

## **Accelaron Pharma Inc**

### **CORTELLIS COMPANY DETAILED PIPELINE REPORT**

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

Publication Date: 18-Feb-2014

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

<b>Company Name</b>	Acceleron Pharma Inc
<b>Parent Company Name</b>	Acceleron Pharma Inc
<b>Website</b>	<a href="http://www.acceleronpharma.com/">http://www.acceleronpharma.com/</a>
<b>Country</b>	US
<b>Number of Drugs in Active Development</b>	4
<b>Number of Inactive Drugs</b>	5
<b>Number of Patents as Owner</b>	40
<b>Number of Patents as Third Party</b>	0
<b>Number of Deals</b>	8
<b>Key Indications</b>	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
<b>Key Target-based Actions</b>	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
<b>Key Technologies</b>	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 [Return to Table of Contents](#)

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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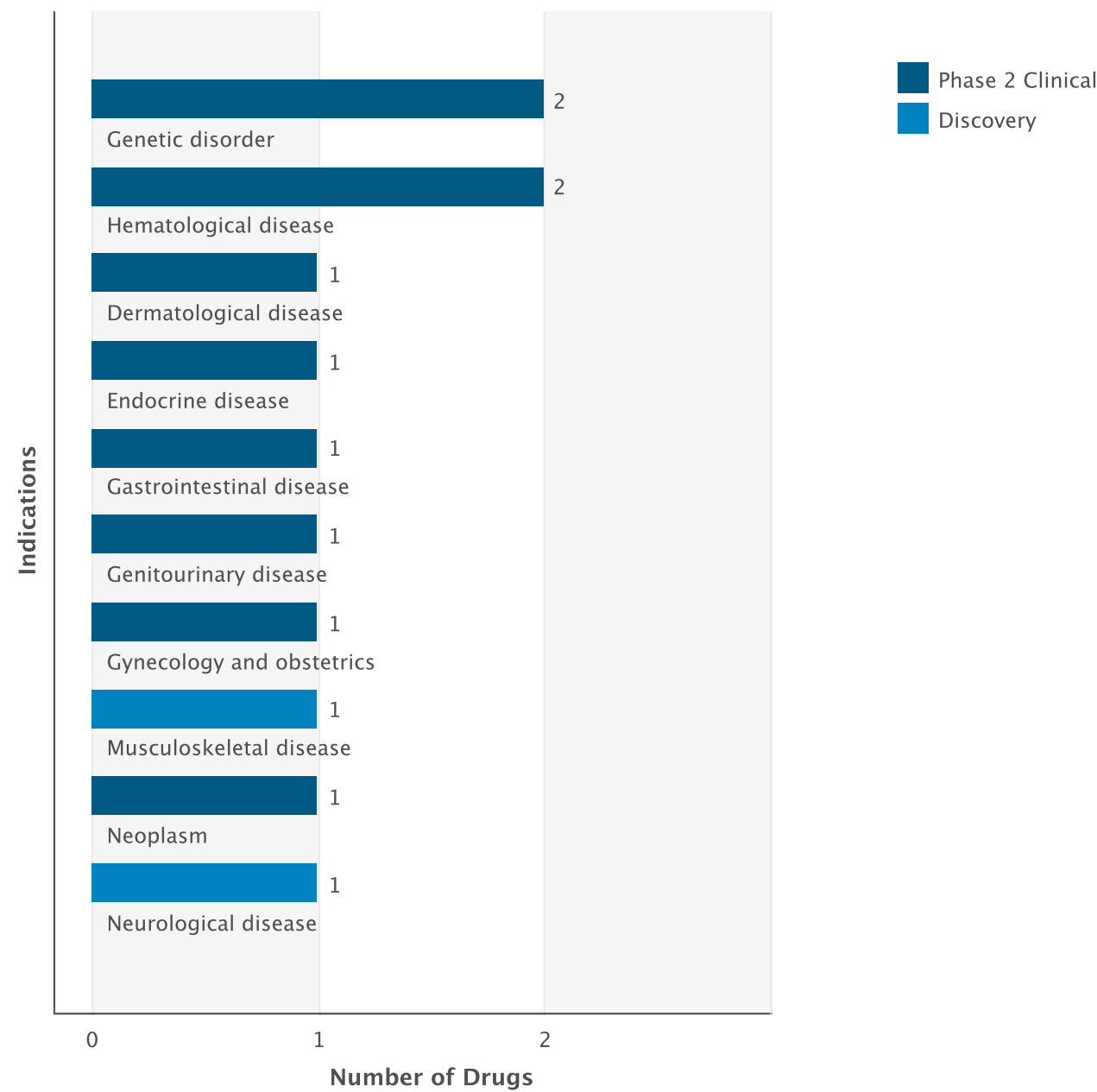


# PRODUCT PORTFOLIO SUMMARY

## DRUGS

### Drugs by Indication

Active Drugs by Indication Chart



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## Drugs by Indication Table

