THOMSON REUTERS
CORTELLIS™

Ultragenyx Pharmaceutical Inc

CORTELLIS COMPANY DETAILED PIPELINE REPORT

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

Publication Date: 19-Jan-2015

THOMSON REUTERS
3 Times Square
New York, New York 10036
United States

Tel: +1 646 223 4000

thomsonreuters.com

Return to Table of Contents

THOMSON REUTERS

ABOUT CORTELLIS COMPANY DETAILED PIPELINE REPORT

Thomson Reuters 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 Thomson Reuters, using information from *Thomson Reuters 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.

Cortellis Company Detailed Pipeline reports are the second in a series of that track pharmaceutical and biotechnology companies worldwide. All *Cortellis for Competitive Intelligence* content is subject to the most comprehensive editorial review process available, conducted by scientists, pharma professionals, regulatory experts, and generics specialists. Featuring timely drug pipeline information expertly uncovered and integrated from over 400 global meetings each year, you'll always be on top of the latest developments.

Chosen by leading life sciences companies, their executives and investors, *Cortellis for Competitive Intelligence* accelerates your deal-making and gives you timely insights on the development landscape.

Discover undiscovered opportunities in drug development and licensing faster with *Thomson Reuters Cortellis™ for Competitive Intelligence*

DISCLAIMER

The information contained in this report is based on sources believed to be correct but Thomson Reuters does not guarantee the accuracy, timeliness, or completeness of this information. Opinions, if any, are those held by the author of any individual report or article at the time of initial publication and do not necessarily reflect the views of Thomson Reuters.

Information in this report on companies is intended for reference use only, and does not constitute a recommendation to buy or sell any particular security or other investment and does not constitute an offer to buy from or sell to any particular investor. Any company or securities mentioned in this report may not be suitable for any particular investor, depending on that investor's financial position and needs.

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

Ultragenyx Pharmaceutical Inc

COMPANY OVERVIEW

Company Name	Ultragenyx Pharmaceutical Inc
Parent Company Name	Ultragenyx Pharmaceutical Inc
Website	http://www.ultragenyx.com/
Country	US
Number of Drugs in Active Development	5
Number of Inactive Drugs	2
Number of Patents as Owner	7
Number of Patents as Third Party	2
Number of Deals	14
Key Indications	Myopathy,Carbohydrate metabolism disorder,Fatty acid oxidation disorder,Huntingtons chorea,Lysosome storage disease,Osteomalacia,Sly syndrome,X linked dominant hypophosphatemic rickets,Hereditary inclusion body myositis,Alzheimers disease,Creutzfeldt Jakob disease,Frontotemporal dementia,Neurodegenerative disease,Neurological disease,Parkinsons disease,Senile dementia,Vascular dementia
Key Target-based Actions	Unspecified enzyme stimulator,Lysosomal protective protein stimulator,Neural cell adhesion molecule modulator,Exo-alpha sialidase modulator
Key Technologies	Biological therapeutic,Parenteral formulation unspecified,Enzyme,Tablet formulation,Oral formulation,Sustained release formulation,Protein recombinant,Small molecule therapeutic,Hybridization technology,Capsule formulation,Enteric coated formulation,Glycoprotein,Immobilization technology,Immunoassay,Immunodetection,Monoclonal antibody,Oral quick release formulation,Oral sustained release formulation

COMPANY PROFILE

SUMMARY

Ultragenyx Pharmaceutical Inc is a biotechnology company focused on development of therapeutics for treatment of rare diseases.

COMPANY LOCATION

In April 2012, the company moved its corporate headquarters to a larger facility in the Bel Marin Keys area of Novato, CA.

FINANCIAL

In July 2014, the company announced an underwritten public offering of 2,017,349 shares of its common stock that includes 1,311,277 and 706,072 shares offered by Ultragenyx and selling stockholders, respectively. At that time, the underwriters would be granted a 30-day option to purchase up to an aggregate of an additional 302,602 shares of common stock. Later that month, the company priced the offering at $40 per share and granted the underwriters an option to buy additional shares. Again later that month, the offering was closed and the company raised approximately $60.2 million through 2,319,951 shares of its common stock with the additional share purchase option exercised in full.

