiGlobin DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Bluebird Bio	Beta thalassemia	France	Phase 2 Clinical	13-Jan-2010
Bluebird Bio	Beta thalassemia	US	Phase 2 Clinical	29-Aug-2013
Bluebird Bio	Sickle cell anemia	US	Phase 1 Clinical	09-Nov-2006

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LentiGlobin DRUG NAMES

Names	Type
beta-globin-transduced autologous stem cell therapy (LentiPak, sickle cell disease/beta-thalassemia), bluebird bio	
beta-globin transduced autologous stem cell therapy (LentiPak, sickle cell disease/beta-thalassemia), Genetix	
LentiGlobin BB305	Research Code
LentiGlobin	Trade Name

LentiGlobin CLINICAL TRIALS

Trials by Phase and Condition Studied

Phase 4 Clinical		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
Beta thalassemia											
0	0	0	0	0	0	1	3	0	0	1	3
Sickle cell anemia											
0	0	0	0	0	0	0	1	0	0	0	1

Total Trials by Phase and Status

Phase 4 Clinical		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 and 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 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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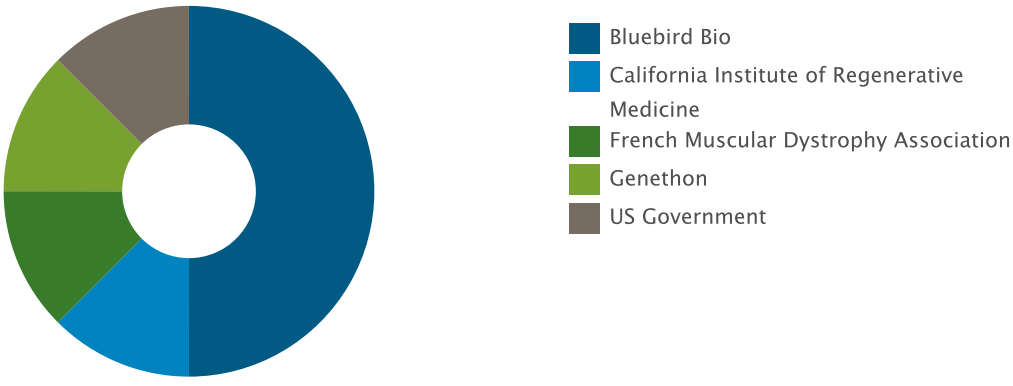
LentiGlobin DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 4

Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Bluebird Bio	4	0	0	0	4
Genethon	0	0	1	0	1
French Muscular Dystrophy Association	0	0	1	0	1
US Government	0	0	1	0	1
California Institute of Regenerative Medicine	0	0	1	0	1

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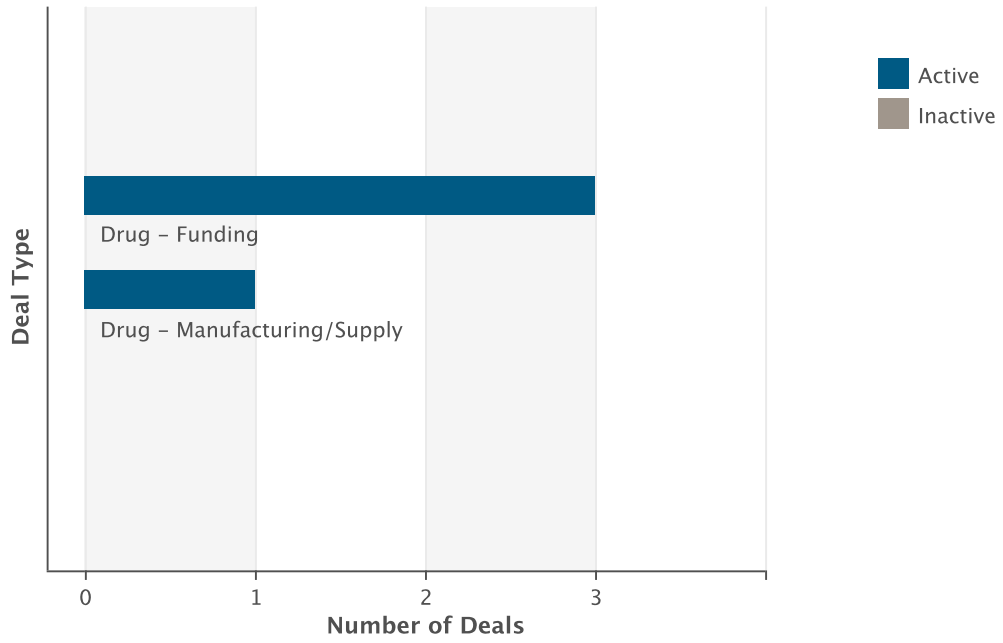
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	3	0	3
Drug - Manufacturing/Supply	1	0	1

