Cortellis

Amicus Therapeutics Inc

CORTELLIS COMPANY DETAILED PIPELINE REPORT

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

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Return to Table of Contents

Clarivate Analytics

ABOUT CORTELLIS COMPANY DETAILED PIPELINE REPORT

Clarivate Analytics 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.

This report was created by Clarivate, using information from *Cortellis for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information.From drug discovery and development activities to patent reports, the latest deals, and partnering opportunities, *Cortellis* can provide the confidence to make the most informed business decisions, faster. *Cortellis for Competitive Intelligence* provides accurateand validated information on pharmaceutical and biotechnology companies globally, their drug pipelines, deals, patents, and clinical trials, plus breaking industry news and conference coverage. All contained in one simple, highly intuitive research platform.

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Return to Table of Contents

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.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

Return to Table of Contents

TABLE OF CONTENTS

Return to Table of Contents

Amicus Therapeutics Inc

COMPANY OVERVIEW

Company Name	Amicus Therapeutics Inc
Parent Company Name	Amicus Therapeutics Inc
Website	http://www.amicusrx.com/
Country	US
Number of Drugs in Active Development	11
Number of Inactive Drugs	7
Number of Patents as Owner	62
Number of Patents as Third Party	1
Number of Deals	26
Key Indications	Pompes disease,Fabry disease,Gaucher disease,Parkinsons disease,Alzheimers disease,Mucopolysaccharidosis type I,Neurological disease,Epidermolysis bullosa,Lysosome storage disease,Psoriasis
Key Target-based Actions	Alpha-glucosidase modulator,Glucosylceramidase stimulator,Chaperonin stimulator,Amyloid protein deposition inhibitor,Alpha-galactosidase stimulator,Alpha-glucosidase stimulator,Alpha-galactosidase modulator,Glucosylceramidase modulator,Alpha-L-iduronidase stimulator,Beta-N-acetylhexosaminidase stimulator,Presenilin 1 stimulator,Unspecified enzyme stimulator
Key Technologies	Biological therapeutic,Recombinant enzyme,Drug combination,Small molecule therapeutic,Oral formulation,Intravenous formulation,Infusion,Enzyme,Subcutaneous formulation,Parenteral formulation

COMPANY PROFILE

SUMMARY

Amicus Therapeutics Inc, established in 2002, is focused on the development of orally active small molecule chaperones that act by stabilizing inhibitor-bound enzyme conformations, leading to enhanced functional activity of enzymes for the potential treatment of neurodegenerative diseases.

COMPANY LOCATION

Amicus is headquartered in Cranbury, NJ.

In September 2008, Amicus opened a research facility in San Diego, CA. In November 2013, the company planned to close the San Diego research facility.

LICENSING AGREEMENTS

In November 2007, Amicus and Shire Human Genetic Therapies, a subsidiary of Shire, agreed to jointly codevelop Amicus' three lead chaperone compounds for the potential treatment of lysosomal storage diseases. Shire would obtain rights to commercialize the products outside the US, whereas Amicus would retain rights to commercialize the products within the US. In October 2009, Amicus and Shire terminated their agreement, giving Amicus worldwide rights to Amigal, Plicera and AT2220. In the fourth quarter of 2009, Shire would pay Amicus $5.2 million as final payment under the collaboration.

By March 2004, the company was working towards identifying compounds that shepherd the correct folding of proteins in various diseases using technology licensed from Mount Sinai School of Medicine. The first target to be examined was involved in lysosomal storage disease; other targets were the cystic fibrosis transmembrane regulator, G-protein-gated inwardly rectifying potassium channels and p53 tumor suppressor.

ACQUISITIONS & SPIN-OFFS

By July 2016, the company had acquired MiaMed for $1.8 million in cash and approximately $4.7 million in Amicus

Return to Table of Contents

common stock. The former shareholders of MiaMed were eligible to receive up to $18 million upon the achievement of clinical and regulatory milestones and up to $65 million upon achievement of commercial milestones.

In August 2015, Amicus and Scioderm entered into a definitive agreement wherein 100% of Scioderm's capital stock would be acquired by Amicus for $229 million of which $125 million would be paid in cash and $104 million would be paid through the issuance of 7 million newly issued Amicus shares. Furthermore, Amicus agreed to pay up to an additional $361 and $257 million to Scioderm shareholders in cash or stock upon achievement of certain clinical and regulatory milestones, and sales milestones, respectively. Moreover, if a Priority Review Voucher for Zorblisa was obtained and subsequently sold, Amicus would pay Scioderm shareholders the lesser of $100 million or 50% of the proceeds of such sale. At that time, the transaction was expected to be completed in the third quarter of 2015. In September 2015, the acquisition was completed.

In November 2013, Amicus acquired Callidus Biopharma. Under terms of agreement, Callidus shareholders would receive $15 million in shares of Amicus' common stock, up to $10 million in milestones through phase II development of the Pompe program and milestone payments of up to $105 million on late-stage development achievements.

FINANCIAL

In February 2018, Amicus Therapeutics announced that it had commenced a $250 million underwritten public offering of its common stock. The company expected to grant the underwriters a 30-day option to purchase up to an additional 15% of the shares of common stock offered in the public offering. In February 2018, the company priced its underwritten offering of 19,354,839 shares of its common stock at $15.50 per share and expected to raise gross proceeds of $300 million. Amicus granted the underwriters a 30-day option to purchase up to an additional 2,903,225 shares of the common stock; the offering was expected to close on February 21, 2018. Later in February 2018, Amicus announced the closing of its offering of common stock; the Company issued a total of 19,354,839 shares at $15.50 per share.

In July 2017, the company had commenced a $225 million underwritten public offering of its common stock. The company expected to grant the underwriters a 30-day option to purchase up to an additional 15% of the shares of common stock offered in the public offering. Later that month, the company priced its 18,367,347 shares of its common stock at $12.25 per share and expected to raise gross proceeds of $225 million. The underwriters were granted a 30-day option to purchase up to an additional 2,755,102 shares of its common stock. At that time, the offering was expected to close on July 18, 2017. Later that month, the offering was closed and the company raised gross proceeds expected to be $258.8 million through a total of 21,122,449 shares in the offering at a price of $12.25 per share that included 2,755,102 shares additionally exercised by the underwriters in full option.

In December 2016, the company intended to offer $225,000,000 aggregate principal amount of convertible senior notes due 2023 in a private placement under the Securities Act of 1933. Amicus granted the initial purchasers an option, exercisable for 30 days, to purchase up to an additional $25 million aggregate principal amount of notes; later that month, the company priced its private offering of $225 million aggregate principal amount of 3.00% convertible senior notes due 2023. The gross proceeds from the offering were expected to be $218.7 million. At that time, the offering was expected to close on or about December 21, 2016. Later, in December 2016, the offering was closed. Aggregate net proceeds were expected to be $243.0 million.

In June 2015, the company was added to the Russell 3000 and small-cap Russell 2000 Indexes.

In June 2015, the company initiated a $150 million underwritten public offering of its common stock. The company expected to grant the underwriters a 30-day option to purchase up to an additional 15% of the shares of common stock offered in the public offering. Later that month, the company announced the pricing of an underwritten offering of 16,981,132 shares of its common stock at $13.25 per share for gross proceeds $225 million. The company had granted the underwriters a 30-day option to buy up to an additional 2,547,170 shares of its common stock. At that time, the offering was expected to close on June 17, 2015. Again later that month, the offering was closed and the company raised gross proceeds of approximately $258.8 million through issuance of 19,528,302 shares including exercise in full of option purchase.

In November 2014, the company priced a 13,850,000 underwritten shares offering of its common stock at $6.50 per share and the gross proceeds were expected to be $90.0 million. Amicus was to grant underwriters a 30-day option to purchase an additional 2,077,500 shares. The offer was expected to close on November 24, 2014; later that month, the offering was closed with a total of 15,927,500 shares at a price of $6.50 per share. The gross proceeds from the offering were expected to be $103.5 million.

In November 2014, the company announced a $75 million underwritten public offering of its common stock and also expected to grant the underwriters a 30-day option to purchase up to an additional 15% of the shares.

In November 2013, the company raised approximately $40 million through private placement and expected debt financing. In the private placement, the company raised $15 million from 7.5 million shares of common stock at $2.00 each and issued warrants for an additional 1.6 million shares at $2.50 each with one-year term exercisable between July 01, 2014 and June 30, 2015. In the debt financing, the company raised $25 million, at a cost of capital of less than 10% which was expected to close in 'coming weeks'. In December 2013, the company completed the debt financing and drew down $15 million under the debt facility with a second tranche of $10 million, available through the end of the fourth

Return to Table of Contents

quarter of 2014.

In May 2012, Amicus was added to the NASDAQ Biotechnology Index, effective on May 21, 2012. In December 2013, NASDAQ reported that, Amicus would be de-listed from the NASDAQ Biotechnology Index, as a result of the annual re-ranking, which would become effective prior to market open on December 23, 2013. In December 2014, the company was added to the NASDAQ Biotechnology Index.

In March 2012, the company priced a 10 million underwritten share offering at $5.70 per share for $54 million net proceeds. Amicus was to grant underwriters a 30-day option to purchase an additional 1.5 million shares. The offer was to close on March 07, 2012. Later in March 2012, the offering closed. Underwriters exercised in full their overallotment option to purchase 1.5 million shares, bringing the total number of shares issued to 11.5 million for net proceeds of $62 million.

In June 2011, the company was added to the Russell 2000 and Russell 3000 indexes.

In February 2010, Amicus planned to raise $18.5 million from a registered direct offering of units made up of 4.95 million shares of common stock and four-year warrants to purchase 1.85 million shares. Each unit, priced at $3.74 each, would consist of a share and a warrant to buy 0.375 of a share. The warrants would be exercisable at $4.43 per share. At that time, the transaction was expected to close on or about March 02, 2010. In March 2010, the offering was closed. The company had raised $18.5 million in total resulting in net proceeds of $17.1 million.

In May 2007, Amicus priced its IPO of 5 million shares at a price of $15 per share; it granted the underwriters a 30-day option to buy up to an additional 0.75 million shares to cover any over- allotments. The company's shares were to begin trading on the NASDAQ Global Market under the trading symbol 'FOLD'.

In March 2006, Amicus filed a registration statement for a US IPO.

In September 2006, Amicus closed a $60 million series D financing, the company's largest to date. The funds would be used to advance the company's lysosomal storage disorders programs and develop its earlier-stage programs for other genetic diseases.

In August 2006, the company withdrew a registration statement for a proposed IPO due to market conditions. The registration statement for the IPO was filed with the SEC in May 2006.

In September 2005, Amicus raised $55 million from a series C financing. The funds would be used to advance the company's pipeline.

In May 2004, Amicus completed a $31 million series B private equity financing round.

R&D GRANTS

In January 2007, Amicus received a grant from The Michael J Fox Foundation for Parkinson's Research to fund development of a Parkinson's disease therapy using the company's pharmacological chaperone technology.

EARLY R&D/TECHNOLOGY

In March 2013, Amicus named its technology platform chaperone-advanced replacement therapy (CHART).

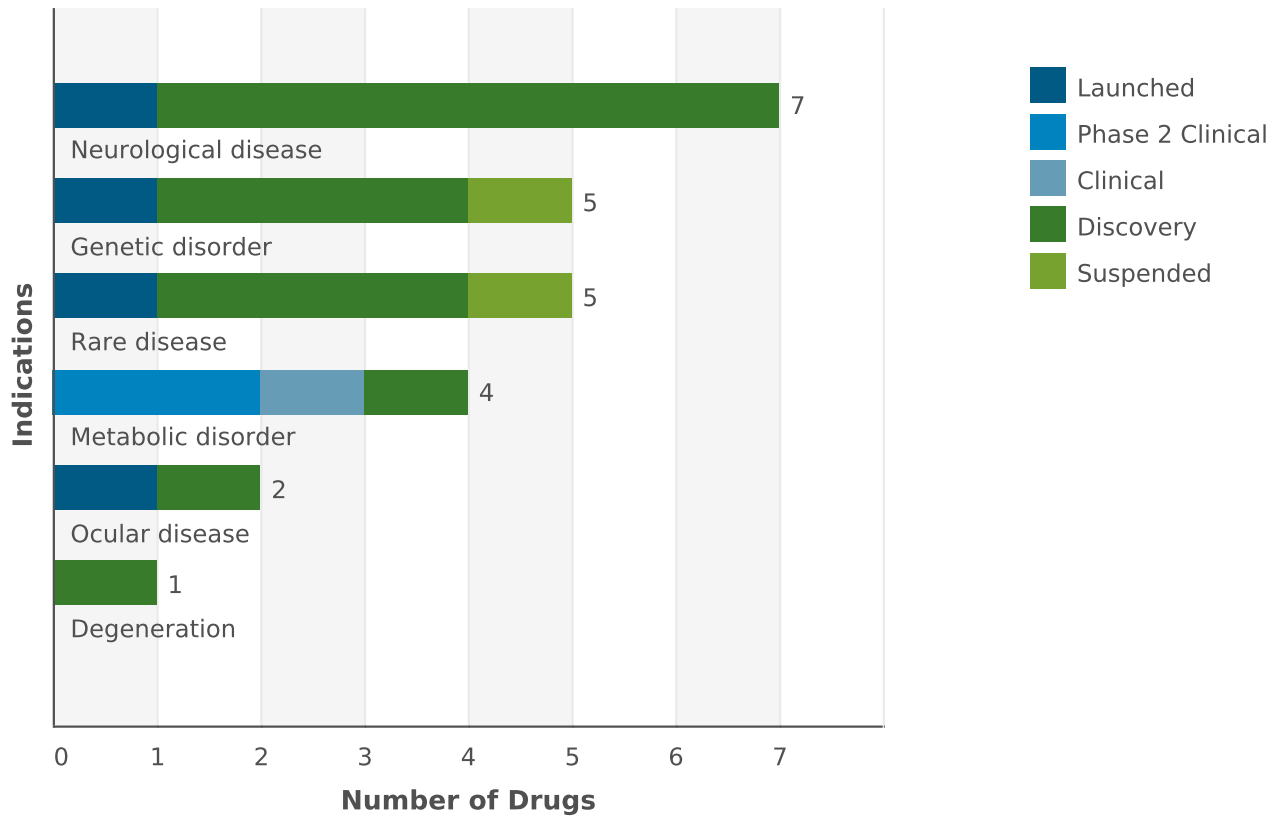
Return to Table of Contents

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



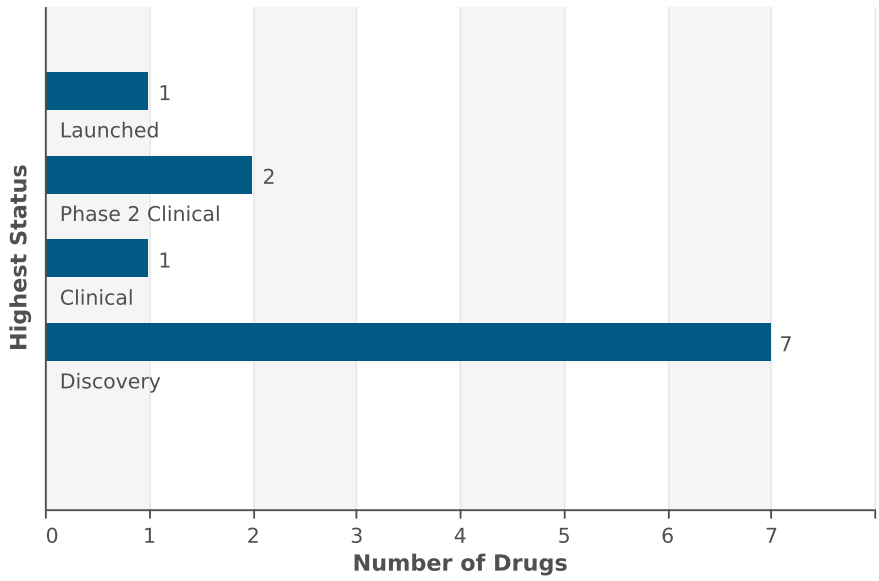
Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	7	6	13
Genetic disorder	5	4	9
Rare disease	5	3	8
Metabolic disorder	4	1	5
Ocular disease	2	1	3
Degeneration	1	1	2
Dermatological disease	0	1	1