In January 2014, Ultragenyx priced its IPO of 5,760,369 shares of common stock at $21.00 per share and granted the underwriters a 30-day over-allotment option to purchase up to an additional 864,054 shares. The stock was to begin trading on the NASDAQ Global Select Market under the ticker symbol 'RARE'. At that time, the IPO was to close on February 05, 2014. In February 2014, the offering was closed. Underwriters had exercised their right in full to purchase additional shares. Net proceeds were expected to be approximately $126.4 million.

In December 2012, the company raised $75 million in a series B financing round.

In July 2012, the company raised $15.1 million towards a goal of raising $30.1 million in a second closing of its $45

Return to Table of Contents

million Series A financing round.

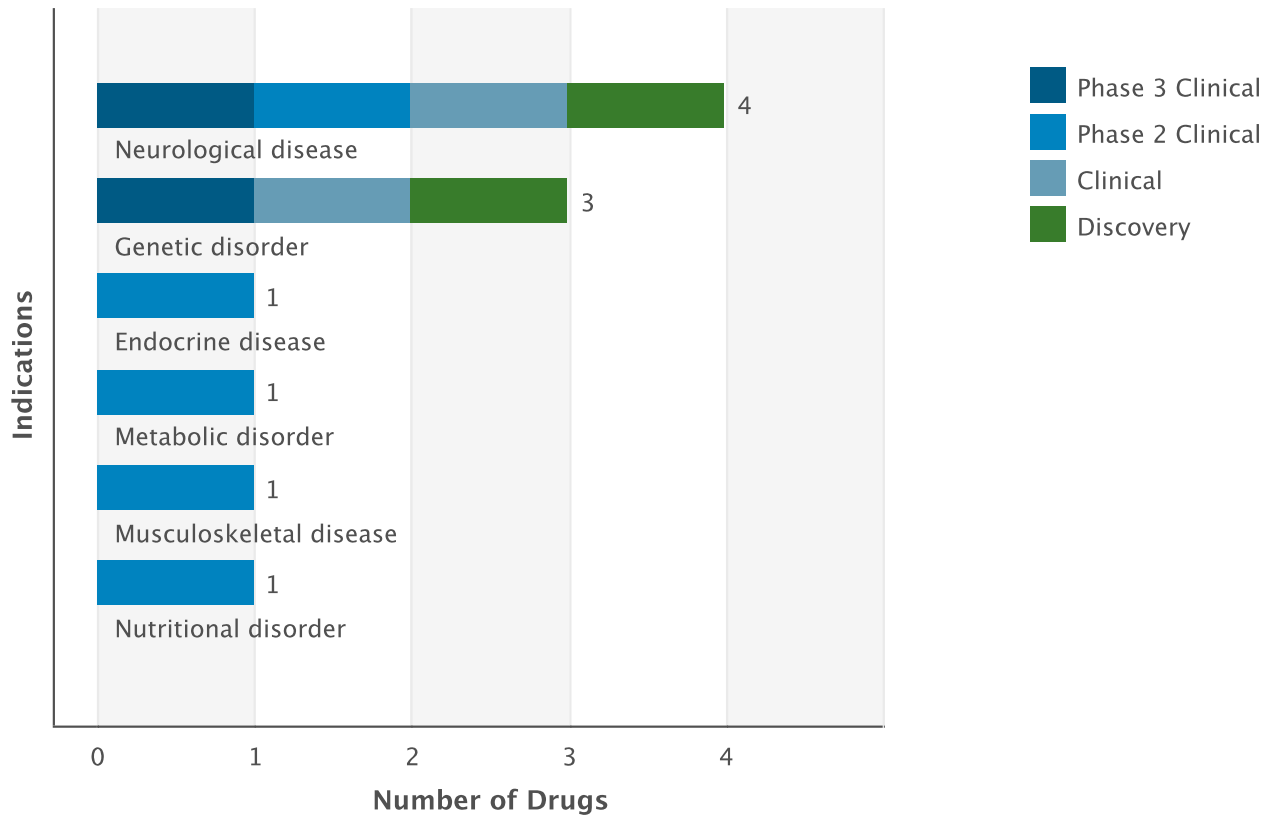
In June 2011, the company closed a $45 million Series A financing round.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