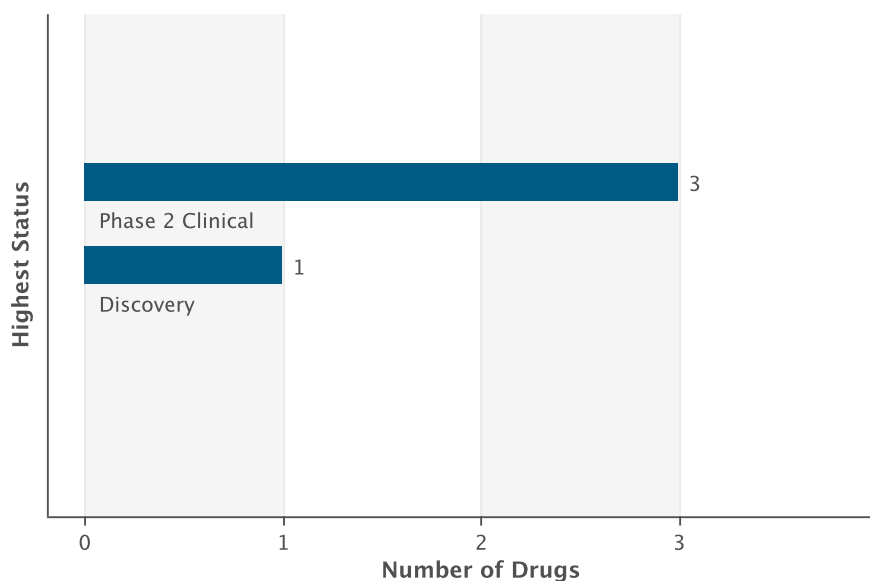
Indication	Active	Inactive	Total
Musculoskeletal disease	1	6	7
Hematological disease	2	2	4
Genetic disorder	2	1	3
Metabolic disorder	0	2	2
Growth disorder	0	2	2
Immune disorder	0	2	2
Inflammatory disease	0	2	2
Neurological disease	1	1	2
Ocular disease	0	1	1
Injury	0	1	1
Nutritional disorder	0	1	1
Dermatological 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

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## CLINICAL TRIALS

### 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

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Endocrine disease	23	0	23
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 DRUG PIPELINE DETAIL

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

### sotatercept

#### sotatercept SNAPSHOT

<b>Drug Name</b>	sotatercept
<b>Key Synonyms</b>	sotatercept
<b>Originator Company</b>	Acceleron Pharma Inc
<b>Active Companies</b>	Acceleron 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>	Hematopoietic stimulant;Bone resorption inhibitor;Osteoblast modulator;Synergist
<b>Technologies</b>	Antibody fragment;Immunoglobulin-G;Subcutaneous formulation;Biological therapeutic;Protein chimeric;Soluble receptor
<b>Last Change Date</b>	30-Jan-2014

#### sotatercept DEVELOPMENT PROFILE

##### SUMMARY

Acceleron and Celgene are developing sotatercept (ACE-011, ACE-011d, RAP-011, ActRIIA-IgG1), a fusion protein containing a soluble form of the activin IIa receptor that inhibits signalling of the receptor and an Fc fragment of IgG1, for the potential treatment of anemia caused by beta-thalassemia, myelodysplastic syndrome (MDS), diamond blackfan, myelofibrosis and chronic kidney disease ,,. In December 2013, the companies are also investigating the drug for the potential treatment of sickle cell anemia. In November 2012, a phase II trial began for the treatment of anemia in patients with MDS ; in November 2013, interim data were expected during the second quarter of 2014. In March 2012, phase IIa trial began in patients with red blood cell transfusion-dependent beta-thalassemia ; in November 2013, interim data were expected during the second quarter of 2014. By July 2010, a phase II trial for renal anemia had been initiated ; in January 2013, the trial was ongoing. In January 2012, an investigator-led phase I/II trial in patients with red blood cell transfusion-dependent Diamond-Blackfan anemia was initiated ; in October 2012, Celgene listed Diamond-Blackfan anemia on its development pipeline. In June 2013, the drug was listed as being in phase II for myelofibrosis. In December 2013, a registration enabling study was planned to be initiated by the end of 2014 or in the beginning of 2015.

The drug is also being developed in anemia of malignancy; a phase II trial began in solid tumor patients not undergoing chemotherapy in June 2010, which was ongoing in March 2013.

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Development was previously undertaken for chemotherapy-induced anemia. In July 2009, a phase II trial in chemotherapy-treated breast cancer patients was initiated and in February 2011, a phase II/III trial in chemotherapy-treated non-small-cell lung cancer (NSCLC) patients was initiated. However both trials were subsequently terminated due to low recruitment, and the indication no longer appears on the companies' pipeline. In October 2008, a phase II trial for multiple myeloma-associated bone loss was initiated, which was completed in October 2009; by March 2013, no further development had been reported.

## sotatercept DEVELOPMENT STATUS

### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Acceleron Pharma Inc	Anemia	US	Phase 2 Clinical	28-Jul-2009
Acceleron Pharma Inc	Beta thalassemia	France	Phase 2 Clinical	31-May-2012
Acceleron Pharma Inc	Diamond Blackfan anemia	US	Phase 2 Clinical	25-Oct-2012
Acceleron Pharma Inc	Myelofibrosis	US	Phase 2 Clinical	18-Jun-2013
Celgene Corp	Anemia	US	Phase 2 Clinical	28-Jul-2009
Celgene Corp	Beta thalassemia	France	Phase 2 Clinical	31-May-2012
Celgene Corp	Diamond Blackfan anemia	US	Phase 2 Clinical	25-Oct-2012
Celgene Corp	Myelofibrosis	US	Phase 2 Clinical	18-Jun-2013
Acceleron Pharma Inc	Sickle cell anemia	US	Discovery	03-Dec-2013
Celgene Corp	Beta thalassemia	US	Discovery	05-Dec-2013
Celgene Corp	Sickle cell anemia	US	Discovery	03-Dec-2013
Acceleron Pharma Inc	Bone resorption	US	No Development Reported	07-Mar-2013
Celgene Corp	Bone resorption	US	No Development Reported	07-Mar-2013