Return to Table of Contents

Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Launched	1
Phase 2 Clinical	2
Clinical	1
Discovery	7
Discontinued	2
No Development Reported	5

Return to Table of Contents

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Drug - Development Services	0	0	2	0	2
Drug - Screening/Evaluation	0	0	1	0	1
Drug - Development/Commercialization License	3	0	1	0	5
Technology - Other Proprietary	0	0	1	0	1
Drug - Early Research/Development	1	0	3	0	4
Drug - Manufacturing/Supply	0	0	2	0	2
Patent - Exclusive Rights	0	0	2	0	2
Drug - Funding	6	0	0	0	6
Company - M&A (in whole or part)	0	3	0	3	3

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Rare disease	4	28
Genetic disorder	3	26
Neurological disease	3	26
Ocular disease	3	19
Metabolic disorder	4	11
Dermatological disease	2	4
Genitourinary disease	0	1

Trials by Phase

Phase	Ongoing	All
Phase 4	1	1
Phase 3	3	8
Phase 2	1	15
Phase 1	2	15

Phase Definitions

Return to Table of Contents

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
Degeneration	5	0	5
Andrology	0	1	1
Gynecology and obstetrics	0	1	1
Dermatological disease	8	1	9
Ulcer	4	0	4
Immune disorder	6	0	6
Psychiatric disorder	2	0	2
Musculoskeletal disease	3	0	3
Neoplasm	2	1	3
Ocular disease	20	0	20
Genetic disorder	33	0	33
Metabolic disorder	29	0	29
Neurological disease	40	1	41
Nutritional disorder	1	0	1
Respiratory disease	1	1	2
Injury	2	0	2
Inflammatory disease	5	0	5
Cardiovascular disease	3	0	3
Endocrine disease	1	1	2
Gastrointestinal disease	2	1	3
Genitourinary disease	1	1	2

Return to Table of Contents

Growth disorder	1	0	1
Rare disease	35	0	35

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

Return to Table of Contents

PRODUCT PORTFOLIO DRUG PIPELINE DETAIL

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

migalastat

migalastat SNAPSHOT

Drug Name	migalastat
Key Synonyms	migalastat;Amigal;migalastat hydrochloride;Galafold
Originator Company	Mount Sinai School of Medicine
Active Companies	Amicus Therapeutics Inc
Inactive Companies	GlaxoSmithKline plc;Mount Sinai School of Medicine;Shire Human Genetic Therapies Inc
Highest Status	Launched
Active Indications	Fabry disease
Target-based Actions	Alpha-galactosidase modulator
Other Actions	
Technologies	Capsule formulation;Oral formulation;Protein folding;Small molecule therapeutic
Last Change Date	13-Aug-2018

migalastat DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics, under license from Mount Sinai School of Medicine, has developed and launched migalastat (Galafold; AT-1001; HGT-3310; formerly Amigal), a pharmacological chaperone that enhances stability and folding of mutated alpha-galactosidase A (a-Gal A). The product is indicated in the EU for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (a-Gal A deficiency) and who have an amenable mutation. In Japan the product is indicated for the treatment of patients aged 16 years and older with a confirmed diagnosis of Fabry disease and who have an amenable mutation. In the US, the product as an oral capsule is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (GLA) variant based on in vitro assay data.

In May 2016, the EC approved the drug for Fabry disease in the EU and migalastat was immediately launched in Germany. In February 2017, the drug was launched in the UK. In May 2018, the drug was launched in Japan for Fabry disease. In August 2018, the US FDA granted Accelerated Approval for migalastat for Fabry disease; the product was immediately launched.

Amicus is also developing migalastat co-formulated with JR-051 (a biosimilar a-Gal A enzyme replacement therapy) for the potential treatment of all Fabry disease patients (independent of genetic mutation).

Return to Table of Contents

Amicus and former licensee GSK were also previously developing migalastat in combination with marketed enzyme replacement therapies (ERTs) for Fabry disease; in October 2012, a phase II trial of migalastat in combination with the marketed ERTs Fabrazyme and Replagal was completed. In February 2013, Amicus and GSK were still considering further development of migalastat co-administered with marketed ERTs ; however, by May 2013, the companies were focusing on co-formulating migalastat with their proprietary investigational ERT, JR-051, in-licensed from JCR Pharmaceuticals, . GSK returned rights in November 2013.

migalastat DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

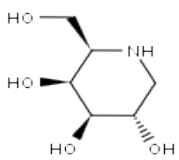
Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Fabry disease	EU	Launched	08-Mar-2017
Amicus Therapeutics Inc	Fabry disease	France	Launched	02-May-2017
Amicus Therapeutics Inc	Fabry disease	Germany	Launched	30-May-2016
Amicus Therapeutics Inc	Fabry disease	Italy	Launched	08-Mar-2017
Amicus Therapeutics Inc	Fabry disease	Japan	Launched	30-May-2018
Amicus Therapeutics Inc	Fabry disease	Spain	Launched	17-Jan-2018
Amicus Therapeutics Inc	Fabry disease	Switzerland	Launched	31-Mar-2018
Amicus Therapeutics Inc	Fabry disease	UK	Launched	27-Feb-2017
Amicus Therapeutics Inc	Fabry disease	US	Launched	10-Aug-2018
Amicus Therapeutics Inc	Fabry disease	Australia	Registered	15-Aug-2017
Amicus Therapeutics Inc	Fabry disease	Canada	Registered	14-Sep-2017
Amicus Therapeutics Inc	Fabry disease	Israel	Registered	11-Jul-2017
Amicus Therapeutics Inc	Fabry disease	Liechtenstein	Registered	30-May-2016
Amicus Therapeutics Inc	Fabry disease	South Korea	Registered	20-Dec-2017
Amicus Therapeutics Inc	Fabry disease	Iceland	Pre-registration	30-May-2016
Amicus Therapeutics Inc	Fabry disease	Norway	Pre-registration	30-May-2016
Amicus Therapeutics Inc	Fabry disease	Taiwan	Pre-registration	22-Mar-2018
Amicus Therapeutics Inc	Fabry disease	Argentina	Phase 3 Clinical	04-Nov-2011
Amicus Therapeutics Inc	Fabry disease	Egypt	Phase 3 Clinical	15-Dec-2010
Amicus Therapeutics Inc	Fabry disease	South Africa	Phase 3 Clinical	15-Dec-2010
Amicus Therapeutics Inc	Fabry disease	South America	Phase 3 Clinical	15-Dec-2010

Return to Table of Contents

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Fabry disease	Turkey	Phase 3 Clinical	24-Oct-2011
Shire Human Genetic Therapies Inc	Fabry disease	US	Discontinued	29-Oct-2009
GlaxoSmithKline plc	Fabry disease	Australia	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Canada	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Egypt	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Europe	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Israel	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Japan	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	South Africa	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	South America	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Taiwan	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	Turkey	Outlicensed	20-Nov-2013
GlaxoSmithKline plc	Fabry disease	US	Outlicensed	20-Nov-2013
Mount Sinai School of Medicine	Fabry disease	US	Outlicensed	30-Apr-2002

migalastat CHEMICAL STRUCTURES

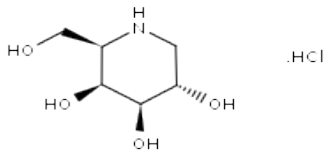
CAS Registry Number:	Confidence Level:
108147-54-2	2



Name	Type
migalastat	INN; USAN
HGT-3310	Research Code

Return to Table of Contents

CAS Registry Number:	Confidence Level:
75172-81-5	1



Name	Type
migalastat hydrochloride	USAN
Amigal	Trade Name
Galafold	Trade Name

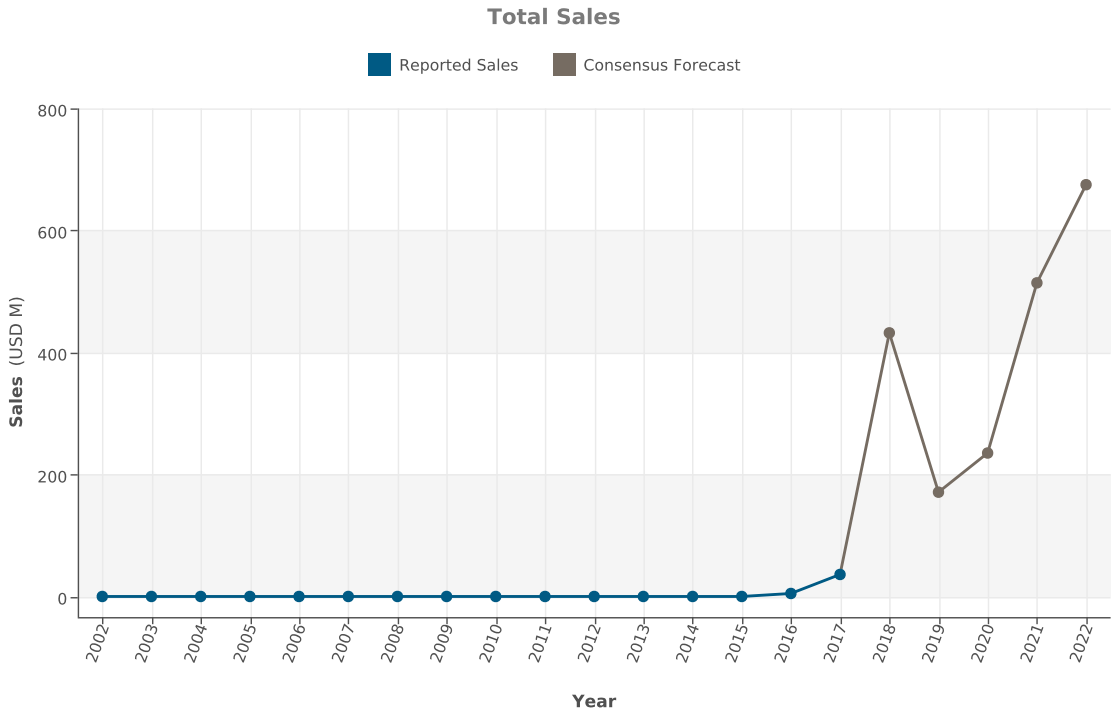
migalastat DRUG NAMES

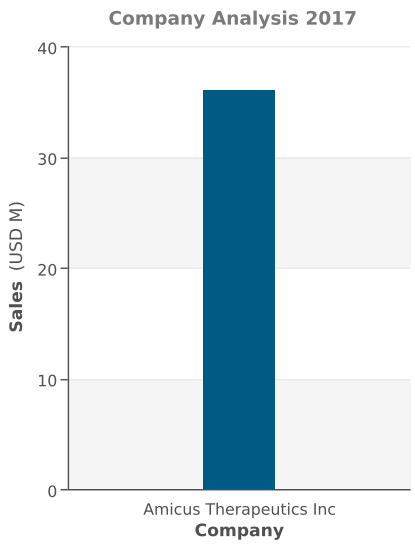
Names	Type
AT-1001 (Fabry disease), Amicus Therapeutics	
Amigal	Trade Name
GR-181413	Research Code
GR181413A	Research Code
Galafold	Trade Name
HGT-3310	Research Code
alpha-galactosidase A modulator (oral, Fabry disease), Amicus/ Shire Human Genetics Therapies	
migalastat	INN, USAN
migalastat hydrochloride	USAN

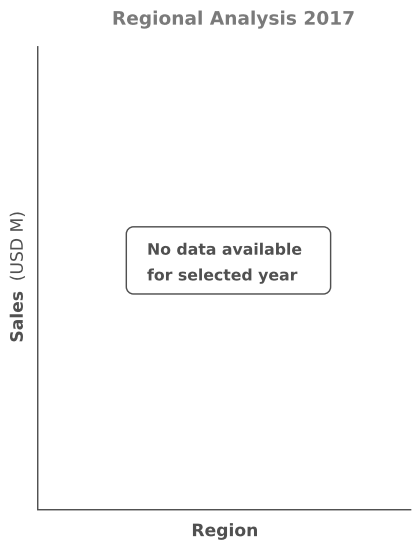
Return to Table of Contents

migalastat SALES AND FORECASTS

CHARTS







Return to Table of Contents

COMMENTARY

CONSENSUS SALES INFORMATION

Consensus forecast data, where available, are presented for Amicus.

REPORTED ANNUAL SALES

Sales for migalastat (Galafold) reported by Amicus for 2017 were $36.9 million, up from $5.0 million in 2016 [2011170].

