

## **Acceleron Pharma Inc**

## **COMPANY AND PIPELINE OVERVIEW REPORT**

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 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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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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## **Acceleron Pharma Inc**

### **COMPANY OVERVIEW**

Company Name	Acceleron Pharma Inc
Parent Company Name	Acceleron Pharma Inc
Website	http://www.acceleronpharma.com/
Country	US
Number of Drugs in Active Development	6
Number of Inactive Drugs	2
Number of Patents as Owner	36
Number of Patents as Third Party	0
Number of Deals	8
Key Indications	Anemia,Beta thalassemia,Bone disease,Multiple myeloma,Rheumatoid arthritis,Duchenne dystrophy,Sarcopenia,Solid tumor,Age related macular degeneration,Bone resorption,Diamond Blackfan anemia,Endometrioid carcinoma,Myelodysplastic syndrome,Squamous cell carcinoma
Key Target-based Actions	TGF beta antagonist,Activin type-II receptor antagonist,GDF modulator,GDF-8 antagonist,Activin type-IIB receptor antagonist,Alk-1 protein kinase inhibitor,Immunoglobulin gamma Fc receptor agonist,Alk-3 protein kinase modulator,Bone morphogenetic protein-10 ligand modulator,Bone morphogenetic protein-9 ligand modulator,TGF beta ligand inhibitor,TNF
Key Technologies	Protein fusion, Biological therapeutic, Antibody fragment, Subcutaneous formulation, Immunoglobulin-G, Receptor fusion, Small molecule therapeutic, Antibody, Intraperitoneal formulation, Parenteral formulation unspecified, Protein chimeric, Soluble receptor, Systemic formulation unspecified

### **COMPANY PROFILE**

### **SUMMARY**

Acceleron Pharma Inc, founded in 2003 and headquartered in Cambridge, MA, is a biopharmaceutical company using approaches that exploit the therapeutic potential of a large family of growth and differentiation factors.

### **COMPANY LOCATION**

In May 2009, Acceleron signed a lease on a third facility in Cambridge, MA. At that time, the company planned to increase its workforce by 50% in 2009.

In June 2008, the company announced that it was to double the size of its facilities in Cambridge, MA and increase its workforce by 40% during 2008.

### LICENSING AGREEMENTS

In February 2008, as part of a codevelopment agreement for ACE-011, Acceleron and Celgene signed an option deal for the the development of three discovery programs in the field of osteoporosis and bone loss.

In August 2004, Artemis Pharmaceuticals GmbH and Acceleron signed a cooperation agreement in mouse genetics. Artemis would apply its ArteMice technology platforms to generate a specifically genetically engineered mouse model system for Acceleron, which would use the mouse model to develop drugs for the treatment of musculoskeletal and metabolic disorders.

### **EARLY R&D**

By May 2007, the company was investigating a number of research programs including ACE-05X for bone loss, ACE-06X and ACE-07X for fat accumulation, and ACE-08X and ACE-07X for muscle loss.

### **FINANCIAL**



In December 2011, Acceleron raised \$30 million from a private financing.

In October 2007, the company raised \$31 million from a series C financing round. At that time, Acceleron planned to use the proceeds to advance its clinical and preclinical pipeline.

By August 2006, Acceleron had obtained \$30 million in Series B financing. In May 2007, investors included Advanced Technology Ventures, OrbiMed Advisors, Polaris Venture Partners, Sutter Hill Investors and Venrock Associates.

In January 2006, the company received \$8 million in debt financing from Hercules Technology Growth Capital.