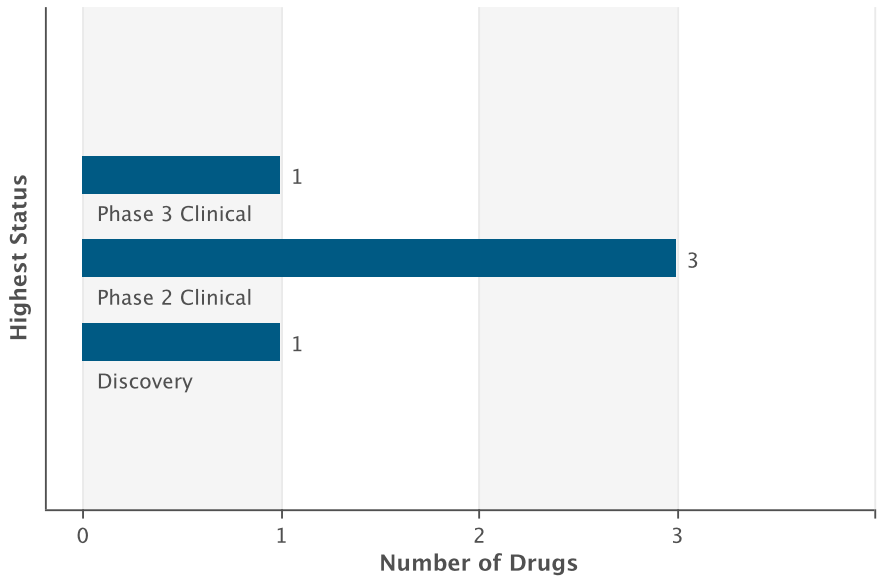
Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	4	2	6
Genetic disorder	3	2	5
Metabolic disorder	1	0	1
Nutritional disorder	1	0	1
Musculoskeletal disease	1	0	1
Endocrine disease	1	0	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
Phase 3 Clinical	1
Phase 2 Clinical	3
Discovery	1
No Development Reported	2

DEALS

Deal Type	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Technology - Other Proprietary	0	0	1	0	1
Patent - Exclusive Rights	0	0	4	0	4
Drug - Funding	1	0	0	0	1
Drug - Development/Commercialization License	0	0	4	0	4
Drug - Manufacturing/Supply	0	0	3	0	3
Technology - Delivery/Formulation	0	0	1	0	1

Return to Table of Contents

CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Neurological disease	3	6
Metabolic disorder	4	6
Genetic disorder	3	4
Musculoskeletal disease	2	2
Endocrine disease	2	2
Nutritional disorder	1	1
Dermatological disease	1	1
Neoplasm	1	1

Trials by Phase

Phase	Ongoing	All
Phase 3	1	1
Phase 2	7	11
Phase 1	1	2
Phase not specified	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

PATENTS *

Indication	As Owner	As Third Party	Total
Cardiovascular disease	1	0	1
Genitourinary disease	1	0	1
Degeneration	1	0	1
Musculoskeletal disease	4	2	6
Genetic disorder	3	0	3

Return to Table of Contents

Metabolic disorder	2	0	2
Neurological disease	8	1	9
Infectious disease	1	0	1
Inflammatory disease	3	2	5