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## sotatercept DRUG NAMES

Names	Type
bone loss therapy, Acceleron	
sotatercept	INN, USAN
RAP-011	Research Code
activin IIa receptor antagonist (anemia, bone loss), Acceleron	
ACE-011d	Research Code
ACE-011	Research Code
ActRIIA-IgG1	

## sotatercept 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
Anemia											
0	0	0	0	4	6	0	0	0	0	4	6
Multiple myeloma											
0	0	0	0	0	1	1	1	0	0	1	2
Osteoporosis											
0	0	0	0	0	0	0	2	0	0	0	2
Diamond Blackfan anemia											
0	0	0	0	0	0	1	1	0	0	1	1
Myelofibrosis											
0	0	0	0	1	1	0	0	0	0	1	1
Beta thalassemia											
0	0	0	0	1	1	0	0	0	0	1	1
Myeloproliferative disorder											
0	0	0	0	1	1	0	0	0	0	1	1

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Solid tumor											
0	0	0	0	0	1	0	0	0	0	0	1
Metastatic breast cancer											
0	0	0	0	0	1	0	0	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	5	9	2	4	0	0	7	13

### 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

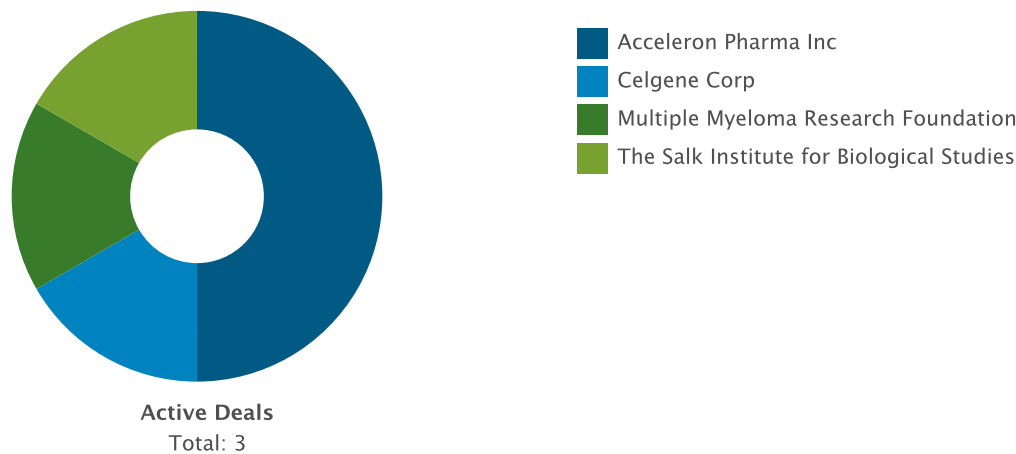
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

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sotatercept DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



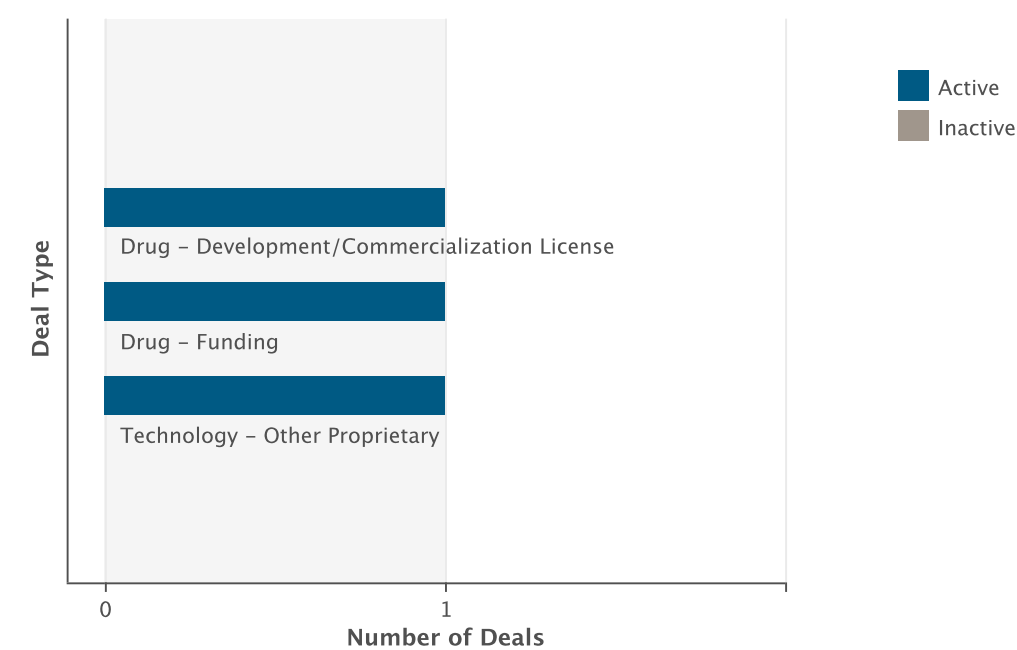
Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Acceleron Pharma Inc	2	0	1	0	3
Celgene Corp	0	0	1	0	1
The Salk Institute for Biological Studies	1	0	0	0	1
Multiple Myeloma Research Foundation	0	0	1	0	1

