

## **Acceleron Pharma Inc**

### **COMPANY AND PIPELINE OVERVIEW REPORT**

Coverage of the company and a summary of the drug pipeline portfolio.

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#### **THOMSON REUTERS**

3 Times Square  
New York, New York 10036  
United States

Tel: +1 646 223 4000

[thomsonreuters.com](http://thomsonreuters.com)

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## GLOSSARY

### Number of Drugs in Active Development

Number of drugs associated with the company or subsidiary that are currently in active development, i.e. the development status for the drug(s) is one of the following: Discovery, Clinical, Phase I, Phase II, Phase III, Pre-registration, Registered, Launched, or Suspended.

### Number of Inactive Drugs

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

### Number of Patents as Owner

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

### Number of Patents as Third Party

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

### Patents summary table

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

### Number of Deals

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

### Key Indications

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

### Key Target-based Actions

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

### Key Technologies

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

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# Acceleron Pharma Inc

## COMPANY OVERVIEW

Company Name	Acceleron Pharma Inc
Parent Company Name	Acceleron Pharma Inc
Website	<a href="http://www.acceleronpharma.com/">http://www.acceleronpharma.com/</a>
Country	US
Number of Drugs in Active Development	4
Number of Inactive Drugs	5
Number of Patents as Owner	40
Number of Patents as Third Party	0
Number of Deals	8
Key Indications	Anemia,Beta thalassemia,Sickle cell anemia,Muscle weakness,Ovary tumor,Cancer,Diamond Blackfan anemia,Endometrioid carcinoma,Fallopian tube cancer,Hepatocellular carcinoma,Metastatic renal cancer,Myelofibrosis,Peritoneal tumor,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,Immunoglobulin gamma Fc receptor agonist,Alk-1 protein kinase inhibitor,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,Immunoglobulin-G,Antibody fragment,Subcutaneous formulation,Receptor fusion,Small molecule therapeutic,Protein chimeric,Soluble receptor,Antibody

## COMPANY PROFILE

### SUMMARY

Acceleron Pharma Inc, founded in 2003 and headquartered in Cambridge, MA, is a biopharmaceutical company developing novel protein therapeutics for cancer and rare diseases.

### 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.

### FINANCIA

In January 2014, the company filed registration statement for a proposed public offering of common stock. Later that

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month, the company priced the offering of 2,400,000 shares of common stock at a price of \$50 per share. The underwriters were granted a 30-day option to buy up to an additional 360,000 shares of common stock. Later in January 2014, the offering closed, with full exercise of the underwriters' option. At that time, Acceleron had raised aggregate net proceeds of approximately \$129 million from the sale.

In December 2013, the company was selected for addition to the Russell 2000, Russell 3000 and Russell Global Indexes, and would join the indexes after the NASDAQ market closes on December 20, 2013.

In August 2013, Acceleron planned an IPO of its common stock. In September 2013, Acceleron priced its IPO of 5,580,000 shares of common stock at \$15.00 per share and granted the underwriters a 30-day option to purchase up to an additional 837,000 shares. Separately, in a concurrent, side-by-side private placement, Acceleron's collaboration partner, Celgene agreed to purchase 666,667 shares of common stock from Acceleron at the public offering price. Acceleron's stock has been approved for listing on the NASDAQ Global Market and was expected to begin trading under the ticker symbol "XLRN" on September 19, 2013. The IPO closed in September 2013. At that time, underwriters exercised in full option to purchase shares and the company raised gross proceeds of \$106.3 million. Net proceeds from the offering were \$96.7 million.

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.