* 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

UX-003

UX-003 SNAPSHOT

Drug Name	UX-003
Key Synonyms	
Originator Company	St Louis University
Active Companies	Ultragenyx Pharmaceutical Inc
Inactive Companies	St Louis University
Highest Status	Phase 3 Clinical
Active Indications	Sly syndrome
Target-based Actions	Beta-glucuronidase stimulator
Other Actions	
Technologies	Recombinant enzyme;Biological therapeutic;Intravenous formulation;Infusion
Last Change Date	14-Jan-2015

UX-003 DEVELOPMENT PROFILE

SUMMARY

Ultragenyx, under license from St Louis University, is developing UX-003, a recombinant human beta-glucuronidase (rhGUS), as an enzyme replacement therapy (ERT) for the potential iv treatment of lysosomal storage diseases, including mucopolysaccharidosis type 7 (MPS7), also known as Sly syndrome,. In November 2014, a phase III trial was initiated in the US. In October 2013, a phase I/II trial in UK was initiated ; in March 2014, preliminary data were presented.

UX-003 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Ultragenyx Pharmaceutical Inc	Sly syndrome	US	Phase 3 Clinical	24-Nov-2014
Ultragenyx Pharmaceutical Inc	Sly syndrome	UK	Phase 2 Clinical	28-Oct-2013
St Louis University	Sly syndrome	US	Discontinued	05-Jan-2012

Return to Table of Contents

UX-003 DRUG NAMES

Names	Type
ERT program (Sly syndrome), Ultragenyx Pharmaceutical	
UX-003	Research Code
ERT program (mucopolysaccharidosis type 7), Ultragenyx Pharmaceutical	
enzyme replacement therapy program (mucopolysaccharidosis type 7), St Louis University	
ERT program (mucopolysaccharidosis type 7), St Louis University	
ERT program (Sly syndrome), St Louis University	
recombinant human beta-glucuronidase (rhGUS, infusion, mucopolysaccharidosis type 7), Ultragenyx	
enzyme replacement therapy program (mucopolysaccharidosis type 7), Ultragenyx Pharmaceutical	
recombinant human beta-glucuronidase (rhGUS, mucopolysaccharidosis type 7), Ultragenyx	

UX-003 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
Sly syndrome											
0	0	1	1	0	0	1	1	0	1	2	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	1	1	0	0	1	1	0	1	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)