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Deals by Type Chart



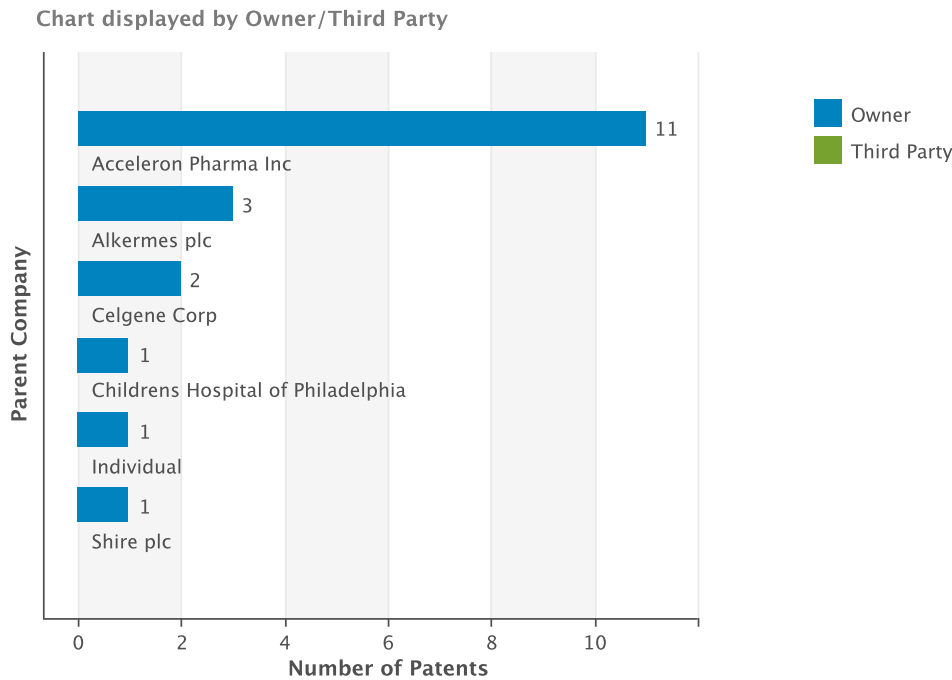
Deals by Type Table

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

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PATENTS

Patents by Parent Company Chart

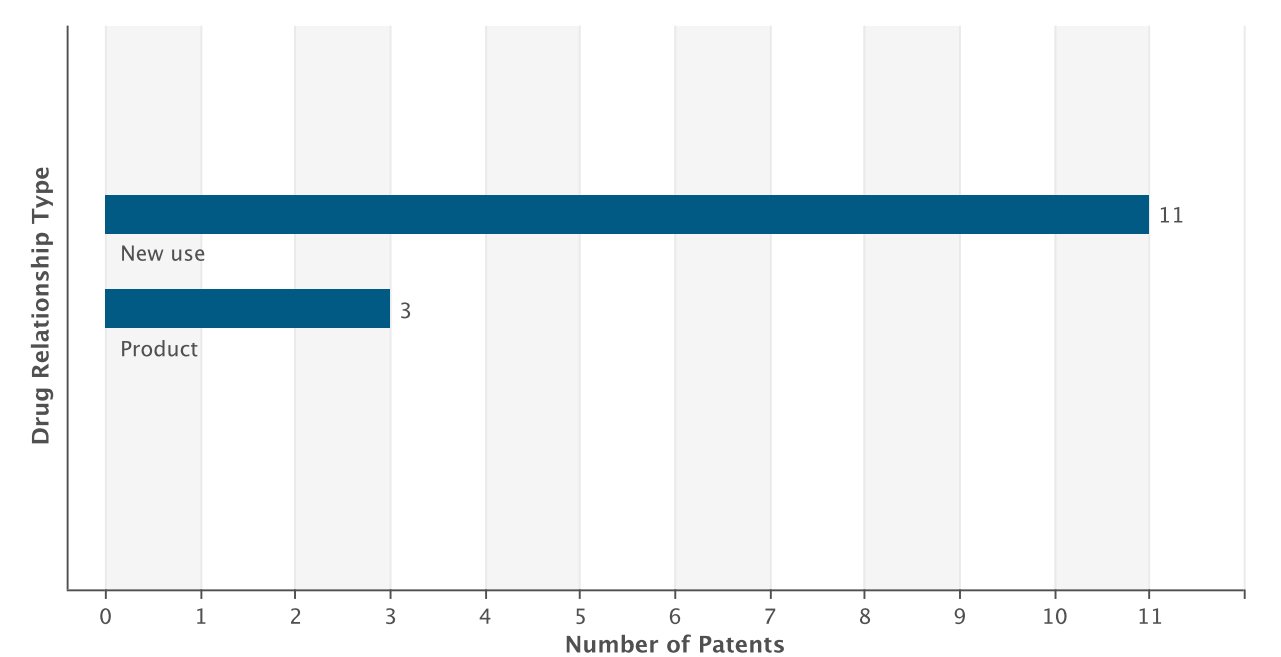


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Acceleron Pharma Inc	11	0	11
Alkermes plc	3	0	3
Celgene Corp	2	0	2
Shire plc	1	0	1
Individual	1	0	1
Childrens Hospital of Philadelphia	1	0	1

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	11
Product	3

## dalantercept

### dalantercept SNAPSHOT

<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-10 ligand modulator;Bone morphogenetic protein-9 ligand modulator;Alk-1 protein kinase inhibitor;TGF beta antagonist
<b>Other Actions</b>	Anticancer;Angiogenesis inhibitor;Anticancer protein kinase inhibitor;Ocular antineovascularisation agent
<b>Technologies</b>	Antibody fragment;Receptor fusion;Subcutaneous formulation;Biological therapeutic;Protein chimeric;Protein fusion
<b>Last Change Date</b>	04-Dec-2013