REPORTED QUARTERLY SALES

Sales for migalastat (Galafold) reported by Amicus for 1Q 2017 were $4.2 million [1925953]. Sales for migalastat (Galafold) reported by Amicus for 2Q 2017 were $7.2 million [1951093]. Sales for migalastat (Galafold) reported by Amicus for 3Q 2017 were $10.9 million [1979825]. Sales for migalastat (Galafold) reported by Amicus for 4Q 2017 were $14.6 million [2011952]. Sales for migalastat (Galafold) reported by Amicus for 1Q 2018 were $16.7 million [2031737].

REGIONAL DEVELOPMENT AND MARKETING RIGHTS

By April 2002, Amicus had licensed exclusive worldwide patent rights from originator Mount Sinai School of Medicine to develop and commercialize various pharmacological chaperones; this agreement was presumed to include migalastat. The agreement is due to terminate at the time of expiry of the final patent in 2019, subject to any patent-term extensions. The license agreement between Amicus and Mount Sinai was amended in 2006 to expand the exclusive patent rights belonging to Amicus and in 2008 to consolidate previous amendments into one agreement and to clarify royalty and milestone payments [505431], [534745], [1173958]. In February 2012, Amicus reported that the agreement could be extended to 2024 if migalastat was developed for combination therapy, subject to the granting of any extension to the terms of the licensed patents [1268930].

In November 2007, Amicus and Shire Human Genetic Therapies agreed to jointly codevelop migalastat. Amicus would retain rights to commercialize the product in the US and Shire would obtain rights to commercialize the product in territories outside the US. Amicus would lead worldwide development operations until the end of phase II clinical trials and the companies would share responsibility for phase III clinical trial execution [849101]. In October 2009, the companies terminated their collaboration and Amicus regained exclusive worldwide rights to the drug [1053053], [1173958].

In October 2010, Amicus entered an agreement with GlaxoSmithKline (GSK) whereby GSK was exclusively licensed to develop, manufacture and commercialize migalastat worldwide; development costs would be shared 50/50 in 2011; from 2012 and beyond, development costs would be shared 25/75 between Amicus and GSK, respectively. Under the agreement, GSK would be responsible for marketing and reimbursement applications. At the end of 2011, GSK assumed responsibility for all chemistry, manufacturing and controls (CMC) [1143012], [1268930]. In July 2012, Amicus gained commercial rights for the product in the US. Commencing in 2013, Amicus and GSK would be responsible for 40 and 60% of the development costs, respectively [1309169]. In November 2013, Amicus and GSK amended their agreement whereby Amicus regained worldwide rights to develop and commercialize the drug [1501723]. In March 2014, Amicus stated that under the revised agreement, GSK was no longer responsible for costs related to the drug as of January 1, 2014 [1531081].

migalastat 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

Return to Table of Contents

Fabry disease											
1	1	2	6	0	8	0	3	0	0	3	19
Renal disease											
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											
1	1	2	6	0	8	0	4	0	0	3	19

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)

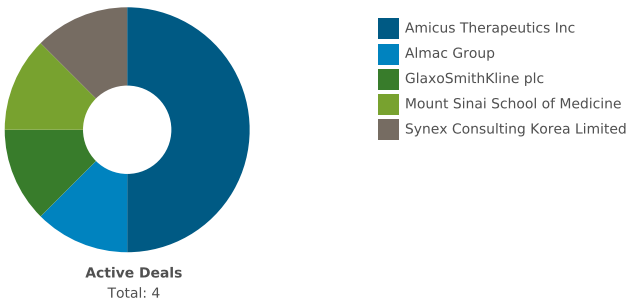
Phase 1 Clinical
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

Return to Table of Contents

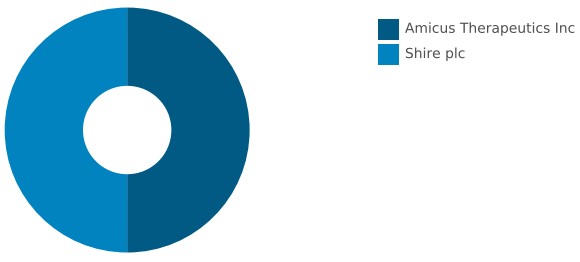
migalastat DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 4



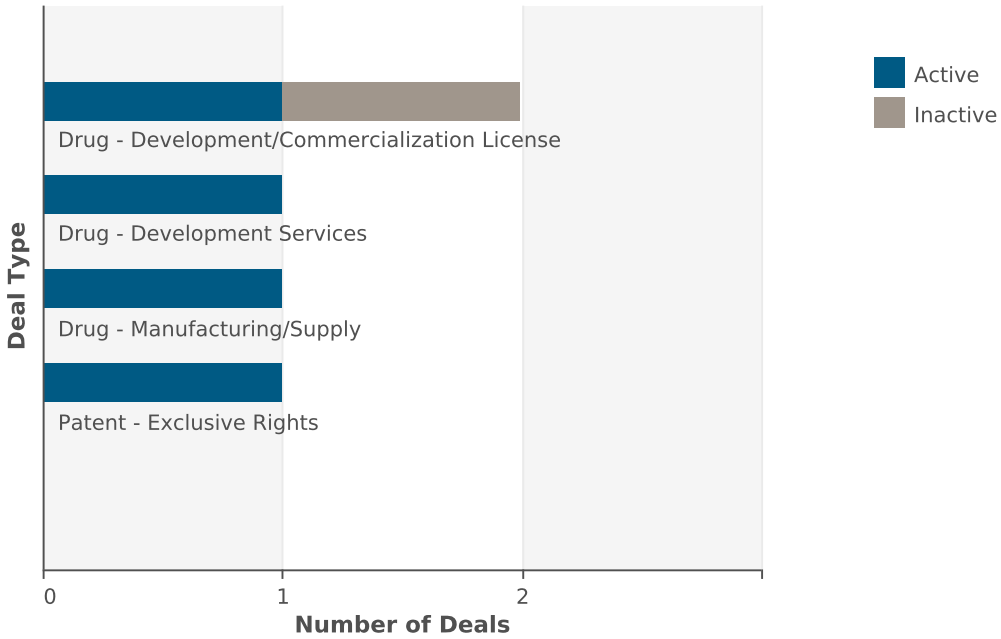
Inactive Deals
Total: 1

Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	1	1	3	0	5
Shire plc	0	0	0	1	1
Almac Group	1	0	0	0	1
Mount Sinai School of Medicine	1	0	0	0	1
GlaxoSmithKline plc	0	0	1	0	1
Synex Consulting Korea Limited	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



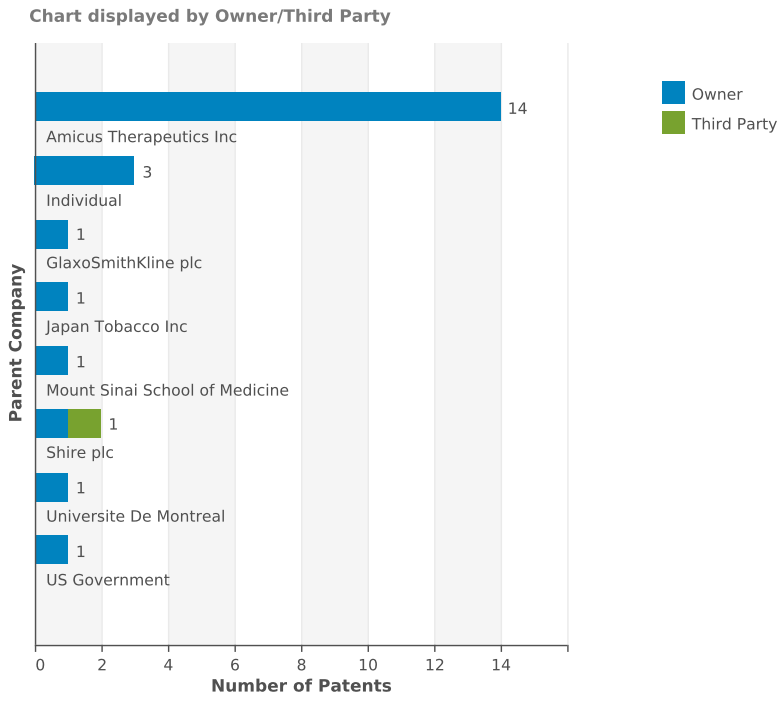
Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development/Commercialization License	1	1	2
Drug - Development Services	1	0	1
Drug - Manufacturing/Supply	1	0	1
Patent - Exclusive Rights	1	0	1

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

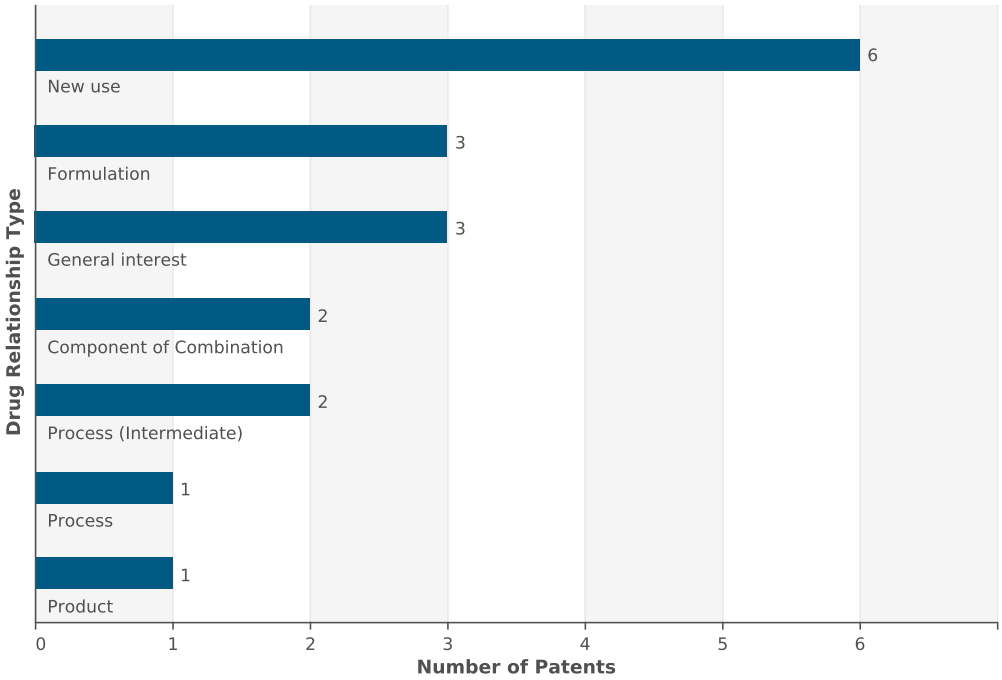


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	14	0	14
Individual	3	0	3
Shire plc	1	1	1
GlaxoSmithKline plc	1	0	1
Mount Sinai School of Medicine	1	0	1
US Government	1	0	1
Japan Tobacco Inc	1	0	1
Universite De Montreal	1	0	1

Return to Table of Contents

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
New use	6
General interest	3
Formulation	3
Component of Combination	2
Process (Intermediate)	2
Product	1
Process	1

Return to Table of Contents

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics SNAPSHOT

Drug Name	ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Pompes disease
Target-based Actions	Alpha-glucosidase stimulator
Other Actions	
Technologies	Biological therapeutic;Drug combination;Recombinant enzyme;Subcutaneous formulation
Last Change Date	27-Jun-2018

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics is developing AT-GAA, a non-fixed dose combination of ATB-200, a next-generation recombinant human acid alpha-glucosidase (rhGAA) enzyme replacement therapy, as a biobetter version of alglucosidase alfa, co-administered with the molecular chaperone miglustat (AT-2221; N-butyl-deoxynojirimycin) for the potential sc injection treatment of Pompe disease,. In December 2015, a phase I/II trial was initiated in patients with Pompe disease.

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics DEVELOPMENT STATUS

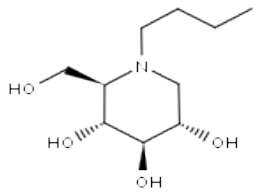
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Pompes disease	Australia	Phase 2 Clinical	27-Sep-2016
Amicus Therapeutics Inc	Pompes disease	Europe	Phase 2 Clinical	25-Aug-2016
Amicus Therapeutics Inc	Pompes disease	US	Phase 2 Clinical	22-Dec-2015

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
72599-27-0	1



Name	Type
miglustat	INN
Brazaves	Trade Name
Zavesca	Trade Name
Vevesca	Trade Name
Ze Wei Ke	Trade Name
OGT-918	Research Code
AT-2221	Research Code
ACT-149071	Research Code
SC-48334	Research Code
NB-DNJ	
N-butyldeoxynojirimycin	

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics DRUG NAMES

Names	Type
AT-GAA	
ATB-200 + AT-2221 (non-FDC, Pompe disease), Amicus	
ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics	
miglustat plus proprietary enzyme replacement therapy (sc, Pompe disease), Amicus Therapeutics	
recombinant human acid alpha-glucosidase + miglustat (non-FDC, Pompe disease), Amicus Therapeutics	

Return to Table of Contents

ATB-200 + miglustat (non-FDC, Pompe disease), Amicus Therapeutics 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
Pompes disease											
0	0	0	0	0	0	1	2	0	0	1	3

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	2	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)

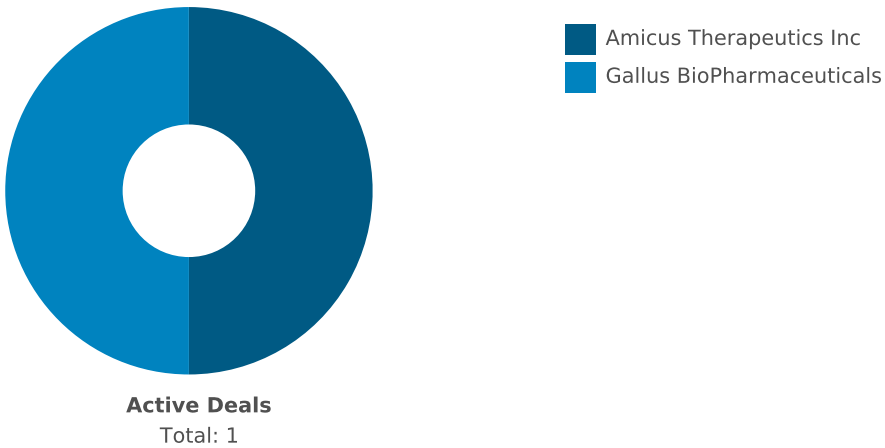
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