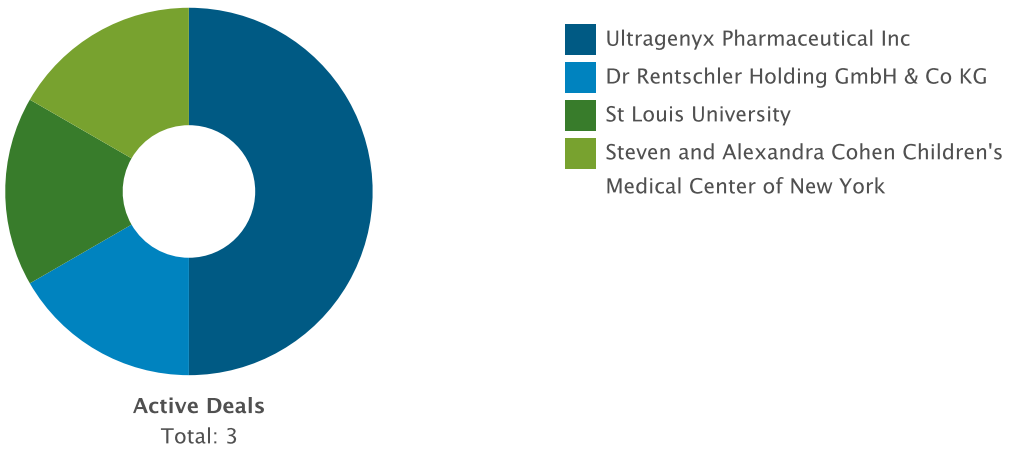
Return to Table of Contents

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

UX-003 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



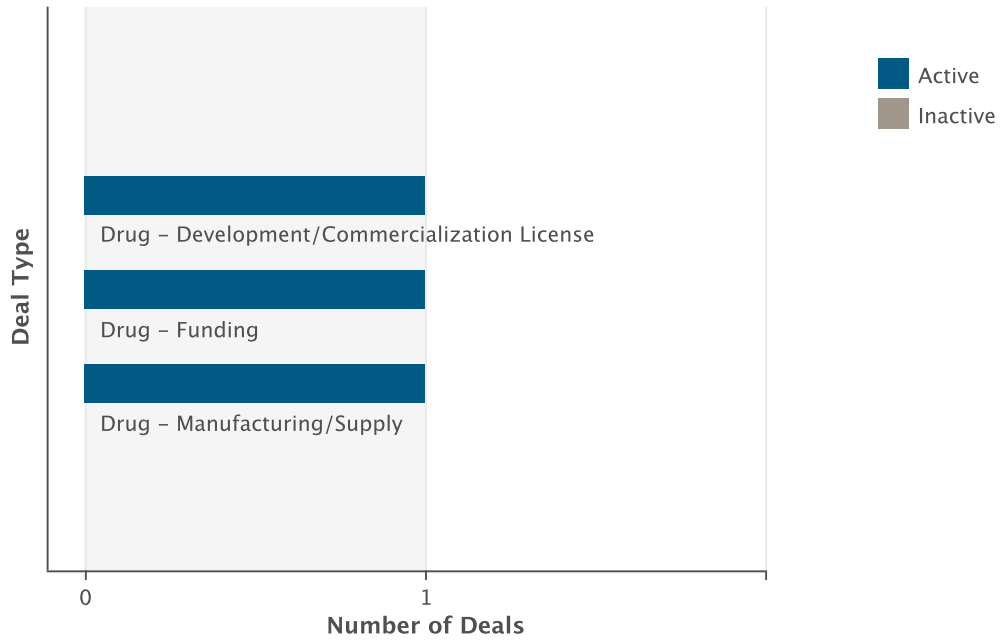
Active Deals
Total: 3

Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Ultragenyx Pharmaceutical Inc	1	0	2	0	3
St Louis University	1	0	0	0	1
Steven and Alexandra Cohen Children's Medical Center of New York	0	0	1	0	1
Dr Rentschler Holding GmbH & Co KG	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