### dalantercept DEVELOPMENT PROFILE

#### SUMMARY

Acceleron is developing dalantercept (ACE-041, RAP-041), a chimeric protein consisting of a soluble activin receptor-like kinase 1 (ALK1) receptor with an Fc receptor, which inhibits angiogenesis by preventing the interaction of members of the TGFbeta protein superfamily (BMP9 and BMP10) with ALK1 to prevent signalling through the ALK1 receptor, for the potential sc treatment of squamous cell carcinoma, metastatic renal cell carcinoma, ovarian cancer, fallopian tube and primary peritoneal carcinoma,, , ,,. In December 2013, the company is also investigating dalantercept for the potential treatment of hepatocellular cancer (HCC). At that time, a phase II trial for HCC was expected to be initiated in the first quarter or early in the second quarter of 2014. In October 2011, a phase II trial in patients with squamous cell carcinoma of the head and neck (SCCHN) began. In November 2012, a phase II study was initiated in patients with persistent or recurrent epithelial ovarian, fallopian tube, or primary peritoneal carcinoma and at that time, the study was expected to complete in October 2015. In December 2012, a phase II study in patients with metastatic renal cell carcinoma was initiated and at that time, the study was expected to complete in December 2018; in November 2013, enrollment was completed for the dose-escalation stage. In October 2009, a phase I trial began in patients with solid tumors or multiple myeloma.

The company was previously developing the drug for the treatment of endometrial cancer,, , ,,. In September 2012, a phase II trial in endometrial cancer was initiated ; however by December 2013, dalantercept had shown no sufficient activity to warrant development as a monotherapy for endometrial cancer and will not advance to the second stage of this trial.

The company was previously investigating the drug for the treatment of age-related macular degeneration (AMD), solid tumors, multiple myeloma. In September 2011, the drug was listed as being in phase I development for AMD, solid tumors and multiple myeloma ; however in June 2013, the indications were no longer listed on the current pipeline.

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The company was previously investigating the drug for arthritis,. However, no further development was reported for this indication since May 2006.

## **dalantercept DEVELOPMENT STATUS**

### **CURRENT DEVELOPMENT STATUS**

<b>Company</b>	<b>Indication</b>	<b>Country</b>	<b>Development Status</b>	<b>Date</b>
Acceleron Pharma Inc	Fallopian tube cancer	US	Phase 2 Clinical	30-Nov-2012
Acceleron Pharma Inc	Metastatic renal cancer	US	Phase 2 Clinical	31-Dec-2012
Acceleron Pharma Inc	Ovary tumor	US	Phase 2 Clinical	30-Nov-2012
Acceleron Pharma Inc	Peritoneal tumor	US	Phase 2 Clinical	30-Nov-2012
Acceleron Pharma Inc	Squamous cell carcinoma	US	Phase 2 Clinical	21-Oct-2011
Acceleron Pharma Inc	Hepatocellular carcinoma	US	Discovery	03-Dec-2013
Acceleron Pharma Inc	Endometrioid carcinoma	US	Suspended	22-Nov-2013
Acceleron Pharma Inc	Age related macular degeneration	US	No Development Reported	18-Jun-2013
Acceleron Pharma Inc	Arthritis	US	No Development Reported	16-Nov-2007
Acceleron Pharma Inc	Multiple myeloma	US	No Development Reported	18-Jun-2013
Acceleron Pharma Inc	Solid tumor	US	No Development Reported	18-Jun-2013

## **dalantercept DRUG NAMES**

<b>Names</b>	<b>Type</b>
dalantercept	INN, USAN
ACE-041	Research Code
chimeric receptor TGF beta antagonist (sc, solid tumors/multiple myeloma/AMD), Acceleron	
recombinant ALK-1 signaling inhibitor (angiogenesis), Acceleron	
Alk1-Fc	
RAP-041	Research Code

## **dalantercept CLINICAL TRIALS**

### **Trials by Phase and Condition Studied**

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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
Hepatocellular carcinoma											
0	0	0	0	1	1	0	0	0	0	1	1
Ovary tumor											
0	0	0	0	1	1	0	0	0	0	1	1
Renal cell carcinoma											
0	0	0	0	1	1	0	0	0	0	1	1
Squamous cell carcinoma											
0	0	0	0	1	1	0	0	0	0	1	1
Fallopian tube cancer											
0	0	0	0	1	1	0	0	0	0	1	1
Metastatic head and neck cancer											
0	0	0	0	1	1	0	0	0	0	1	1
Peritoneal tumor											
0	0	0	0	1	1	0	0	0	0	1	1
Endometrioid carcinoma											
0	0	0	0	0	1	0	0	0	0	0	1
Advanced solid tumor											
0	0	0	0	0	0	0	1	0	0	0	1
Multiple myeloma											
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	4	5	0	1	0	0	4	6

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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