Return to Table of Contents

DEALS

Deals by Parent Company Chart

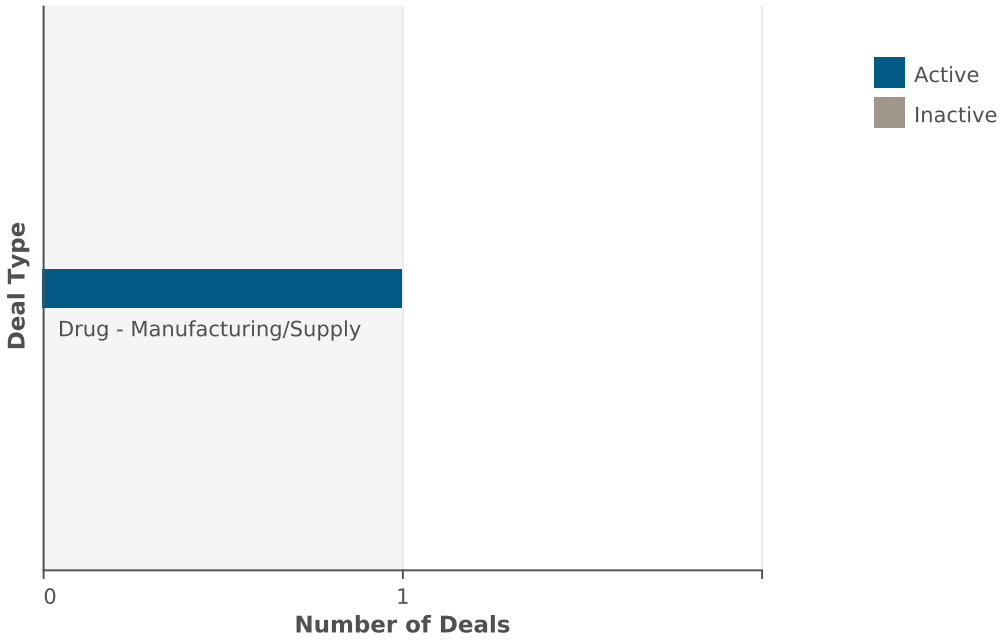


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Gallus BioPharmaceuticals	1	0	0	0	1
Amicus Therapeutics Inc	0	0	1	0	1

Return to Table of Contents

Deals by Type Chart



Deals by Type Table

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

Return to Table of Contents

duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics

duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics SNAPSHOT

Drug Name	duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Pompes disease
Target-based Actions	Alpha-glucosidase modulator
Other Actions	
Technologies	Biological therapeutic;Drug combination;Infusion;Oral formulation;Recombinant enzyme
Last Change Date	23-Oct-2015

duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics is developing AT-2220-ERT, a combination of the molecular chaperone duvoglustat hydrochloride (orally) plus enzyme replacement therapy (ERT) as an infusion, for the potential treatment of Pompe's disease. In October 2011, a phase II trial was initiated ; in January 2013, positive data from the trial were reported. At that time, the company planned to initiate a repeat-dose clinical study in the third quarter of 2013. In September 2015, a phase III trial was planned to initiate in 2016.

Amicus was developing duvoglustat as a monotherapy for Pompe's disease.

Amicus is also developing an iv formulation of AT-2220-ERT and a miglustat/next-generation human recombinant alglucosidase alfa (rhGAA) enzyme replacement therapy combination product for potential sc injection.

duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

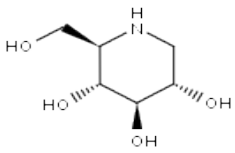
Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Pompes disease	Canada	Phase 2 Clinical	04-Oct-2011
Amicus Therapeutics Inc	Pompes disease	France	Phase 2 Clinical	04-Oct-2011
Amicus Therapeutics Inc	Pompes disease	UK	Phase 2 Clinical	04-Oct-2011

Return to Table of Contents

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Pompes disease	US	Phase 2 Clinical	04-Oct-2011

duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
19130-96-2	2



Name	Type
duvoglustat	INN; USAN
1-deoxynojirimycin	
deoxynojirimycin	

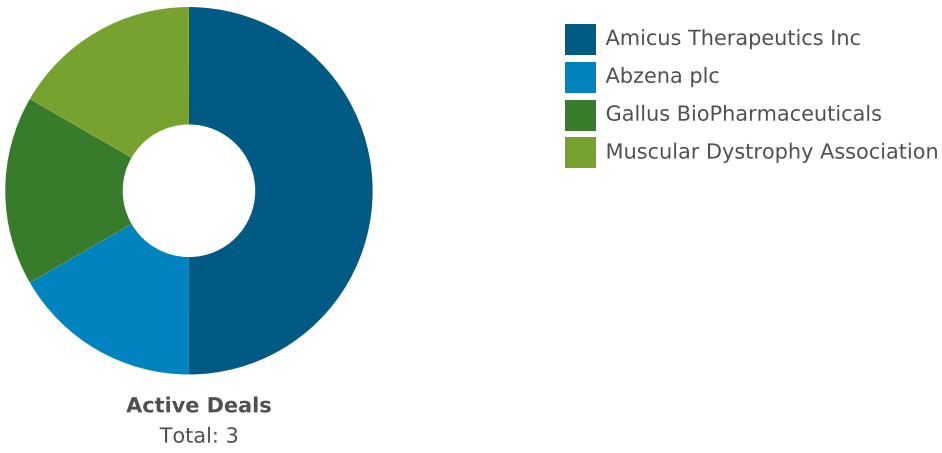
duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics DRUG NAMES

Names	Type
1-deoxynojirimycin	
1-deoxynojirimycin + enzyme replacement therapy (oral, Pompe disease), Amicus Therapeutics	
AT-2220 + enzyme replacement therapy	
AT-2220-ERT	Research Code
Pompe disease enzyme replacement therapy, Amicus	
duvoglustat hydrochloride plus enzyme replacement therapy (Pompe disease), Amicus Therapeutics	

Return to Table of Contents

DEALS

Deals by Parent Company Chart

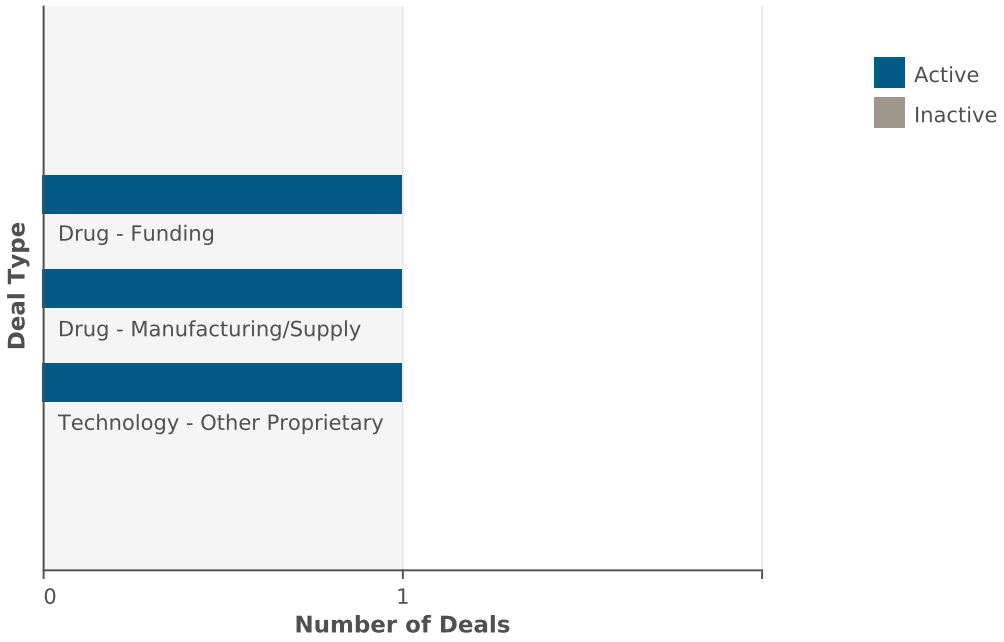


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	1	0	2	0	3
Muscular Dystrophy Association	0	0	1	0	1
Abzena plc	1	0	0	0	1
Gallus BioPharmaceuticals	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



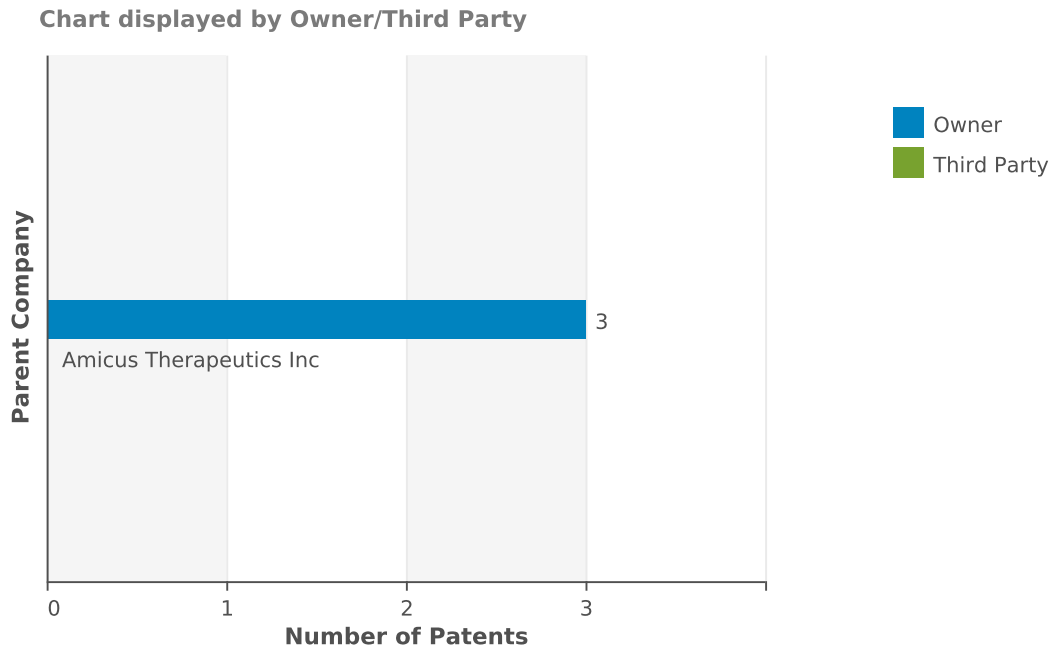
Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Manufacturing/Supply	1	0	1
Drug - Funding	1	0	1
Technology - Other Proprietary	1	0	1

Return to Table of Contents

PATENTS

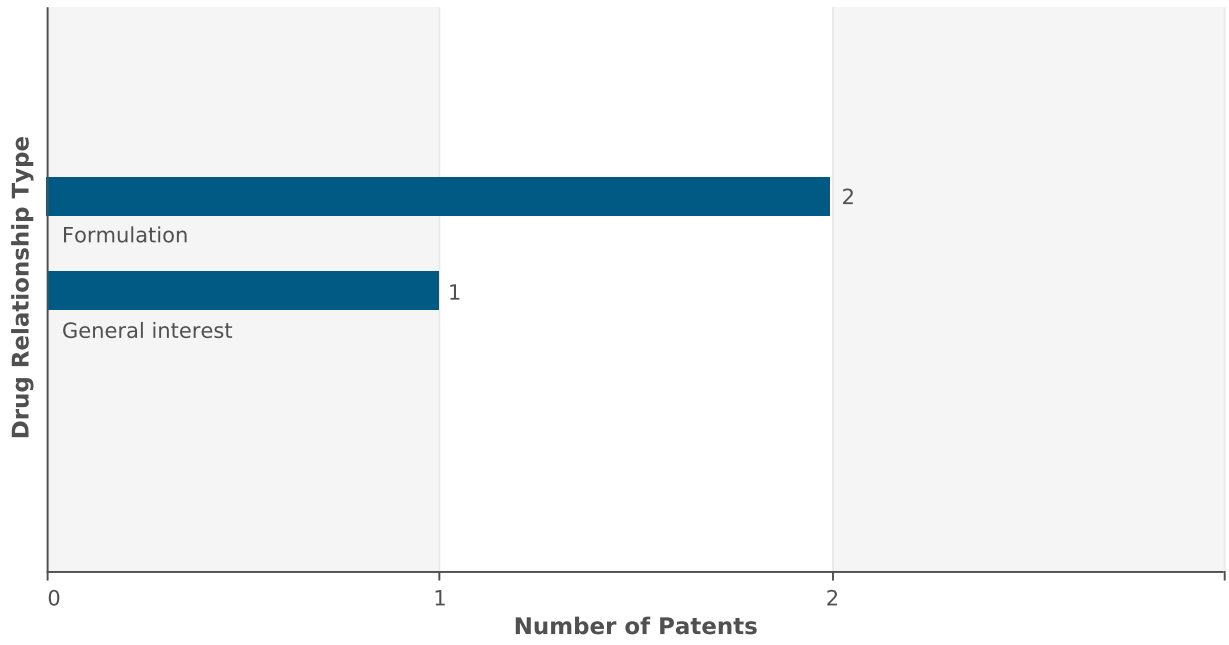
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	3	0	3

Patents by Drug Relationship Type Chart



Return to Table of Contents

Patents by Drug Relationship Type Table

Drug Relationship	Total
Formulation	2
General interest	1

Return to Table of Contents

duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics

duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics SNAPSHOT

Drug Name	duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Clinical
Active Indications	Pompes disease
Target-based Actions	Alpha-glucosidase modulator
Other Actions	
Technologies	Biological therapeutic;Drug combination;Intravenous formulation;Recombinant enzyme
Last Change Date	29-Oct-2015

duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics is developing AT-2220-IV, a combination of the molecular chaperone duvoglustat hydrochloride plus enzyme replacement therapy (ERT), for the potential iv bolus treatment of Pompe's disease. In March 2014, a phase I study was expected to begin the first half of 2014.