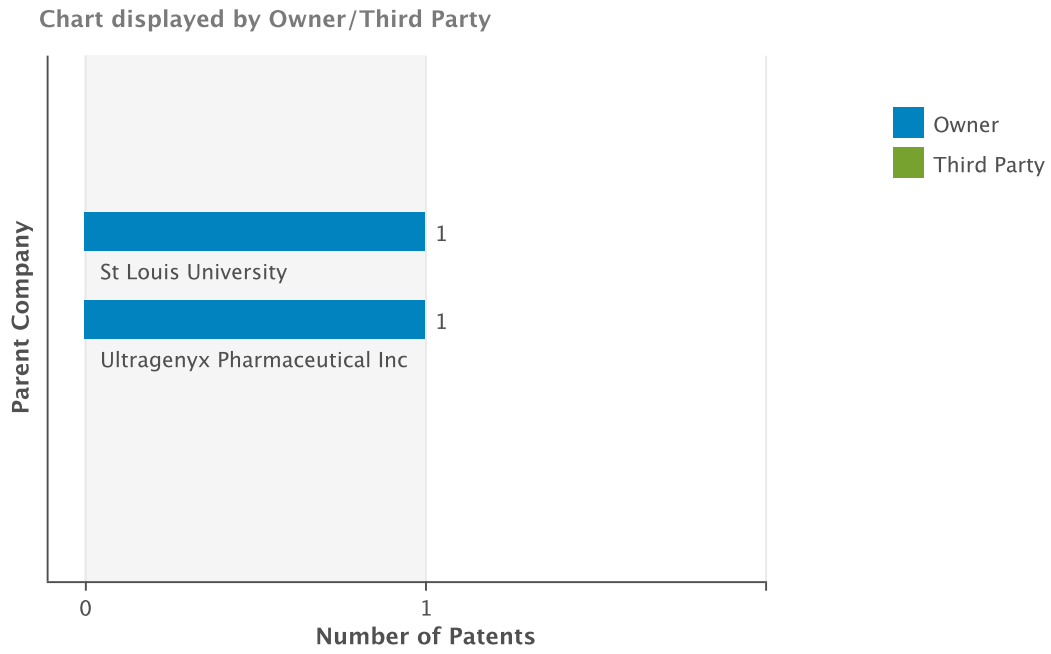
Deals by Type Table

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

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

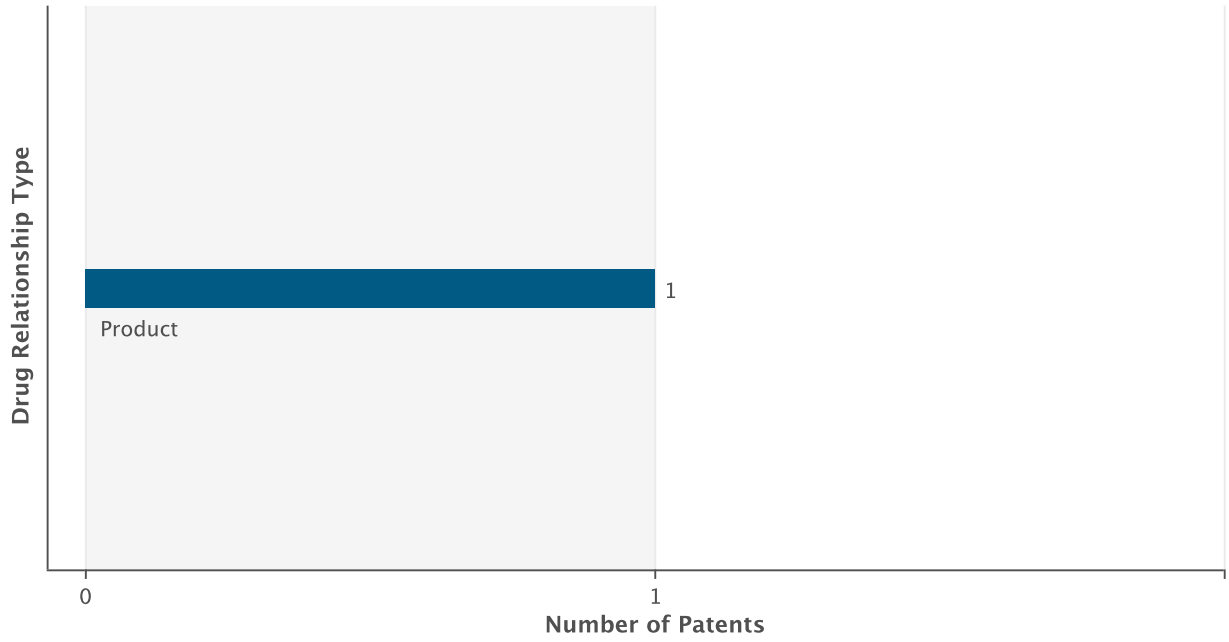


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
St Louis University	1	0	1
Ultragenyx Pharmaceutical 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

KRN-23

KRN-23 SNAPSHOT

Drug Name	KRN-23
Key Synonyms	
Originator Company	Kyowa Hakko Kirin Co Ltd
Active Companies	Kyowa Hakko Kirin Co Ltd;Ultragenyx Pharmaceutical Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	X linked dominant hypophosphatemic rickets;Osteomalacia
Target-based Actions	Fibroblast growth factor 23 ligand inhibitor
Other Actions	Immunomodulator;Phosphate raising agent
Technologies	Biological therapeutic;Subcutaneous formulation;Intravenous formulation;Protein recombinant;Monoclonal antibody human;Immunoglobulin-
Last Change Date	14-Jan-2015

KRN-23 DEVELOPMENT PROFILE

SUMMARY

Kyowa Hakko Kirin, in collaboration with Ultragenyx, is developing KRN-23 (UX-023), an anti-fibroblast growth factor-23 (FGF-23) recombinant, fully human IgG1 mAb, for the potential injectable treatment of X-linked hypophosphatemic (XLH) rickets,. The companies are also investigating the mAb for the potential treatment of tumor-induced osteomalacia (TIO) .