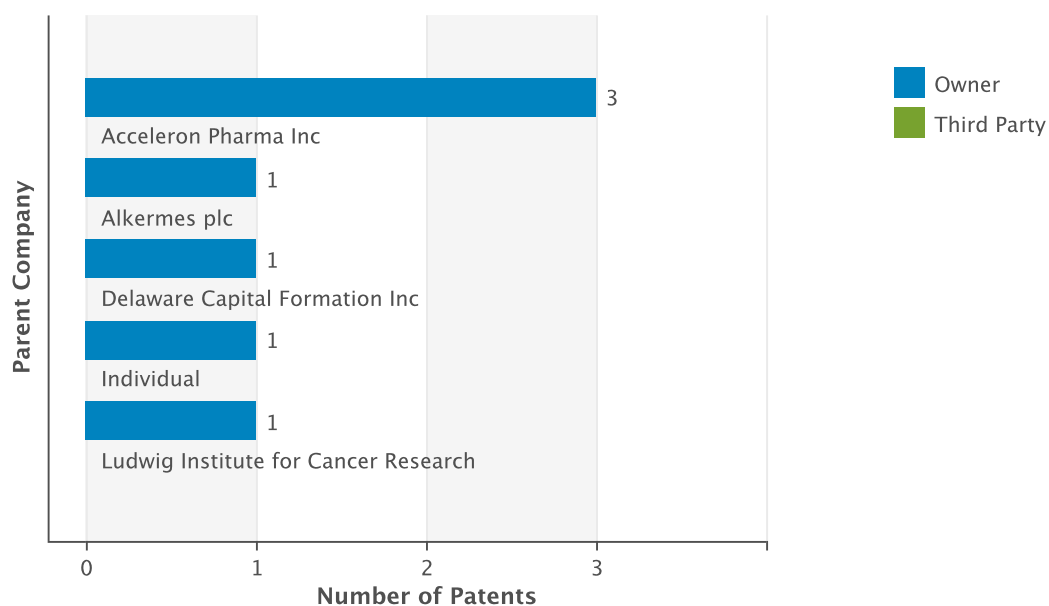
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

## dalantercept DEALS AND PATENTS

### PATENTS

#### Patents by Parent Company Chart

Chart displayed by Owner/Third Party

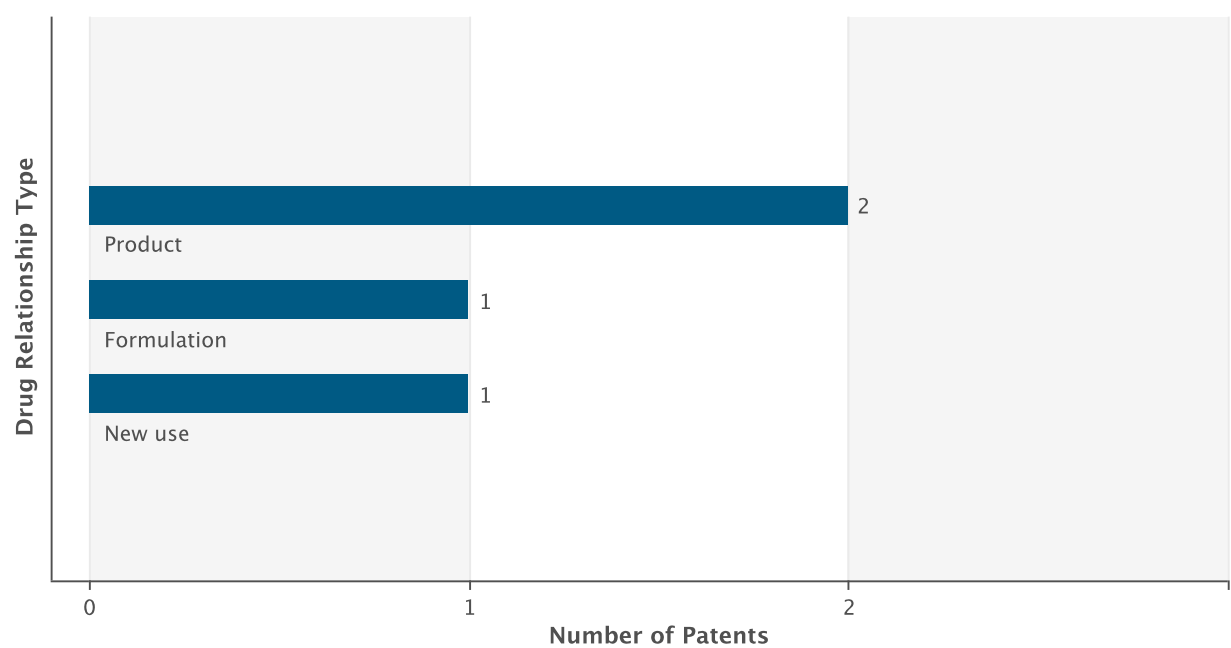


#### Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Acceleron Pharma Inc	3	0	3
Ludwig Institute for Cancer Research	1	0	1
Individual	1	0	1
Delaware Capital Formation Inc	1	0	1
Alkermes plc	1	0	1

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Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	2
New use	1
Formulation	1



# luspatercept

## luspatercept SNAPSHOT

<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>	GDF modulator;TGF beta antagonist;TGF beta ligand inhibitor
<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>	03-Jan-2014

## luspatercept DEVELOPMENT PROFILE

### SUMMARY

Acceleron Pharma, in collaboration with Celgene, is developing luspatercept (ACE-536, ACE-536d), a growth and differentiation factor (GDF) protein-based therapy and a soluble receptor fusion protein consisting of a modified human activin receptor type-IIb extracellular domain linked to a human IgG1 Fc domain, that modulates GDF ligands and receptors, and selectively increases red blood cells and hemoglobin by inhibiting TGF beta protein superfamily members, for the potential treatment of anemia associated with myelodysplastic syndrome and beta thalassemia,,,,. In December 2013, the companies are also investigating the drug for the potential treatment of sickle cell anemia. In January 2013, a phase II trial for beta-thalassemia was initiated in Italy ; in November 2013, interim data were expected during the second quarter of 2014. In January 2013, a phase II trial was initiated in Germany for the treatment of anemia in patients with myelodysplastic syndrome; in November 2013, interim data were expected during the second quarter of 2014. In December 2013, a registration enabling study was planned to be initiated by the end of 2014 or in the beginning of 2015.