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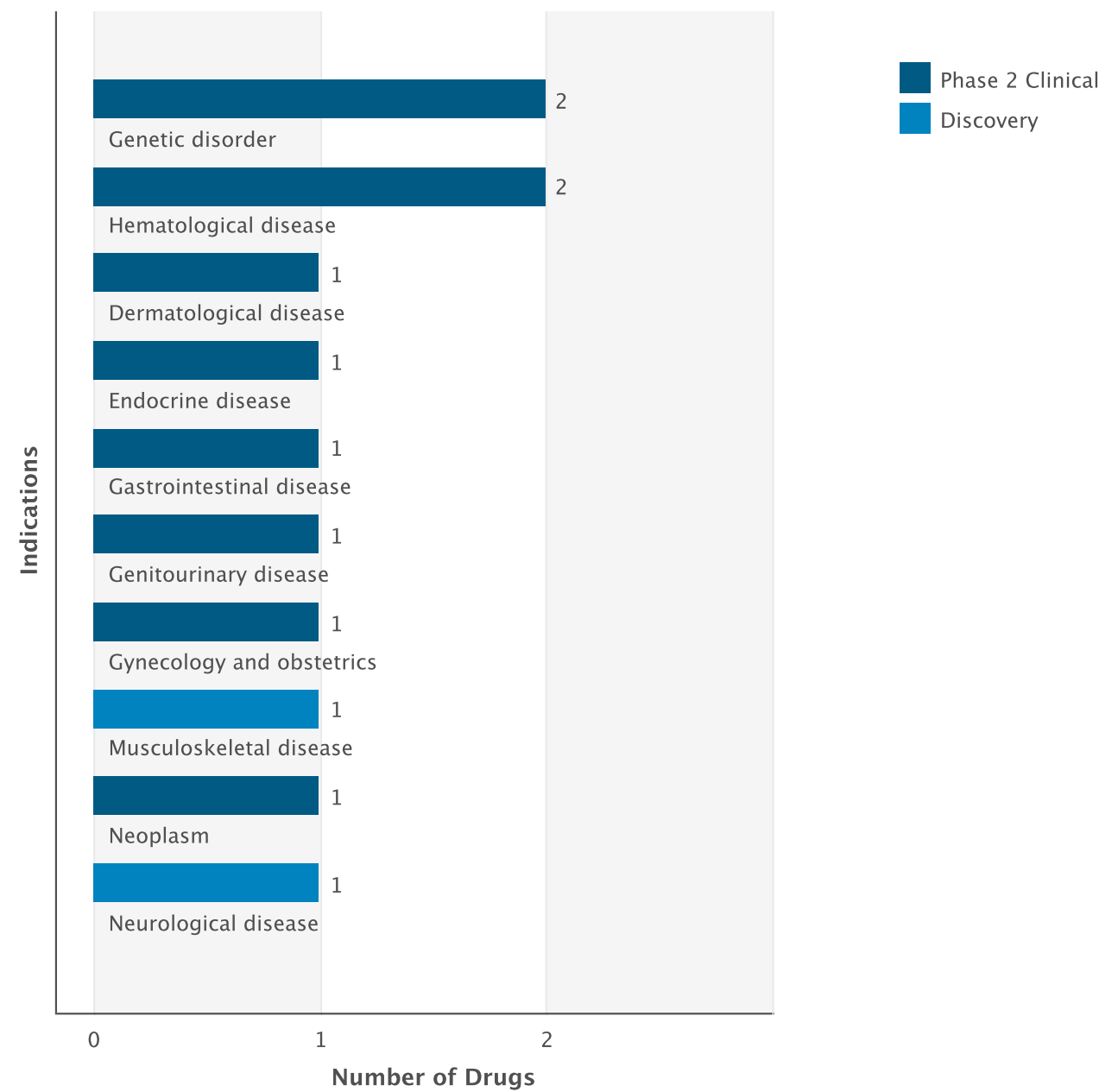


PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



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## Drugs by Indication Table

Indication	Active	Inactive	Total
Musculoskeletal disease	1	6	7
Hematological disease	2	2	4
Genetic disorder	2	1	3
Metabolic disorder	0	2	2
Growth disorder	0	2	2
Immune disorder	0	2	2
Inflammatory disease	0	2	2
Neurological disease	1	1	2
Ocular disease	0	1	1
Injury	0	1	1
Nutritional disorder	0	1	1
Dermatological disease	1	0	1
Genitourinary disease	1	0	1
Neoplasm	1	0	1
Gynecology and obstetrics	1	0	1
Endocrine disease	1	0	1
Gastrointestinal disease	1	0	1

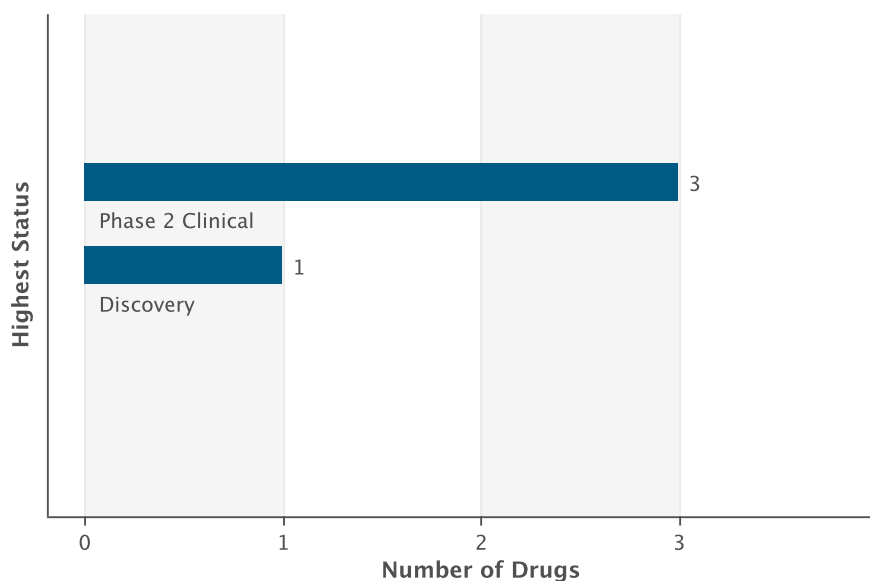
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## Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