Amicus is also developing an oral formulation of AT-2220-ERT and a miglustat/next-generation human recombinant alglucosidase alfa (rhGAA) enzyme replacement therapy combination product for potential sc injection.

duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics DEVELOPMENT STATUS

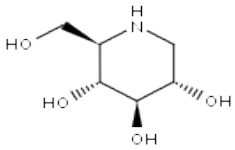
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Pompes disease	US	Clinical	14-Feb-2013

duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
19130-96-2	2



Name	Type
duvoglustat	INN; USAN
1-deoxynojirimycin	
deoxynojirimycin	

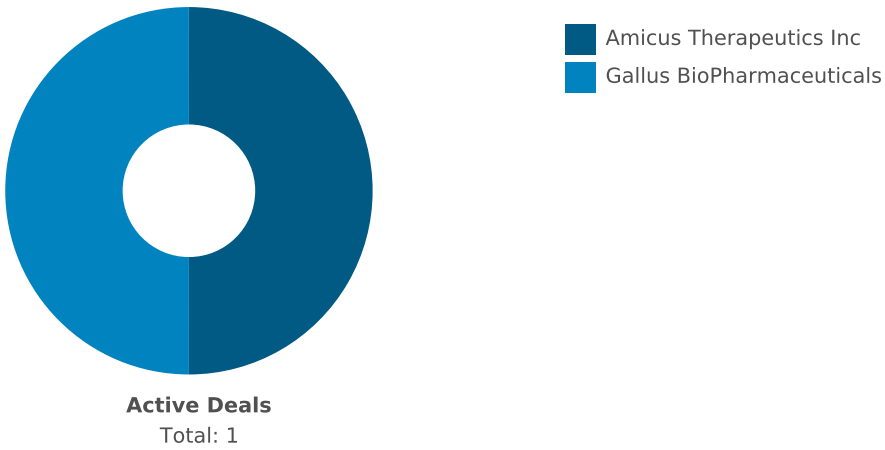
duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics DRUG NAMES

Names	Type
AT-2220-ERT (iv, Pompe disease), Amicus	
AT2220-IV	Research Code
Pompe disease enzyme replacement therapy (iv), Amicus	
duvoglustat hydrochloride plus enzyme replacement therapy (iv, Pompe disease), Amicus Therapeutics	

Return to Table of Contents

DEALS

Deals by Parent Company Chart

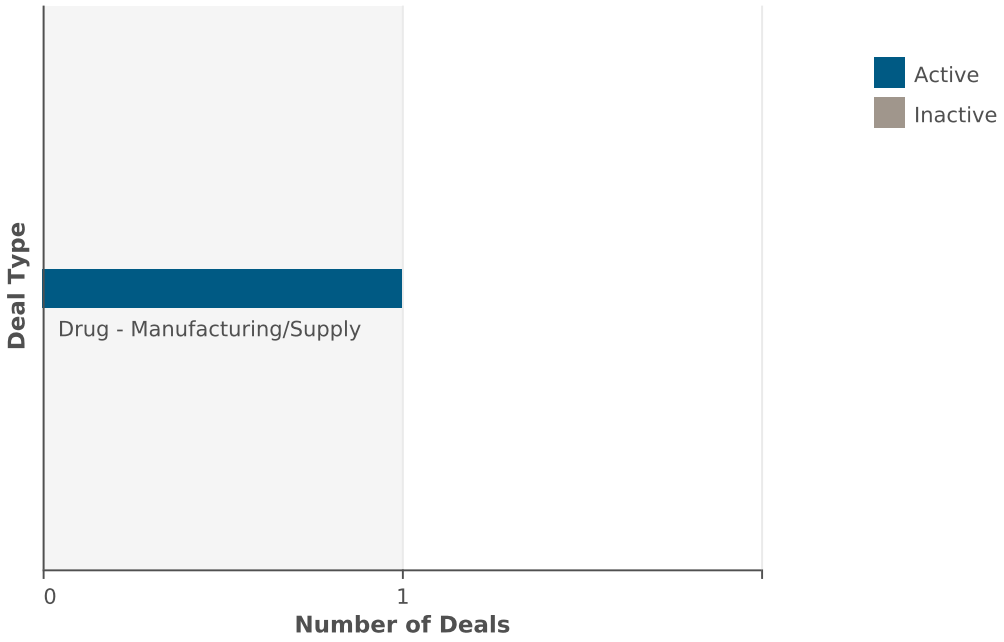


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Gallus BioPharmaceuticals	1	0	0	0	1
Amicus Therapeutics Inc	0	0	1	0	1

Return to Table of Contents

Deals by Type Chart



Deals by Type Table

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

Return to Table of Contents

protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics

protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics SNAPSHOT

Drug Name	protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics
Key Synonyms	
Originator Company	MiaMed Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	MiaMed Inc
Highest Status	Discovery
Active Indications	Neurological disease
Target-based Actions	Serine threonine kinase 9 modulator
Other Actions	
Technologies	Small molecule therapeutic
Last Change Date	20-Dec-2017

protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics, following the acquisition of MiaMed, is investigating a protein replacement therapy, for the potential treatment of cyclin-dependent kinase-like 5 (CDKL5) deficiency. In July 2016, preclinical development was ongoing.

protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Neurological disease	US	Discovery	06-Jul-2016
MiaMed Inc	Neurological disease	US	Outlicensed	06-Jul-2016

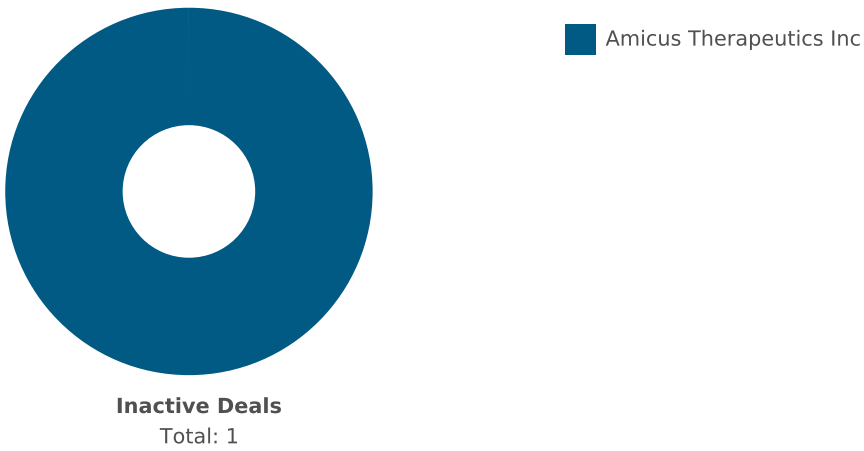
protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics DRUG NAMES

Names	Type
protein replacement therapy (CDKL5 deficiency), Amicus Therapeutics	
protein replacement therapy (CDKL5 deficiency), MiaMed	

Return to Table of Contents

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	0	1	0	1	1

Return to Table of Contents

Deals by Type Chart

Deals by Type Table

Deal Type	Active	Inactive	Total
Company - M&A (in whole or part)	0	1	1

Return to Table of Contents

ATB-200

ATB-200 SNAPSHOT

Drug Name	ATB-200
Key Synonyms	
Originator Company	Callidus Biopharma Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	Callidus Biopharma Inc
Highest Status	Discovery
Active Indications	Pompes disease
Target-based Actions	Alpha-glucosidase stimulator
Other Actions	
Technologies	Biological therapeutic;Intravenous formulation;Recombinant enzyme
Last Change Date	08-May-2018

ATB-200 DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics, following the acquisition of Callidus Biopharma, is investigating ATB-200, a recombinant human acid alpha-glucosidase (rhGAA) enzyme replacement therapy (ERT), for the potential iv treatment of lysosomal storage disorders, including Pompe disease,. In February 2017, preclinical data were presented .

The company is also investigating a ATB-200 co-administered with miglustat (AT-2221) for Pompe disease.

ATB-200 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Pompes disease	Australia	Discovery	04-Feb-2016
Amicus Therapeutics Inc	Pompes disease	Germany	Discovery	04-Feb-2016
Amicus Therapeutics Inc	Pompes disease	Netherlands	Discovery	04-Feb-2016
Amicus Therapeutics Inc	Pompes disease	UK	Discovery	04-Feb-2016
Amicus Therapeutics Inc	Pompes disease	US	Discovery	20-Nov-2013

Return to Table of Contents

ATB-200 DRUG NAMES

Names	Type
ATB-200	Research Code
enzyme replacement therapy (lysosome storage disorder) Callidus Biopharm	
enzyme replacement therapy (lysosome storage disorder), Amicus	
recombinant human acid-alpha glucosidase (iv, Pompe disease), Amicus	
rhGAA (iv, Pompe disease), Amicus	

ATB-200 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
Pompes disease											
0	0	0	0	0	0	1	2	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	0	0	1	2	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)

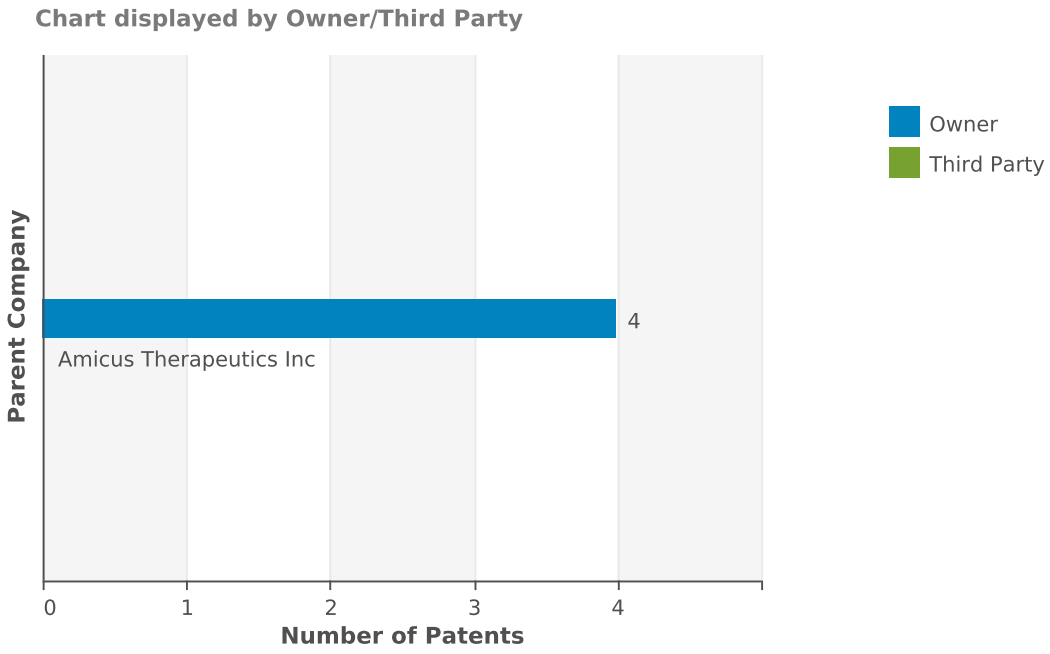
Phase 1 Clinical
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

Return to Table of Contents

ATB-200 DEALS AND PATENTS

PATENTS

Patents by Parent Company Chart

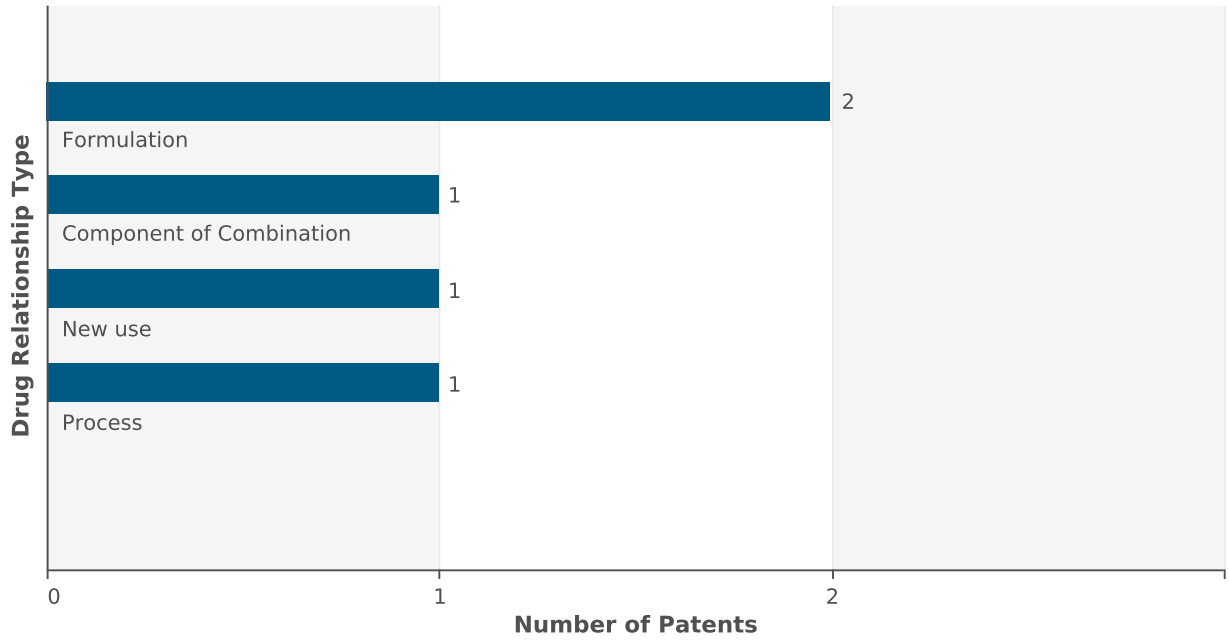


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	4	0	4

Return to Table of Contents

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Formulation	2
Component of Combination	1
New use	1
Process	1

Return to Table of Contents

AT-3375

AT-3375 SNAPSHOT

Drug Name	AT-3375
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Gaucher disease;Parkinsons disease
Target-based Actions	Glucosylceramidase stimulator
Other Actions	Antiparkinsonian
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	13-Mar-2017

AT-3375 DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics, with support from the Michael J Fox Foundation, is investigating AT-3375, the lead from a series of pharmacological chaperone molecules based on afegostat, which bind to glucocerebrosidase (GCase) and stimulate proper folding and trafficking of the enzyme, for the potential treatment of Parkinson's disease and Gaucher disease. By December 2009, lead compounds with efficacy in animal models had been identified. In January 2012, IND-enabling studies were underway. In May 2015, development was ongoing.

This program is based on Amicus' oral molecular chaperone afegostat (AT-2101), which was in development for Gaucher disease.