## luspatercept DEVELOPMENT STATUS

### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Acceleron Pharma Inc	Anemia	Germany	Phase 2 Clinical	17-Jan-2013
Acceleron Pharma Inc	Beta thalassemia	Italy	Phase 2 Clinical	11-Jan-2013
Celgene Corp	Anemia	Germany	Phase 2 Clinical	17-Jan-2013
Celgene Corp	Beta thalassemia	Italy	Phase 2 Clinical	11-Jan-2013

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Company	Indication	Country	Development Status	Date
Acceleron Pharma Inc	Beta thalassemia	US	Discovery	11-Mar-2013
Acceleron Pharma Inc	Sickle cell anemia	US	Discovery	03-Dec-2013
Celgene Corp	Beta thalassemia	US	Discovery	11-Mar-2013
Celgene Corp	Sickle cell anemia	US	Discovery	03-Dec-2013

## luspatercept DRUG NAMES

Names	Type
RAP-536	Research Code
growth and differentiation factor (GDF) protein-based therapy (anemia associated with myelodysplastic syndrome), Acceleron luspatercept	PINN, USAN
ACE-536	Research Code
Anemia therapy, Acceleron	
GDF ligands and receptors modulator (anemia), Acceleron	
ACE-536d	Research Code
GDF ligands and receptors modulator (anemia associated with myelodysplastic syndrome), Acceleron/ Celgene	

## luspatercept 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
Anemia											
0	0	0	0	1	1	0	0	0	0	1	1
Beta thalassemia											
0	0	0	0	1	1	0	0	0	0	1	1

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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	2	2	0	1	0	0	2	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)

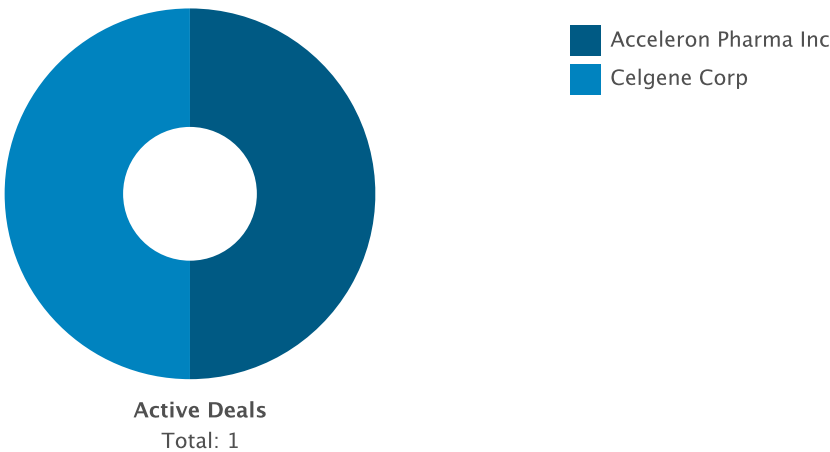
Phase 1 Clinical

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

Iuspatercept DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

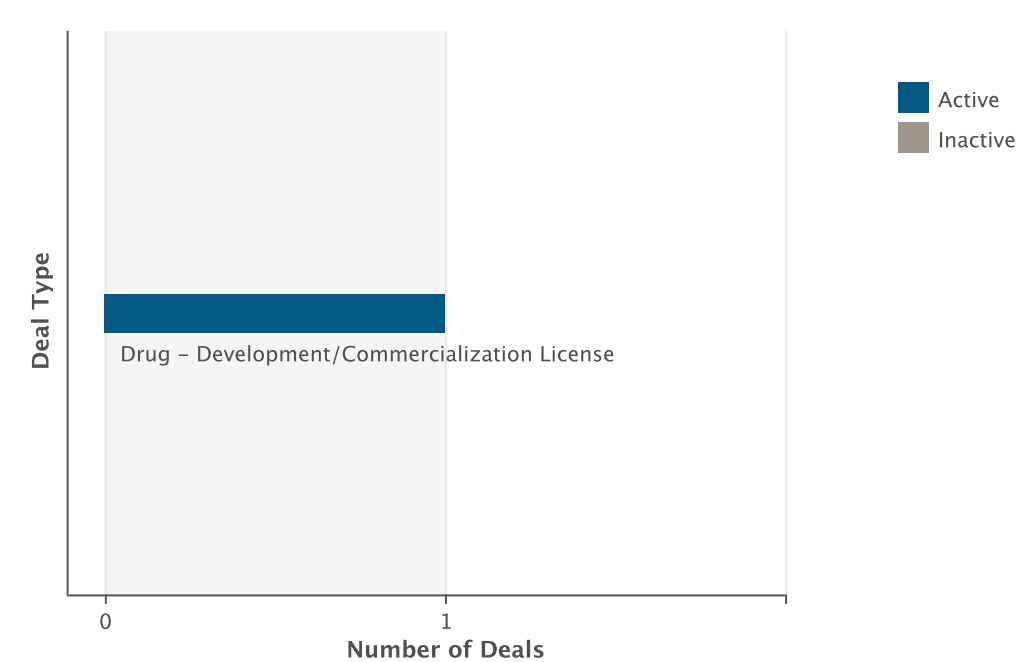


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Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Celgene Corp	0	0	1	0	1
Acceleron Pharma Inc	1	0	0	0	1