In June 2014, a phase II trial was initiated in pediatric hypophosphatemia patients in the US ; in July 2014, the trial was initiated in Europe ; in August 2014, interim data from the trial were expected in 2015 ; in January 2015, 40-week bone/growth data was expected at the end of 2015 or beginning of 2016. In April 2011, a North American phase I/II trial was initiated; in October 2013, the trial was completed ; in June 2014, data were presented. In September 2013, an extension study was being completed. In October 2013, phase I data were presented,,. In July 2014, a phase I trial in Korea and Japan was initiated. In December 2014, a phase II trial for TIO was planned to be initiated in the US. The trial was expected to complete by April 2016 ; in January 2015, the trial was planned to be initiated in the first half of 2015 and data were expected by the end of 2015.

KRN-23 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Kyowa Hakko Kirin Co Ltd	X linked dominant hypophosphatemic rickets	Canada	Phase 2 Clinical	27-Apr-2011
Kyowa Hakko Kirin Co Ltd	X linked dominant hypophosphatemic rickets	Europe	Phase 2 Clinical	01-Jul-2014

Return to Table of Contents

Company	Indication	Country	Development Status	Date
Kyowa Hakko Kirin Co Ltd	X linked dominant hypophosphatemic rickets	US	Phase 2 Clinical	27-Apr-2011
Ultragenyx Pharmaceutical Inc	X linked dominant hypophosphatemic rickets	Canada	Phase 2 Clinical	03-Sep-2013
Ultragenyx Pharmaceutical Inc	X linked dominant hypophosphatemic rickets	Europe	Phase 2 Clinical	01-Jul-2014
Ultragenyx Pharmaceutical Inc	X linked dominant hypophosphatemic rickets	US	Phase 2 Clinical	03-Sep-2013
Kyowa Hakko Kirin Co Ltd	X linked dominant hypophosphatemic rickets	Japan	Phase 1 Clinical	02-Jul-2014
Kyowa Hakko Kirin Co Ltd	X linked dominant hypophosphatemic rickets	South Korea	Phase 1 Clinical	02-Jul-2014
Kyowa Hakko Kirin Co Ltd	Osteomalacia	US	Discovery	24-Nov-2014
Ultragenyx Pharmaceutical Inc	Osteomalacia	US	Discovery	24-Nov-2014

KRN-23 DRUG NAMES

Names	Type
UX-023	Research Code
human mAb (injectable formulation, hypophosphatemia), Kyowa Hakko	
KRN-23	Research Code

KRN-23 SALES AND FORECASTS

COMMENTARY

REGIONAL DEVELOPMENT AND MARKETING RIGHTS

In August 2013, Ultragenyx Pharmaceutical entered into a collaboration and license agreement with Kyowa Hakko Kirin for the development and commercialization of KRN-23 to treat X-linked hypophosphatemia (XLH) in the US, Canada and European Union (EU), the profit share territory, Switzerland, Turkey and European territory. Kyowa Hakko Kirin would commercialize KRN-23 in the EU, while Ultragenyx would develop and commercialize the product in Mexico, Central and South America [1473363] [1473332], [1575903].