AT-3375 DEVELOPMENT STATUS

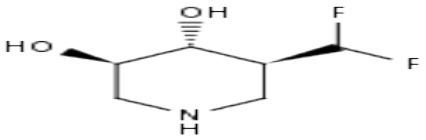
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Gaucher disease	US	Discovery	09-Feb-2012
Amicus Therapeutics Inc	Parkinsons disease	US	Discovery	20-Aug-2007

AT-3375 CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
	5



AT-3375 DRUG NAMES

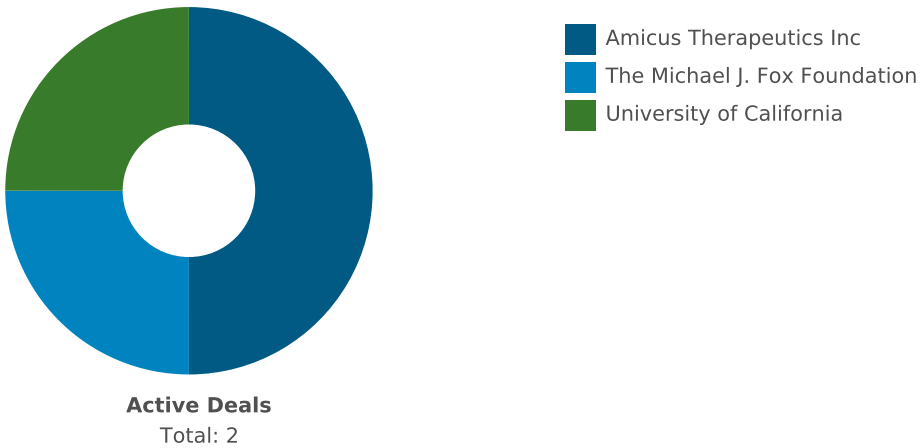
Names	Type
AT-3375	Research Code
glucocerebrosidase modulators (Parkinson's disease), Amicus	
pharmacological chaperones (oral, Parkinson's disease), Amicus	

Return to Table of Contents

AT-3375 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

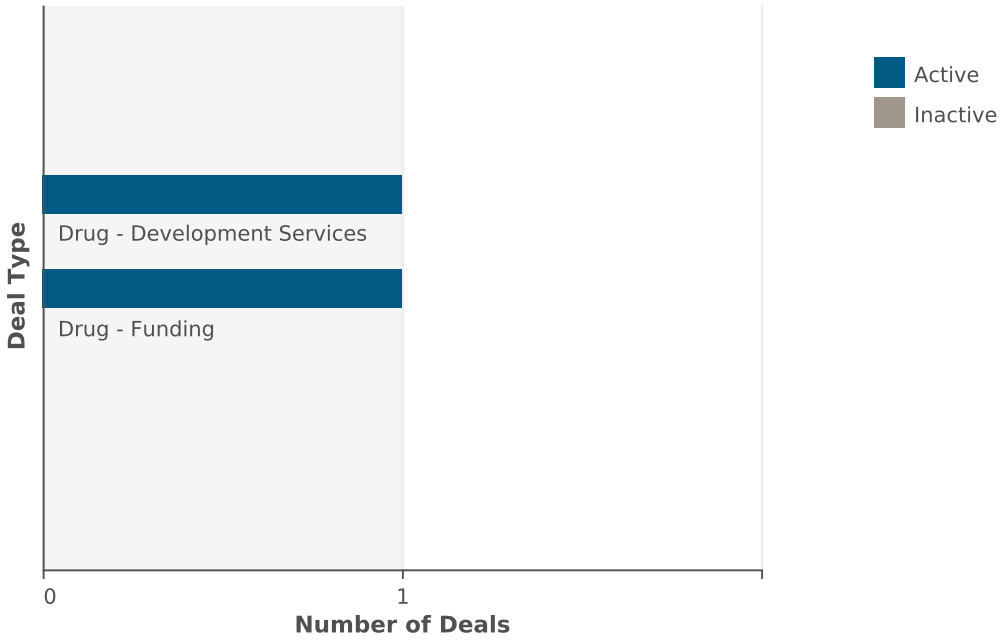


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	1	0	1	0	2
The Michael J. Fox Foundation	0	0	1	0	1
University of California	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



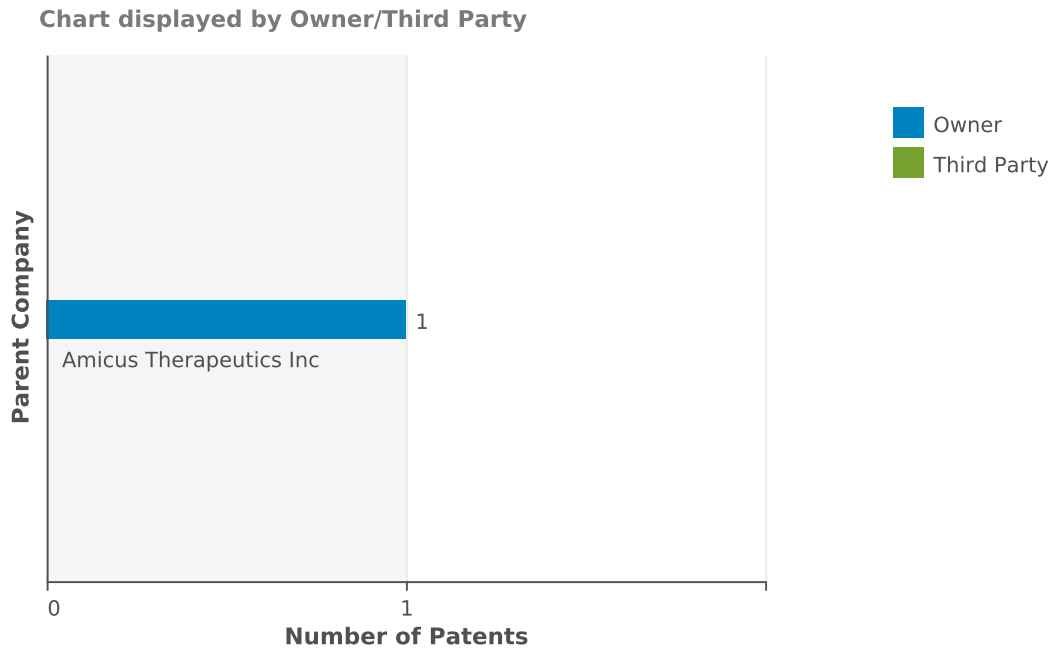
Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Development Services	1	0	1
Drug - Funding	1	0	1

Return to Table of Contents

PATENTS

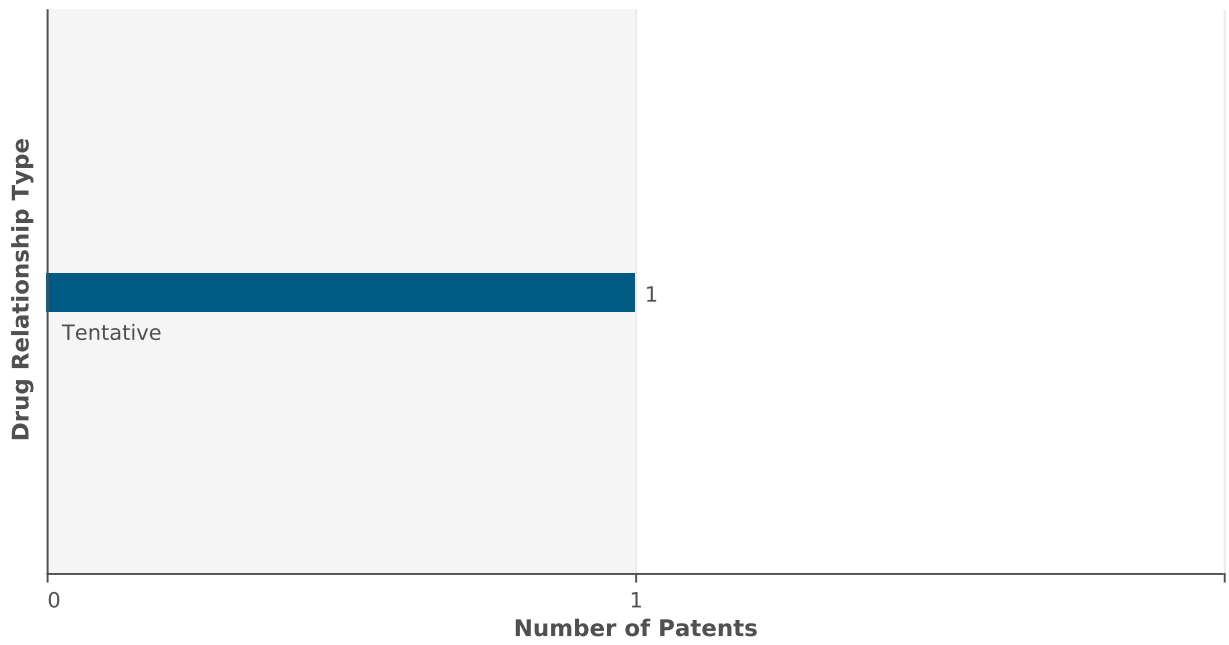
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	1	0	1

Patents by Drug Relationship Type Chart



Return to Table of Contents

Patents by Drug Relationship Type Table

Drug Relationship	Total
Tentative	1

Return to Table of Contents

OT-1001

OT-1001 SNAPSHOT

Drug Name	OT-1001
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc;Orphic Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Alzheimers disease;GM2 gangliosidosis
Target-based Actions	Amyloid protein deposition inhibitor;Beta-N-acetylhexosaminidase stimulator
Other Actions	Neuroprotectant
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	07-Nov-2015

OT-1001 DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics presumed to be in collaboration with Orphi is investigating OT-1001, a small-molecule pharmacological chaperones that bind to and activate the lysosomal enzyme beta-hexosaminidase for the potential oral treatment of Alzheimer's disease (AD), including sporadic AD,. Orphi is also investigating the drug for the potential treatment of GM2 gangliosidosis including Tay-Sachs disease (TSD) and sandhoff disease. In January 2010, preclinical studies were ongoing ; in March 2015, preclinical data were reported. In December 2016, development was ongoing.

Amicus is also investigating pharmacological chaperones that target presenilin 1 for the treatment of familial AD.

OT-1001 DEVELOPMENT STATUS

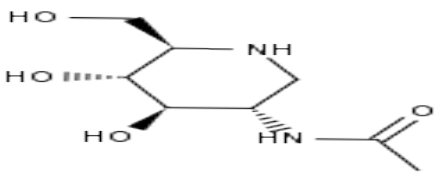
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Alzheimers disease	US	Discovery	11-Jan-2010
Orphic Therapeutics Inc	Alzheimers disease	US	Discovery	28-Oct-2014
Orphic Therapeutics Inc	GM2 gangliosidosis	US	Discovery	16-Mar-2015

OT-1001 CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
	5



Name	Type
DNJNAc	
AdDNJ	

OT-1001 DRUG NAMES

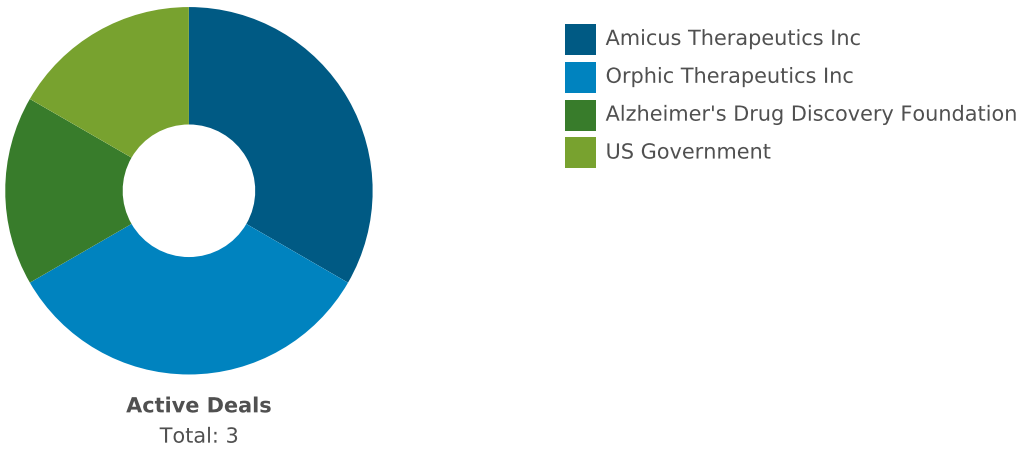
Names	Type
AdDNJ	
OT-1001	Research Code
beta-hexosaminidase stimulators (Alzheimer's disease), Amicus	
pharmacological chaperones (oral, Alzheimer's disease), Amicus	

Return to Table of Contents

OT-1001 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

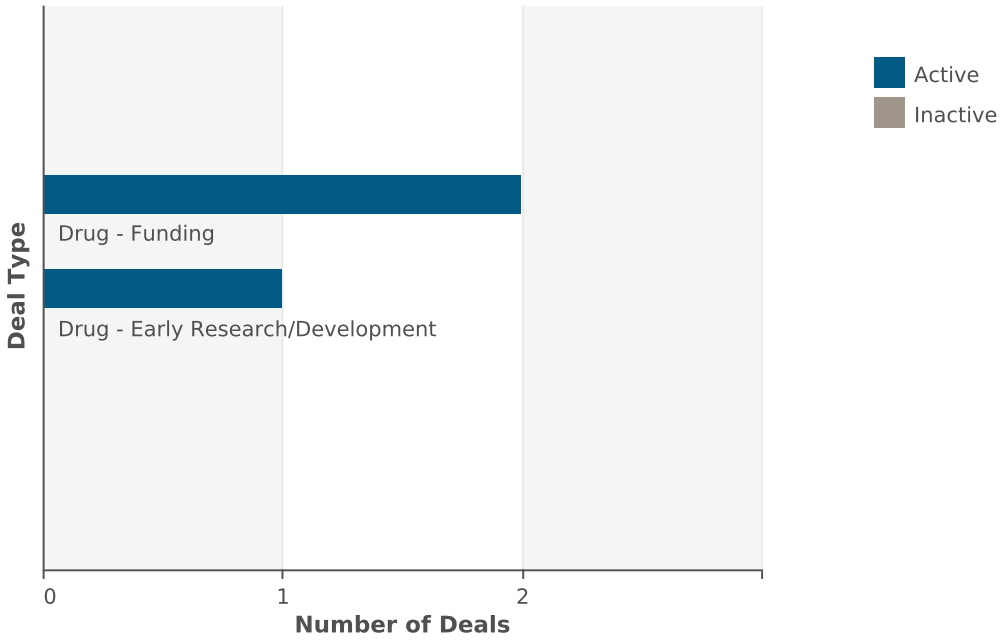


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Orphic Therapeutics Inc	1	0	1	0	2
Amicus Therapeutics Inc	2	0	0	0	2
US Government	0	0	1	0	1
Alzheimer's Drug Discovery Foundation	0	0	1	0	1