Deals by Type Chart



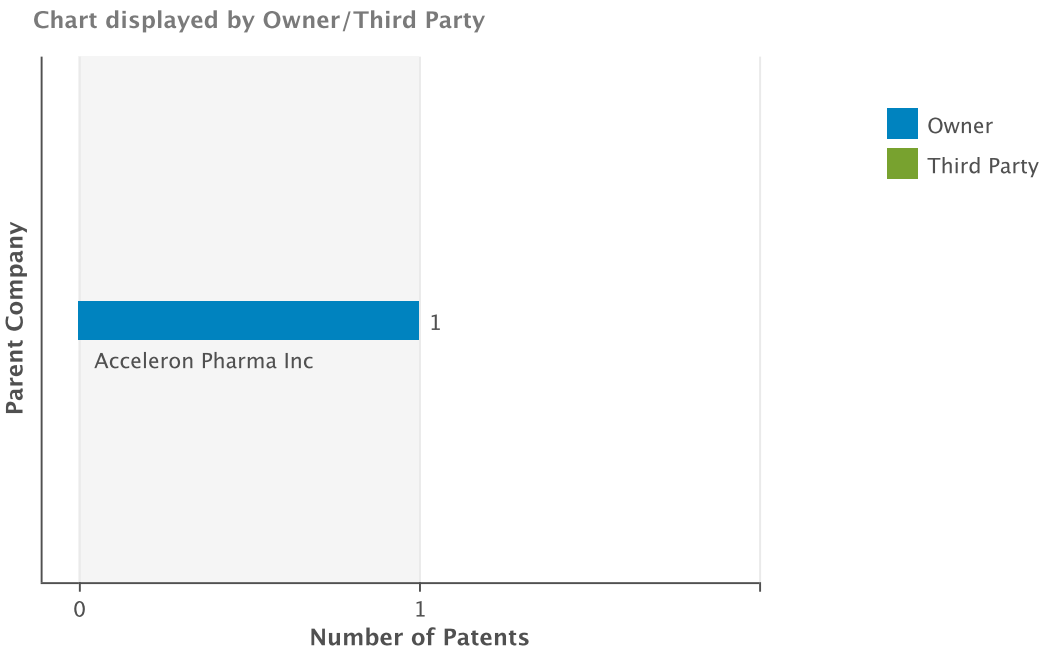
Deals by Type Table

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

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PATENTS

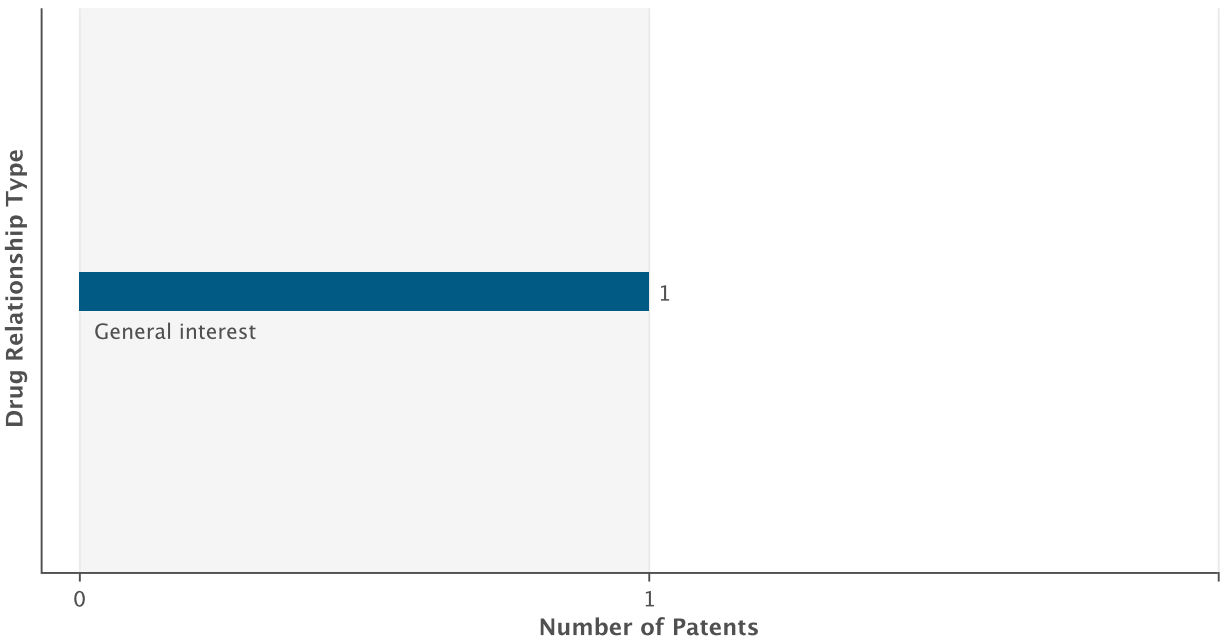
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Acceleron Pharma Inc	1	0	1

Patents by Drug Relationship Type Chart



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Patents by Drug Relationship Type Table

Drug Relationship	Total
General interest	1

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## ACE-083

### ACE-083 SNAPSHOT

Drug Name	ACE-083
Key Synonyms	
Originator Company	Acceleron Pharma Inc
Active Companies	Acceleron Pharma Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Muscle weakness
Target-based Actions	
Other Actions	Unspecified drug target
Technologies	Small molecule therapeutic
Last Change Date	20-Dec-2013

### ACE-083 DEVELOPMENT PROFILE

#### SUMMARY

Acceleron is investigating a locally acting agent, ACE-083, for the potential treatment of muscle weakness by increasing muscle mass and strength. In November 2013, preclinical activities were ongoing, in preparation for the submission of an IND in 2014.

### ACE-083 DEVELOPMENT STATUS

#### CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Acceleron Pharma Inc	Muscle weakness	US	Discovery	06-Nov-2013

### ACE-083 DRUG NAMES

Names	Type
ACE-083	Research Code

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