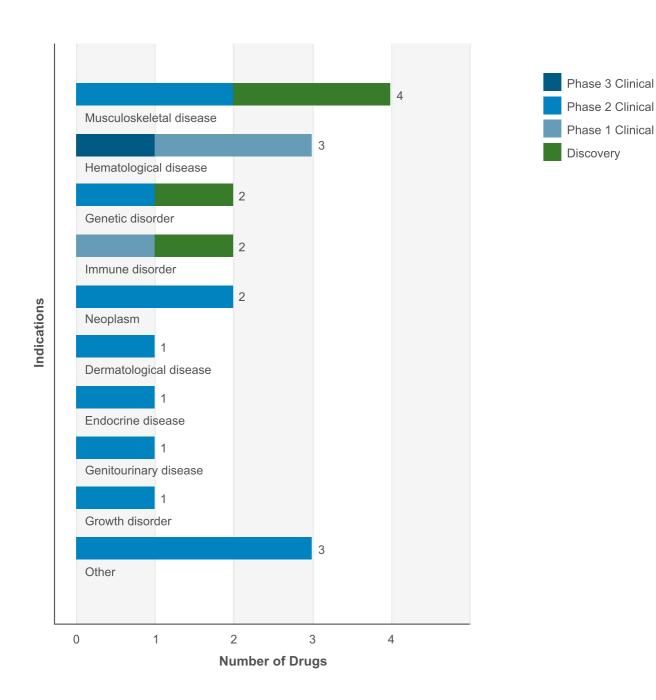
In February 2004, Acceleron raised \$25 million in its first round of private equity financing.



# PRODUCT PORTFOLIO SUMMARY DRUGS

## Drugs by Indication

Active Drugs by Indication Chart



## Drugs by Indication Table

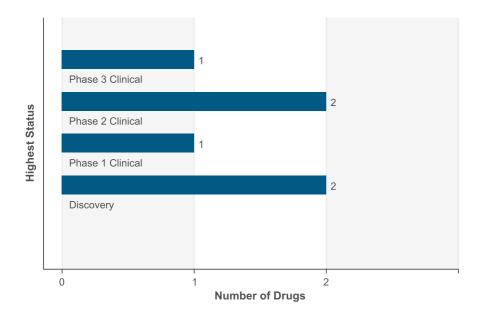
Indication	Active	Inactive	Total
Musculoskeletal disease	4	2	6



Hematological disease	3	0	3
Metabolic disorder	0	2	2
Growth disorder	1	1	2
Genetic disorder	2	0	2
Neoplasm	2	0	2
Inflammatory disease	1	1	2
Immune disorder	2	0	2
Ocular disease	1	0	1
Genitourinary disease	1	0	1
Nutritional disorder	0	1	1
Dermatological disease	1	0	1
Endocrine disease	1	0	1
Injury	1	0	1
Neurological disease	1	0	1
Gynecology and obstetrics	1	0	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	2
Phase 1 Clinical	1
Discovery	2
No Development Reported	2

## **DEALS**

Deal Type	Prin	cipal	Par	tner	Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	2	0	2
Drug - Funding	3	0	0	0	3
Drug - Development/Commercialization License	2	0	0	0	2
Technology - Delivery/Formulation	1	0	0	0	1

## **CLINICAL TRIALS**

# Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	2	7
Endocrine disease	0	5
Musculoskeletal disease	0	5
Hematological disease	1	4
Gynecology and obstetrics	0	3
Growth disorder	0	2
Immune disorder	0	2
Degeneration	0	2
Genitourinary disease	1	2
Neurological disease	0	2
Dermatological disease	1	1
Gastrointestinal disease	0	1



## Trials by Phase

Phase	Ongoing	All
Phase 2	3	8
Phase 1	0	6
Phase not specified	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
Immune disorder	13	0	13
Psychiatric disorder	1	0	1
Neoplasm	17	0	17
Ocular disease	2	0	2
Genetic disorder	1	0	1
Metabolic disorder	10	0	10
Mouth disease	1	0	1
Neurological disease	9	0	9
Degeneration	8	0	8
Andrology	1	0	1
Cardiovascular disease	11	0	11
Endocrine disease	22	0	22
Genitourinary disease	2	0	2
Dermatological disease	1	0	1
Gastrointestinal disease	12	0	12
Growth disorder	11	0	11



Hematological disease	11	0	11
Musculoskeletal disease	27	0	27
Nutritional disorder	11	0	11
Respiratory disease	3	0	3
Infectious disease	3	0	3
Injury	5	0	5
Inflammatory disease	9	0	9
Gynecology and obstetrics	7	0	7

<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 DRUGS**

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

## sotatercept

Drug Name	sotatercept
Key Synonyms	sotatercept
Originator Company	Acceleron Pharma Inc
Active Companies	Celgene Corp, Acceleron Pharma Inc
Inactive Companies	
Highest Status	Phase 3 Clinical
Active Indications	Anemia, Myelodysplastic syndrome, Beta thalassemia, Bone resorption, Diamond Blackfan anemia
Target-based Actions	Activin type-II receptor antagonist
Other Actions	Osteoblast modulator, Hematopoietic stimulant, Bone resorption inhibitor, Anticancer
Technologies	Antibody fragment, Immunoglobulin-G, Subcutaneous formulation, Biological therapeutic, Protein fusion
Last Change Date	08-Nov-2012