Return to Table of Contents

Deals by Type Chart



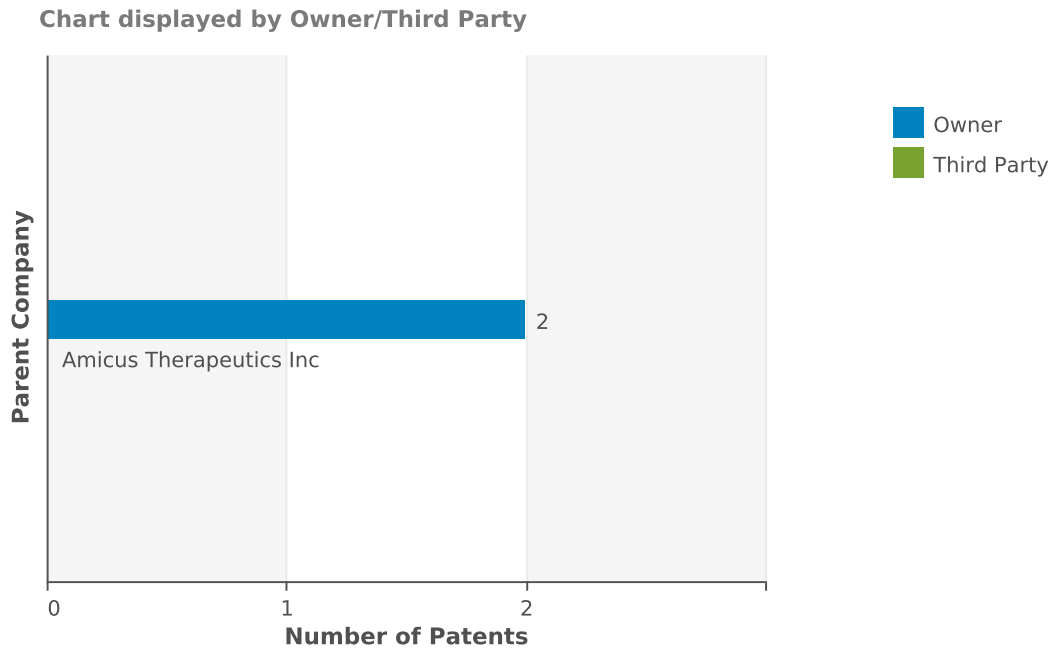
Deals by Type Table

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

Return to Table of Contents

PATENTS

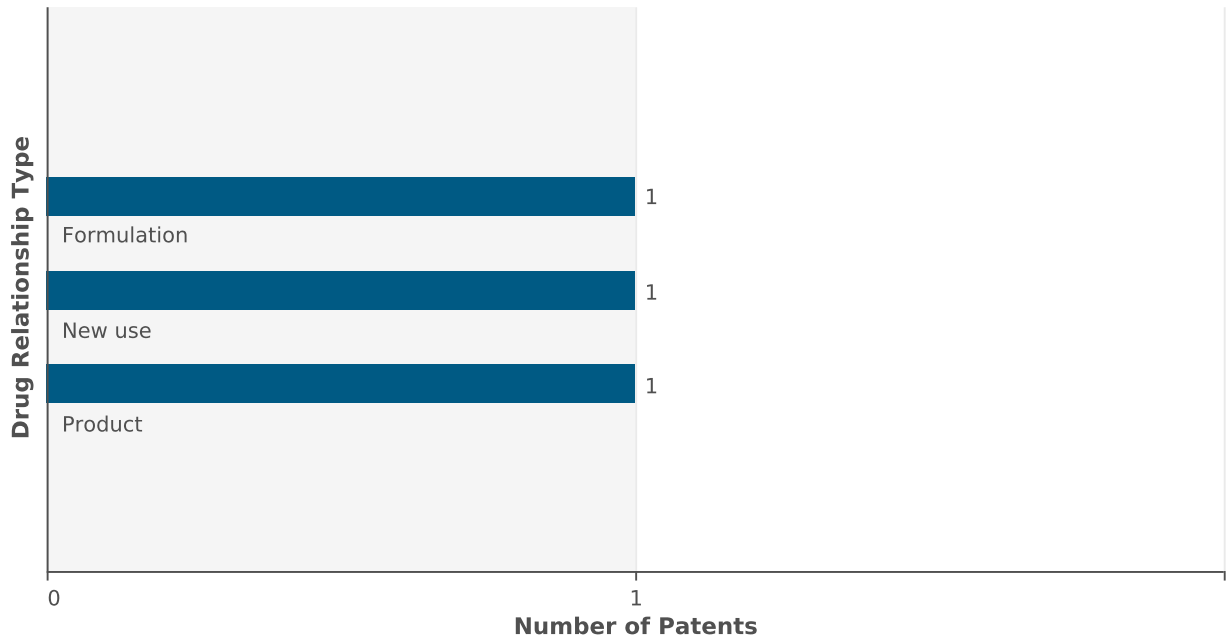
Patents by Parent Company Chart



Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	2	0	2

Patents by Drug Relationship Type Chart



Return to Table of Contents

Patents by Drug Relationship Type Table

Drug Relationship	Total
Formulation	1
Product	1
New use	1

Return to Table of Contents

afegostat

afegostat SNAPSHOT

Drug Name	afegostat
Key Synonyms	Plicera;afegostat tartrate;afegostat
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc;Shire Human Genetic Therapies Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Gaucher disease;Parkinsons disease
Target-based Actions	Glucosylceramidase stimulator;Chaperonin stimulator
Other Actions	
Technologies	Oral formulation;Small molecule therapeutic
Last Change Date	09-Oct-2014

afegostat DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics is investigating afegostat (isofagomine; Plicera; HGT-3410; AT-2101; structure shown), an orally available pharmacological chaperone molecule, which binds to glucocerebrosidase (Gba) and stimulates proper folding and trafficking of the enzyme, for the potential treatment of Parkinsons disease (PD),. In March 2012, the drug was listed on company's website.In October 2014, positive preclinical data were published.

Amicus was previously developing the drug for Gaucher disease in collaboration with Shire Human Genetic Therapies. In March 2007, the first of two planned phase II trials began. However, in October 2009, negative phase II data were reported. At that time, Amicus did not plan to conduct phase III trials of the drug. The phase II data were to be further analyzed, but it was presumed that development was suspended. In May 2010, Amicus was evaluating options for the clinical development of afegostat in combination with enzyme replacement therapy for Gaucher disease.

Amicus is investigating a combination therapy of afegostat and enzyme replacement therapy for Gaucher disease.

afegostat DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

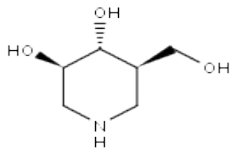
Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Parkinsons disease	US	Discovery	16-Feb-2010
Amicus Therapeutics Inc	Gaucher disease	Paraguay	Suspended	05-Oct-2009

Return to Table of Contents

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Gaucher disease	UK	Suspended	05-Oct-2009
Amicus Therapeutics Inc	Gaucher disease	US	Suspended	05-Oct-2009
Shire Human Genetic Therapies Inc	Gaucher disease	US	Suspended	05-Oct-2009

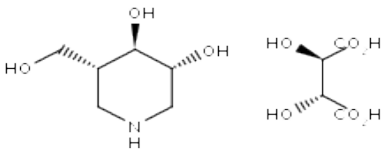
afegostat CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
169105-89-9	1



Name	Type
afegostat	INN; USAN
isofagomine	

CAS Registry Number:	Confidence Level:
	2



Name	Type
afegostat tartrate	USAN
Plicera	Trade Name
HGT-3410	Research Code
AT-2101	Research Code
isofagomine L-(+)-tartrate	

Return to Table of Contents

Name	Type
isofagomine tartrate	

afegostat DRUG NAMES

Names	Type
AT-2101	Research Code
Gauchers disease chaperone therapy, Amicus	
HGT-3410	Research Code
Plicera	Trade Name
afegostat	INN, USAN
afegostat tartrate	USAN
glucocerebrosidase modulator (Gaucher's disease), Amicus	
isofagomine	
isofagomine L-(+)-tartrate	
isofagomine tartrate	

afegostat 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
Gaucher disease											
0	0	0	0	0	0	0	3	0	0	0	4
Gauchers disease type I											
0	0	0	0	0	3	0	1	0	0	0	4

Return to Table of Contents

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	3	0	4	0	0	0	7

Phase Definitions

Phase 3 Clinical
Includes Phase 3, Phase 3b, Phase 3a, Phase 2/3 (where enrolment count is 300 or over)

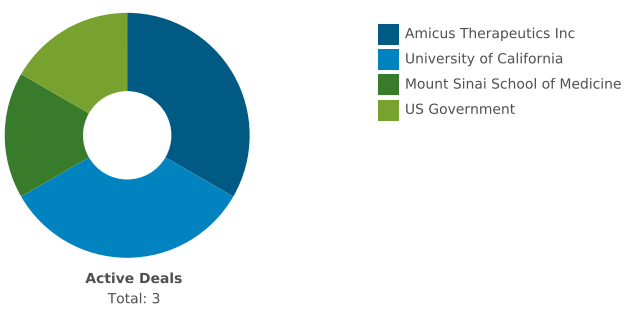
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

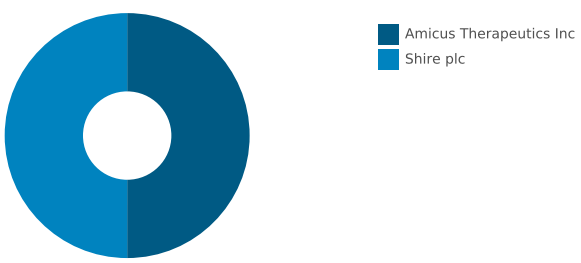
afegostat DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 3



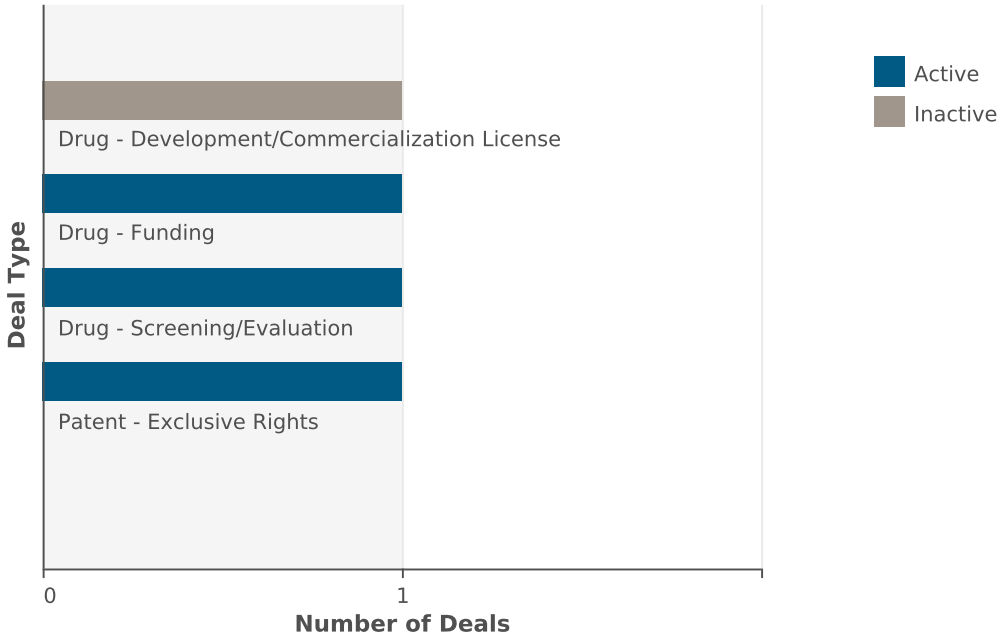
Inactive Deals
Total: 1

Return to Table of Contents

Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	0	1	2	0	3
University of California	2	0	0	0	2
Mount Sinai School of Medicine	1	0	0	0	1
Shire plc	0	0	0	1	1
US Government	0	0	1	0	1

Deals by Type Chart



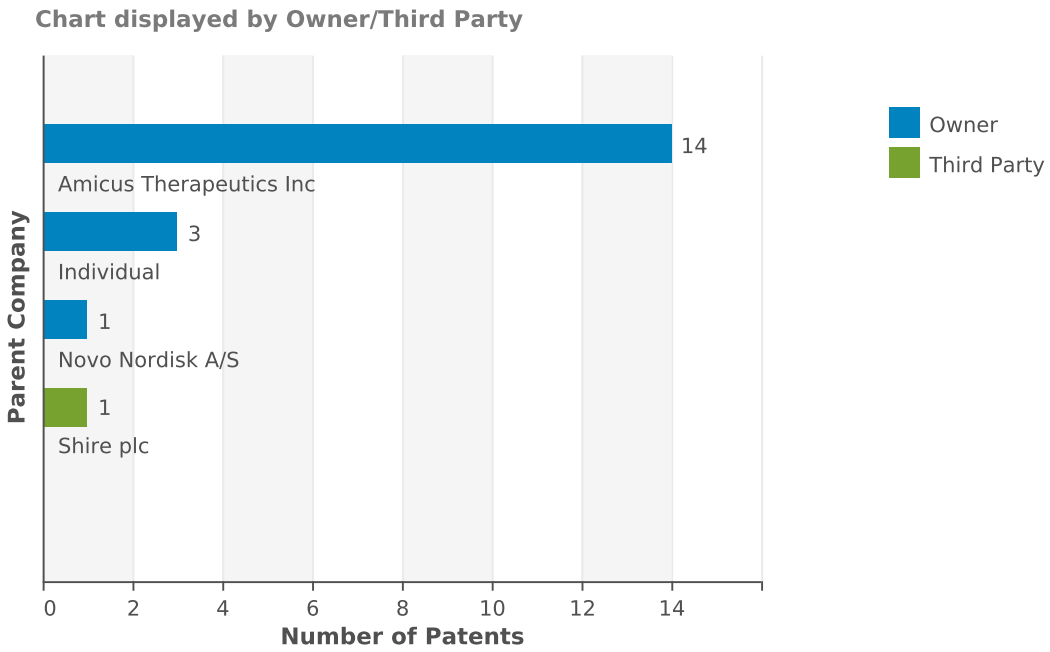
Deals by Type Table

Deal Type	Active	Inactive	Total
Patent - Exclusive Rights	1	0	1
Drug - Development/Commercialization License	0	1	1
Drug - Funding	1	0	1
Drug - Screening/Evaluation	1	0	1

