

# **Bluebird Bio**

# **COMPANY AND PIPELINE OVERVIEW REPORT**

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

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# ABOUT COMPANY AND PIPELINE OVERVIEW 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 I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

# **Number of Inactive Drugs**

Number of drugs associated with the company or subsidiary that are currently classified as inactive, i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

#### **Number of Patents as Owner**

Number of patents associated with the company where the company is listed as owner; i.e. the relationship type (or way the patent refers to the company) is: Patent Assignee/Owner, Patent owner (not assignee), Licensee for development and marketing, Licensee – marketing only (Distributor), Patent assignee of family member, Inferred assignee.

### **Number of Patents as Third Party**

Number of patents associated with the company where the company is listed as third party; i.e. the relationship type (or way the patent refers to the company) is: Patent assignee (not owner), Ex-Licensee for development and marketing, Ex-Licensee marketing only (Distributor), Customer of technology, Ex-Customer of technology, Patent opponent or infringer, Affiliate organization of inventor, Owner of underlying technology.

### Patents summary table

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

#### **Number of Deals**

A count of deals where the company or one of its subsidiaries is the primary company.

### **Key Indications**

Displays top ten key indications for the company and its subsidiaries based on frequency (indications occurring with high and identical frequency are always included, and this may result in more than ten Key Indications being listed). Includes both indications associated with patents where the company is patent owner and indications associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

### **Key Target-based Actions**

Displays top ten key target-based actions for the company and its subsidiaries based on frequency (actions occurring with high and identical frequency are always included, and this may result in more than ten Key Target-based Actions being listed). Includes both target-based actions associated with patents where the company patent owner and target-based actions associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended. A target-based action is one that is associated with a target.

#### **Key Technologies**

Displays top ten key technologies for the company and its subsidiaries based on frequency (technologies occurring with high and identical frequency are always included, and this may result in more than ten Key Technologies being listed). Includes both key technologies associated with patents where the company relationship is patent owner and key technologies associated with drugs in active development. A drug is classified as 'active' if it features on a row (or rows) in the current development status table where the status is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

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**PLEASE NOTE:** the financials section where present in the report includes: Share Information, Stock Performance chart (including the consensus recommendation), and Major Shareholders. Financial Performance presents graphical and tabular data on Worldwide Sales, Operating Income and Net Income over time, together with a Quarterly earnings update. Balance Sheet lists Assets, Liabilities and Stockholders Equity, and Forecasts includes: EPS Forecast and Other Forecasts. The data reported in all sections (except share information and major stock holders) are correct as of the publication date of the report (and not the download date). For share information and major stock holders the data is correct for the date shown with these sections



# **TABLE OF CONTENTS**

Company Overview	7
Company Profile	7
Product Portfolio Summary	8
Product Portfolio Drugs	11



## **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.



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**

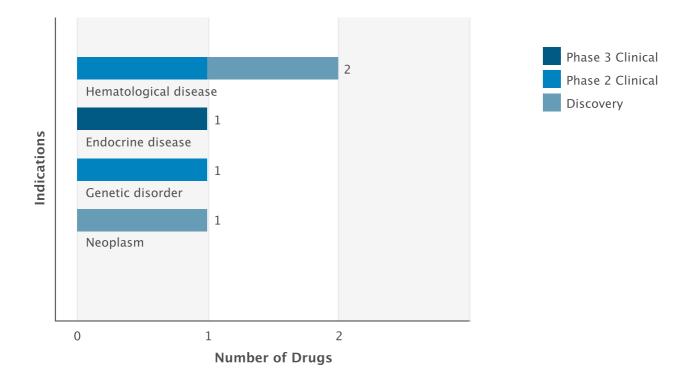
In August 1998, a Phase I SBIR grant was awarded by the NCI to support preclinical development of antiangiogenic gene therapy treatments for cancer.

# PRODUCT PORTFOLIO SUMMARY

### **DRUGS**

## **Drugs by Indication**

Active Drugs by Indication Chart



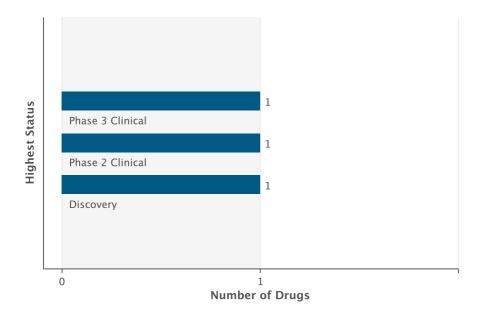


# Drugs by Indication Table

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





# 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	Prin	cipal	Pai	tner	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 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

### **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

<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.

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## **PRODUCT PORTFOLIO DRUGS**

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

## Lenti-D

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

# LentiGlobin

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	Retrovirus based gene therapy, Genetically engineered autologous cell 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



# CAR T-cells (cancer), bluebird bio

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	Solid tumor, Hematological neoplasm
Target-based Actions	
Other Actions	Anticancer, Gene therapy
Technologies	T-lymphocyte, Tumor antigen therapeutic, Biological therapeutic
Last Change Date	04-Dec-2013



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