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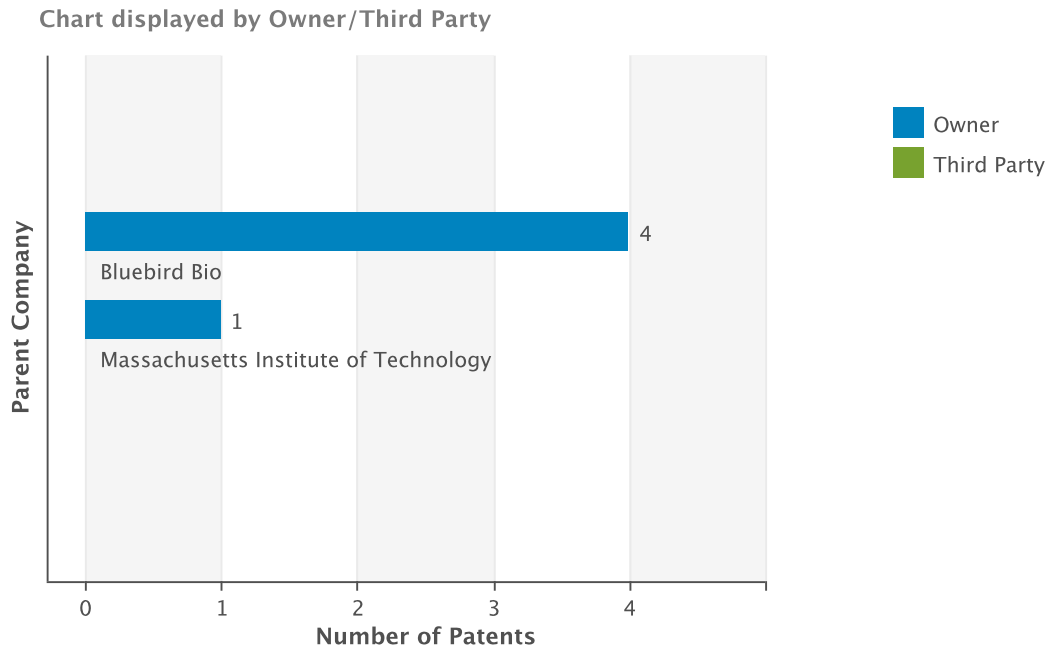
PATENTS

Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Bluebird Bio	4	0	4
Massachusetts Institute of Technology	1	0	1

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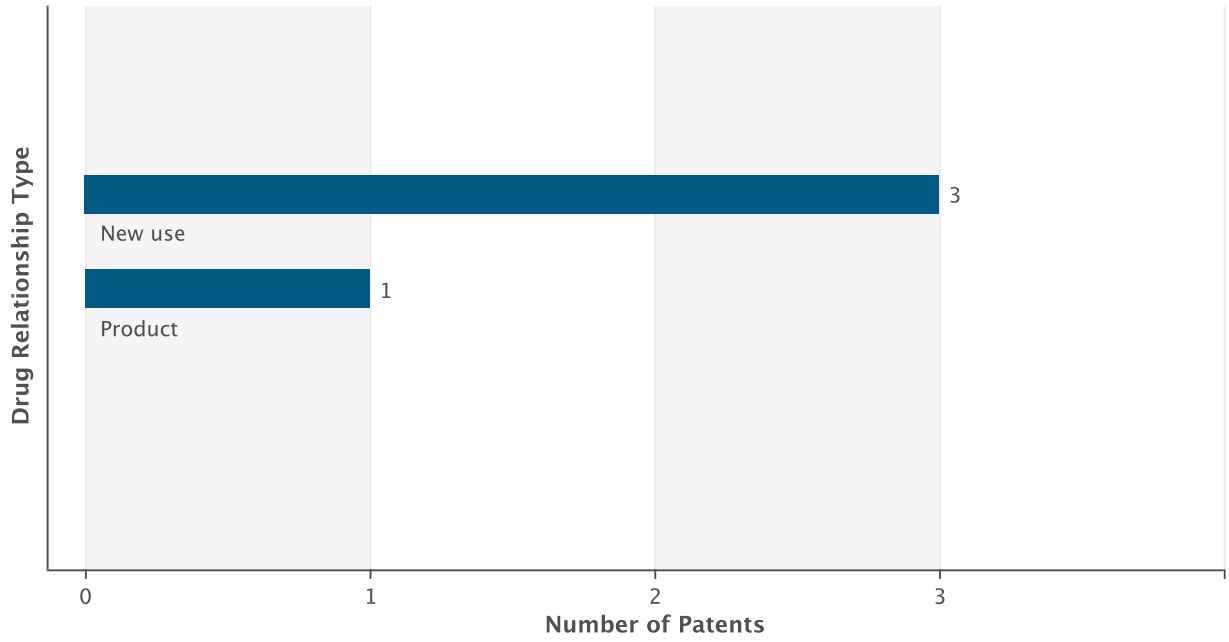
Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	3
Product	1

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CAR T-cells (cancer), bluebird bio

CAR T-cells (cancer), bluebird bio SNAPSHOT

Drug Name	CAR T-cells (cancer), bluebird bio
Key Synonyms	
Originator Company	Bluebird Bio
Active Companies	Celgene Corp;Bluebird Bio
Inactive Companies	
Highest Status	Discovery
Active Indications	Hematological neoplasm;Solid tumor
Target-based Actions	
Other Actions	Gene therapy;Anticancer
Technologies	Tumor antigen therapeutic;Biological therapeutic;T-lymphocyte
Last Change Date	04-Dec-2013

CAR T-cells (cancer), bluebird bio DEVELOPMENT PROFILE

SUMMARY

bluebird bio in collabaration with Celgene is investigating CAR (chimeric antigen receptor) T-cells, genetically modified to target and destroy cancer cells, for the potential treatment of solid tumors and hematological malignancies,. In March 2013, development was ongoing.