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

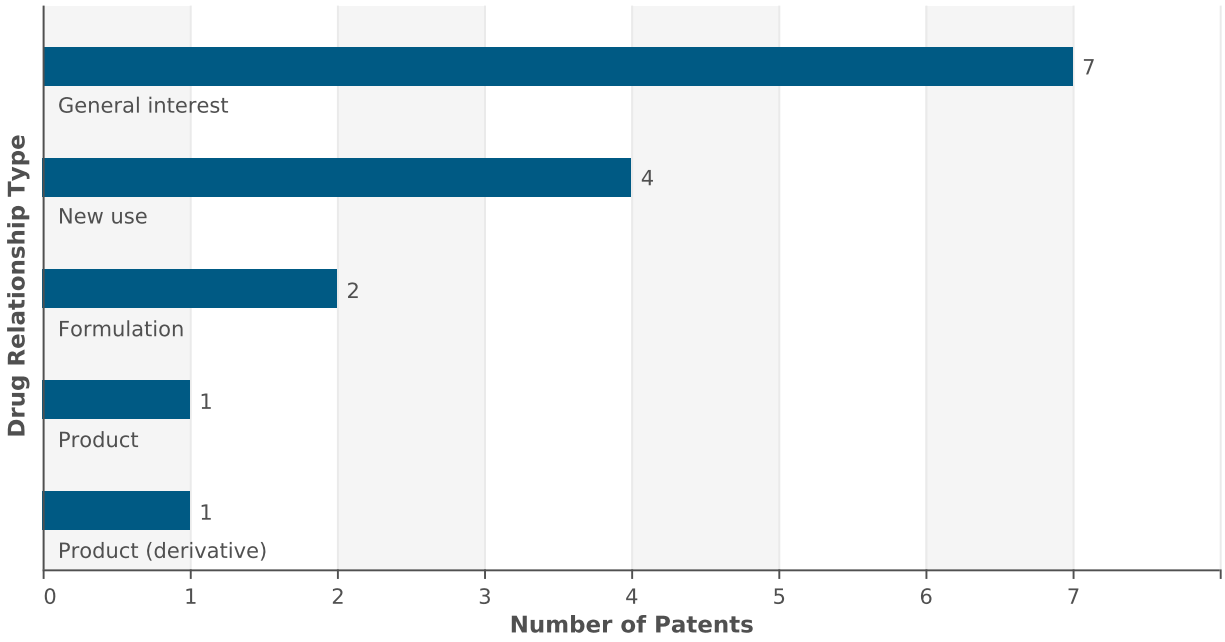


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	14	0	14
Individual	3	0	3
Shire plc	0	1	1
Novo Nordisk A/S	1	0	1

Return to Table of Contents

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
General interest	7
New use	4
Formulation	2
Product	1
Product (derivative)	1

Return to Table of Contents

migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus

migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus SNAPSHOT

Drug Name	migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Fabry disease
Target-based Actions	Alpha-galactosidase stimulator
Other Actions	
Technologies	Biological therapeutic;Drug combination;Intravenous formulation;Recombinant enzyme
Last Change Date	22-May-2018

migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus DEVELOPMENT PROFILE

SUMMARY

Amicus is investigating AT-B101/AT-1001, an iv coformulation of AT-B101 (a next-generation recombinant enzyme replacement therapy (ERT) comprising human alpha-galactosidase A) and migalastat (a pharmacological chaperone that enhances stability and folding of mutated alpha-galactosidase A), for the potential treatment of Fabry disease,. In March 2015, development was ongoing for the next-generation ERT. In March 2018, the program was expected to advance towards clinic in 2019 .

The company was also developing iv coformulation, migalastat hydrochloride + AT-B100 for the potential treatment of Fabry disease, however, by 2015, work on the new ERT had superseeded previous work on migalastat hydrochloride + AT-B100.

migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Fabry disease	US	Discovery	03-Mar-2015

Return to Table of Contents

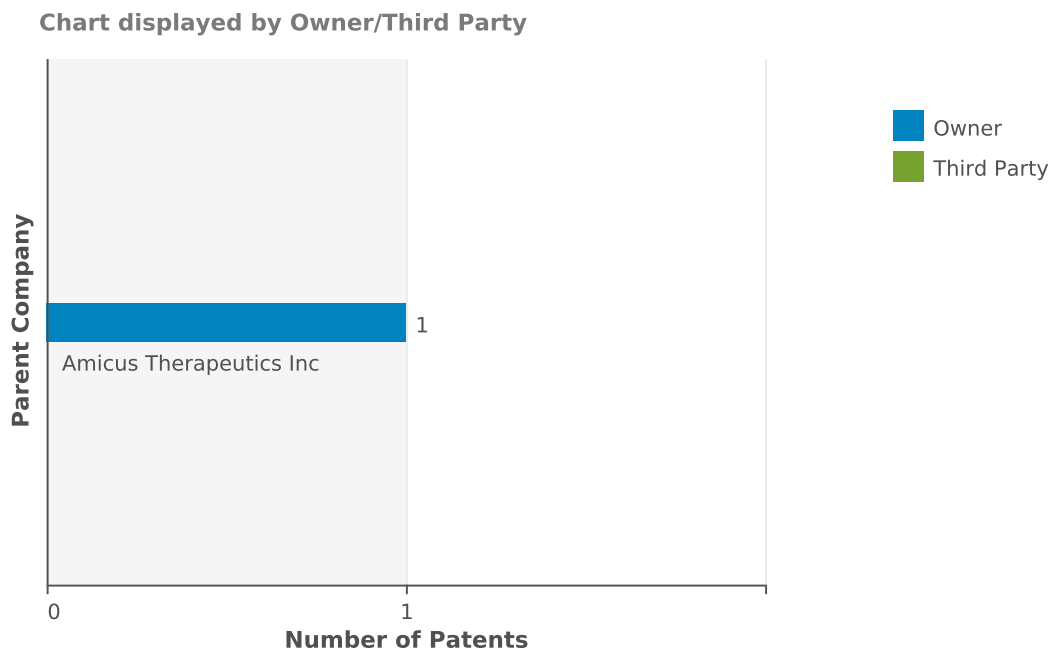
migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus DRUG NAMES

Names	Type
AT-B101/AT-1001	Research Code
migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus	
migalastat + human alpha-galactosidase A (iv coformulation, Fabry disease), Amicus	

migalastat + AT-B101 (iv coformulation, Fabry disease), Amicus DEALS AND PATENTS

PATENTS

Patents by Parent Company Chart

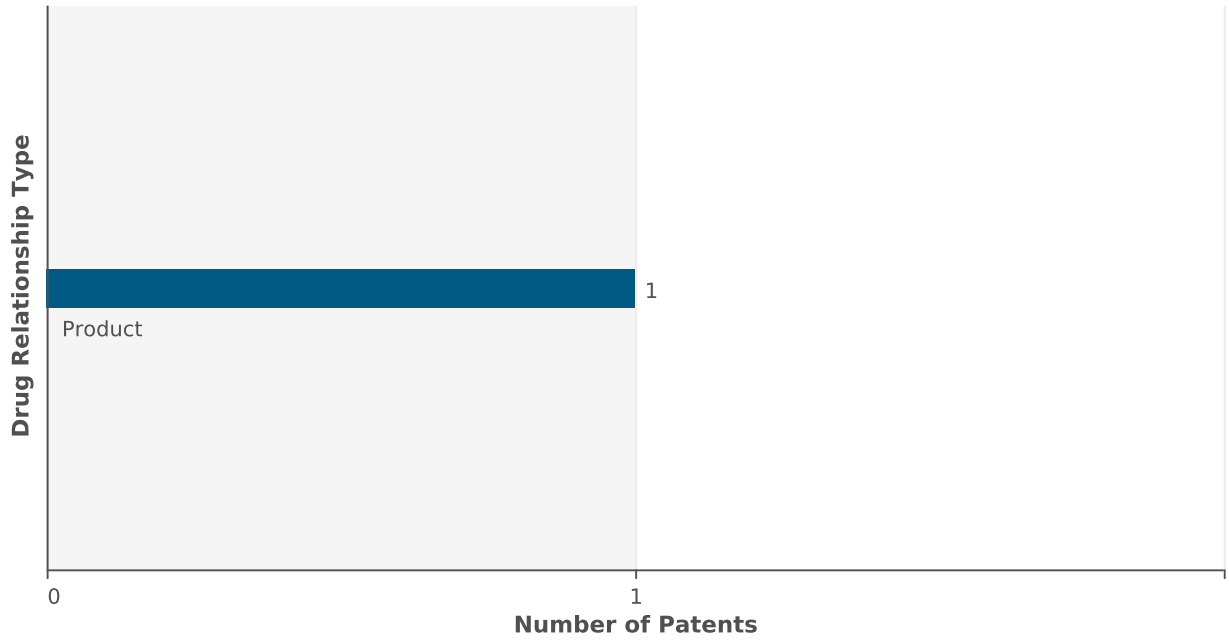


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Amicus Therapeutics Inc	1	0	1

Return to Table of Contents

Patents by Drug Relationship Type Chart



Patents by Drug Relationship Type Table

Drug Relationship	Total
Product	1

Return to Table of Contents

undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics

undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics SNAPSHOT

Drug Name	undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics
Key Synonyms	
Originator Company	Amicus Therapeutics Inc
Active Companies	Amicus Therapeutics Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Mucopolysaccharidosis type I
Target-based Actions	Alpha-L-iduronidase stimulator
Other Actions	
Technologies	Biological therapeutic;Drug combination;Infusion;Parenteral formulation unspecified;Recombinant enzyme
Last Change Date	28-May-2015

undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics DEVELOPMENT PROFILE

SUMMARY

Amicus Therapeutics is investigating a next-generation enzyme replacement therapy (ERT) comprising an undisclosed pharmacological chaperone plus a human recombinant alpha-L-iduronidase (IDUA; rhIDUA) ERT, as a biobetter, using its chaperone-advanced replacement therapy (CHART) platform technology, for the potential infusion treatment of mucopolysaccharidosis type I. In June 2013, the therapy was in preclinical development. In May 2015, the program was listed as being in discovery.

undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Amicus Therapeutics Inc	Mucopolysaccharidosis type I	US	Discovery	25-Jun-2013

Return to Table of Contents

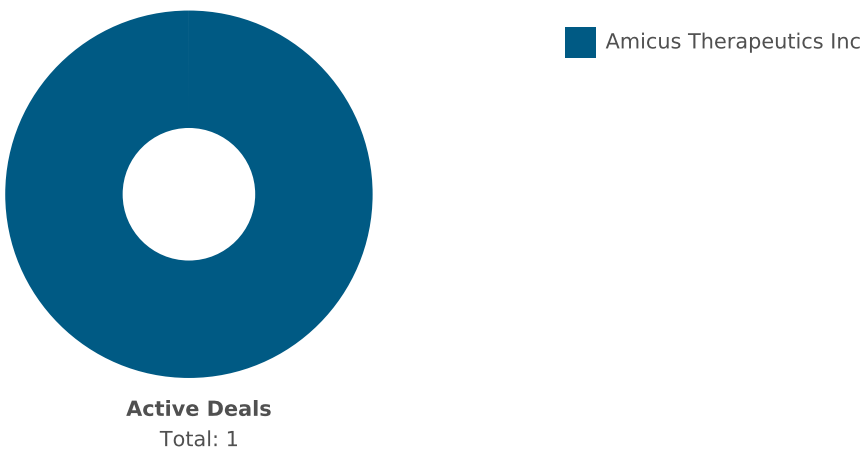
undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics DRUG NAMES

Names	Type
rhIDUA biobetter	
undisclosed chaperone + IDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics	
undisclosed chaperone + recombinant alpha-L-iduronidase enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics	
undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics	

undisclosed chaperone + rhIDUA enzyme replacement therapy (CHART, mucopolysaccharidosis type I), Amicus Therapeutics DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

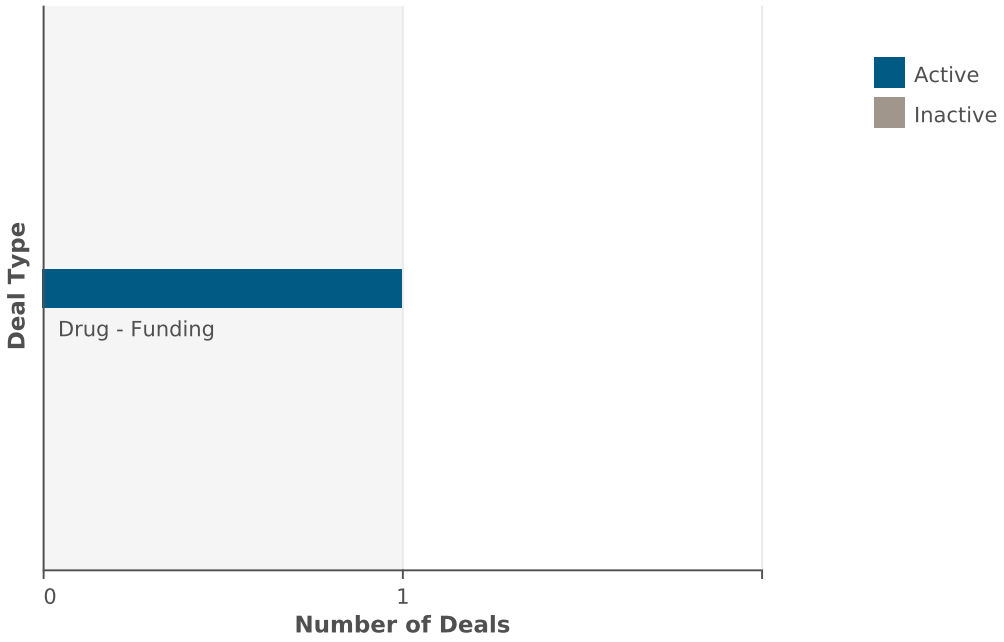


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Amicus Therapeutics Inc	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



Deals by Type Table

Deal Type	Active	Inactive	Total
Drug - Funding	1	0	1

Return to Table of Contents

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Return to Table of Contents