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

## DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	2	0	2
Drug - Funding	3	0	0	0	3
Drug - Development/Commercialization License	1	0	0	0	2
Technology - Delivery/Formulation	1	0	0	0	1

## CLINICAL TRIALS

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## Trials by Condition Studied

Condition Studied	Ongoing	All
Neoplasm	4	8
Endocrine disease	1	6
Hematological disease	3	5
Musculoskeletal disease	0	5
Gynecology and obstetrics	1	4
Genitourinary disease	2	3
Degeneration	0	2
Neurological disease	0	2
Growth disorder	0	2
Gastrointestinal disease	2	2
Dermatological disease	1	1
Immune disorder	0	1
Genetic disorder	1	1

## Trials by Phase

Phase	Ongoing	All
Phase 2	7	11
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
Cardiovascular disease	13	0	13
Endocrine disease	23	0	23

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Gastrointestinal disease	14	0	14
Genitourinary disease	4	0	4
Growth disorder	13	0	13
Hematological disease	15	0	15
Degeneration	9	0	9
Andrology	1	0	1
Immune disorder	16	0	16
Psychiatric disorder	1	0	1
Musculoskeletal disease	30	0	30
Neoplasm	21	0	21
Ocular disease	3	0	3
Genetic disorder	2	0	2
Metabolic disorder	11	0	11
Mouth disease	1	0	1
Neurological disease	11	0	11
Nutritional disorder	11	0	11
Prophylaxis	1	0	1
Respiratory disease	4	0	4
Infectious disease	4	0	4
Injury	6	0	6
Toxicity and intoxication	1	0	1
Inflammatory disease	11	0	11
Gynecology and obstetrics	7	0	7
Dermatological disease	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 DRUGS

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

### luspatercept

<b>Drug Name</b>	luspatercept
<b>Key Synonyms</b>	luspatercept
<b>Originator Company</b>	Acceleron Pharma Inc
<b>Active Companies</b>	Celgene Corp, Acceleron Pharma Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Beta thalassemia, Sickle cell anemia, Anemia
<b>Target-based Actions</b>	TGF beta ligand inhibitor, GDF modulator, TGF beta antagonist
<b>Other Actions</b>	Hematopoietic stimulant
<b>Technologies</b>	Antibody fragment, Receptor fusion, Immunoglobulin-G, Subcutaneous formulation, Biological therapeutic, Protein fusion, Soluble receptor
<b>Last Change Date</b>	27-Feb-2014

### dalantercept

<b>Drug Name</b>	dalantercept
<b>Key Synonyms</b>	dalantercept
<b>Originator Company</b>	Acceleron Pharma Inc
<b>Active Companies</b>	Acceleron Pharma Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Peritoneal tumor, Squamous cell carcinoma, Fallopian tube cancer, Endometrioid carcinoma, Hepatocellular carcinoma, Ovary tumor, Metastatic renal cancer
<b>Target-based Actions</b>	Bone morphogenetic protein-9 ligand modulator, Bone morphogenetic protein-10 ligand modulator, TGF beta antagonist, Alk-1 protein kinase inhibitor
<b>Other Actions</b>	Anticancer, Ocular antineovascularisation agent, Angiogenesis inhibitor, Anticancer protein kinase inhibitor
<b>Technologies</b>	Antibody fragment, Receptor fusion, Subcutaneous formulation, Biological therapeutic, Protein chimeric, Protein fusion
<b>Last Change Date</b>	27-Feb-2014

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## sotatercept

<b>Drug Name</b>	sotatercept
<b>Key Synonyms</b>	sotatercept
<b>Originator Company</b>	Accelaron Pharma Inc
<b>Active Companies</b>	Accelaron Pharma Inc, Celgene Corp
<b>Inactive Companies</b>	
<b>Highest Status</b>	Phase 2 Clinical
<b>Active Indications</b>	Myelofibrosis, Beta thalassemia, Anemia, Sickle cell anemia, Diamond Blackfan anemia
<b>Target-based Actions</b>	Activin type-II receptor antagonist
<b>Other Actions</b>	Synergist, Bone resorption inhibitor, Osteoblast modulator, Hematopoietic stimulant
<b>Technologies</b>	Antibody fragment, Immunoglobulin-G, Subcutaneous formulation, Biological therapeutic, Protein chimeric, Soluble receptor
<b>Last Change Date</b>	27-Feb-2014

## ACE-083

<b>Drug Name</b>	ACE-083
<b>Key Synonyms</b>	
<b>Originator Company</b>	Accelaron Pharma Inc
<b>Active Companies</b>	Accelaron Pharma Inc
<b>Inactive Companies</b>	
<b>Highest Status</b>	Discovery
<b>Active Indications</b>	Muscle weakness
<b>Target-based Actions</b>	
<b>Other Actions</b>	Unspecified drug target
<b>Technologies</b>	Small molecule therapeutic
<b>Last Change Date</b>	27-Feb-2014

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