## ramatercept

Drug Name	ramatercept
Key Synonyms	ramatercept
Originator Company	Acceleron Pharma Inc
Active Companies	Shire plc, Acceleron Pharma Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Duchenne dystrophy, Sarcopenia
Target-based Actions	Activin type-IIB receptor antagonist, GDF-8 antagonist
Other Actions	Muscle system agent
Technologies	Antibody fragment, Immunoglobulin-G, Subcutaneous formulation, Biological therapeutic, Protein fusion
Last Change Date	12-Jul-2012



## dalantercept

Drug Name	dalantercept
Key Synonyms	dalantercept
Originator Company	Acceleron Pharma Inc
Active Companies	Acceleron Pharma Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Squamous cell carcinoma, Solid tumor, Age related macular degeneration, Endometrioid carcinoma, Multiple myeloma
Target-based Actions	TGF beta antagonist, Alk-1 protein kinase inhibitor, Bone morphogenetic protein-9 ligand modulator, Bone morphogenetic protein-10 ligand modulator
Other Actions	Ocular antineovascularisation agent, Angiogenesis inhibitor, Anticancer protein kinase inhibitor
Technologies	Antibody fragment, Receptor fusion, Subcutaneous formulation, Biological therapeutic, Protein chimeric, Protein fusion
Last Change Date	15-Nov-2012

## **ACE-536**

Drug Name	ACE-536
Key Synonyms	
Originator Company	Acceleron Pharma Inc
Active Companies	Celgene Corp, Acceleron Pharma Inc
Inactive Companies	
Highest Status	Phase 1 Clinical
Active Indications	Anemia, Beta thalassemia
Target-based Actions	TGF beta antagonist, TGF beta ligand inhibitor, GDF modulator
Other Actions	Hematopoietic stimulant
Technologies	Antibody fragment, Receptor fusion, Subcutaneous formulation, Biological therapeutic, Protein fusion, Soluble receptor
Last Change Date	26-Oct-2012

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## **ACE-661**

Drug Name     ACE-661       Key Synonyms     Acceleron Pharma Inc       Originator Company     Acceleron Pharma Inc       Active Companies     Acceleron Pharma Inc       Inactive Companies     Inactive Companies       Highest Status     Discovery       Active Indications     Bone disease       Target-based Actions     Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator       Other Actions     Steroid agonist, Bone modulator		
Originator Company Acceleron Pharma Inc Active Companies Acceleron Pharma Inc Inactive Companies Highest Status Discovery Active Indications Bone disease Target-based Actions Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Drug Name	ACE-661
Active Companies  Inactive Companies  Highest Status  Discovery  Active Indications  Bone disease  Target-based Actions  Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Key Synonyms	
Inactive Companies  Highest Status  Discovery  Active Indications  Bone disease  Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Originator Company	Acceleron Pharma Inc
Highest Status  Discovery  Active Indications  Bone disease  Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Active Companies	Acceleron Pharma Inc
Active Indications  Bone disease  Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Inactive Companies	
Target-based Actions  Immunoglobulin gamma Fc receptor agonist, TGF beta antagonist, Alk-3 protein kinase modulator	Highest Status	Discovery
protein kinase modulator	Active Indications	Bone disease
Other Actions Steroid agonist, Bone modulator	Target-based Actions	
<b>5</b> /	Other Actions	Steroid agonist, Bone modulator
<b>Technologies</b> Immunoglobulin-G, Systemic formulation unspecified, Intraperitoneal formulation, Biological therapeutic, Protein fusion	Technologies	
Last Change Date 16-Oct-2012	Last Change Date	16-Oct-2012

## **ACE-772**

Drug Name	ACE-772
Key Synonyms	
Originator Company	Acceleron Pharma Inc
Active Companies	Acceleron Pharma Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Rheumatoid arthritis
Target-based Actions	TNF antagonist
Other Actions	Anti-inflammatory
Technologies	Receptor fusion, Small molecule therapeutic
Last Change Date	05-Jul-2012

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