CAR T-cells (cancer), bluebird bio DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Bluebird Bio	Hematological neoplasm	US	Discovery	27-Mar-2013
Bluebird Bio	Solid tumor	US	Discovery	27-Mar-2013
Celgene Corp	Hematological neoplasm	US	Discovery	21-Mar-2013
Celgene Corp	Solid tumor	US	Discovery	21-Mar-2013

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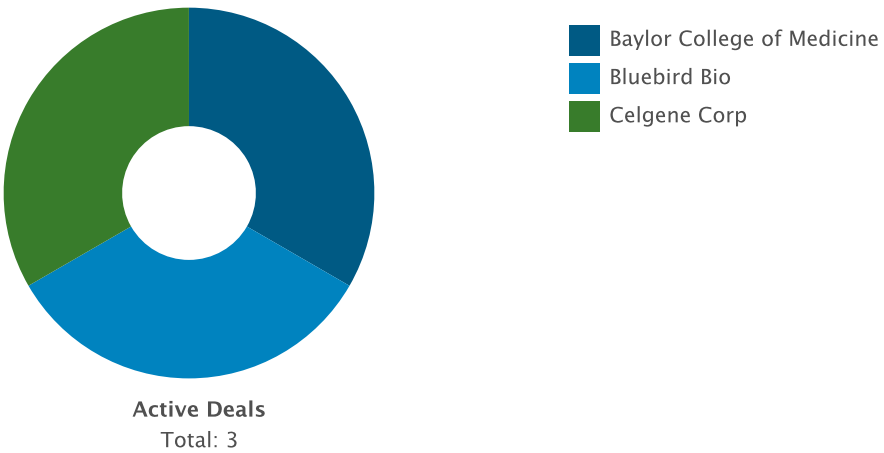
CAR T-cells (cancer), bluebird bio DRUG NAMES

Names	Type
chimeric antigen receptor T-cells (cancer), bluebird bio	
CAR T-cells (cancer), bluebird bio	

CAR T-cells (cancer), bluebird bio DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Celgene Corp	0	0	2	0	2
Baylor College of Medicine	2	0	0	0	2
Bluebird Bio	1	0	1	0	2

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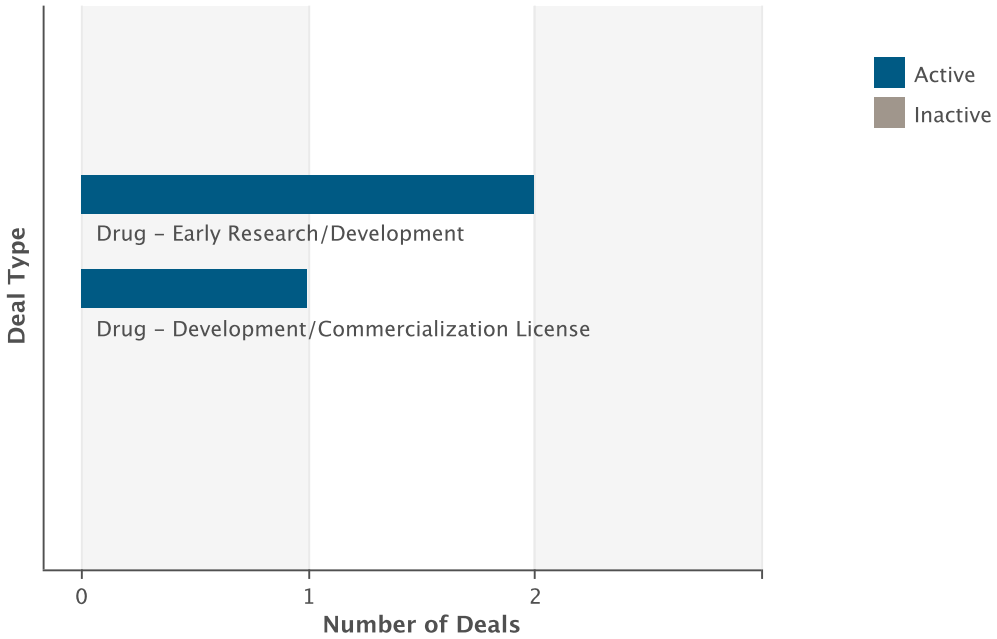
Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Early Research/Development	2	0	2
Drug - Development/Commercialization License	1	0	1

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