KRN-23 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
Hypophosphatemia											
0	0	0	0	1	1	0	3	0	0	1	4

Return to Table of Contents

Osteomalacia											
0	0	0	0	1	1	1	1	0	0	2	2
X linked dominant hypophosphatemic rickets											
0	0	0	0	1	1	1	1	0	0	2	2
Nevus											
0	0	0	0	1	1	0	0	0	0	1	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	3	3	1	4	0	0	4	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

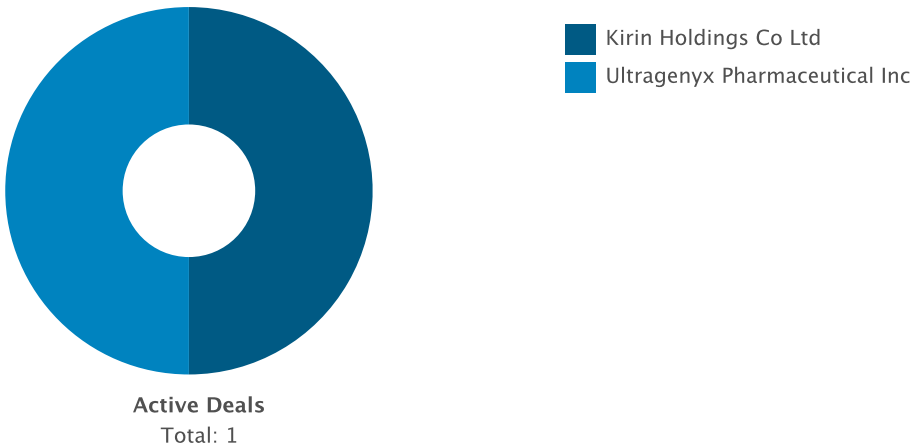
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

Return to Table of Contents

KRN-23 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Kirin Holdings Co Ltd	1	0	0	0	1
Ultragenyx Pharmaceutical Inc	0	0	1	0	1

Return to Table of Contents

Deals by Type Chart

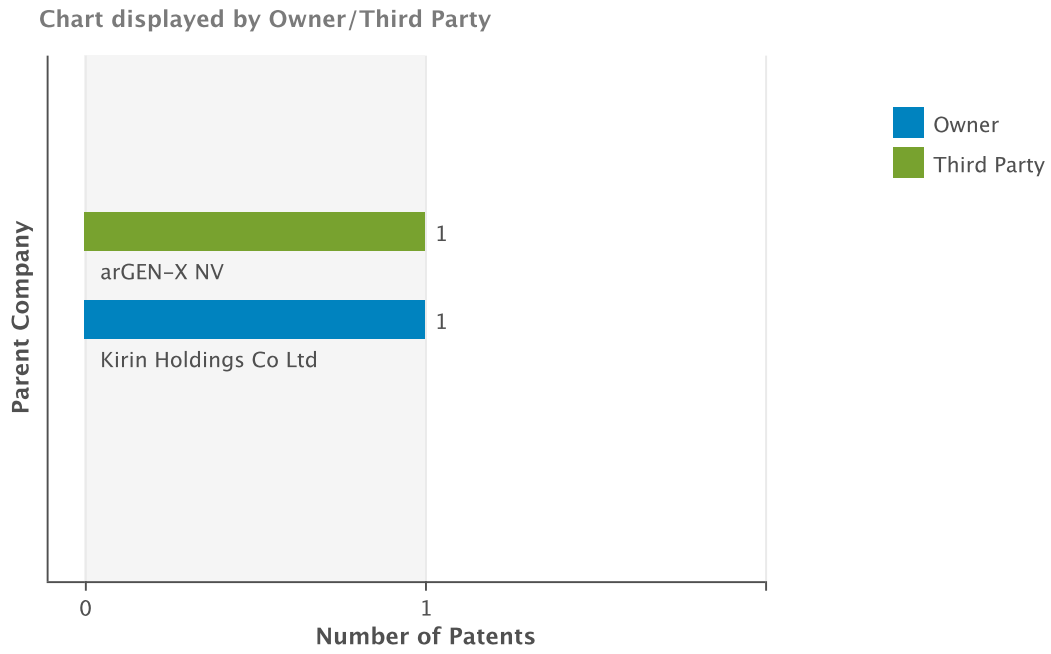
Deals by Type Table

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

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

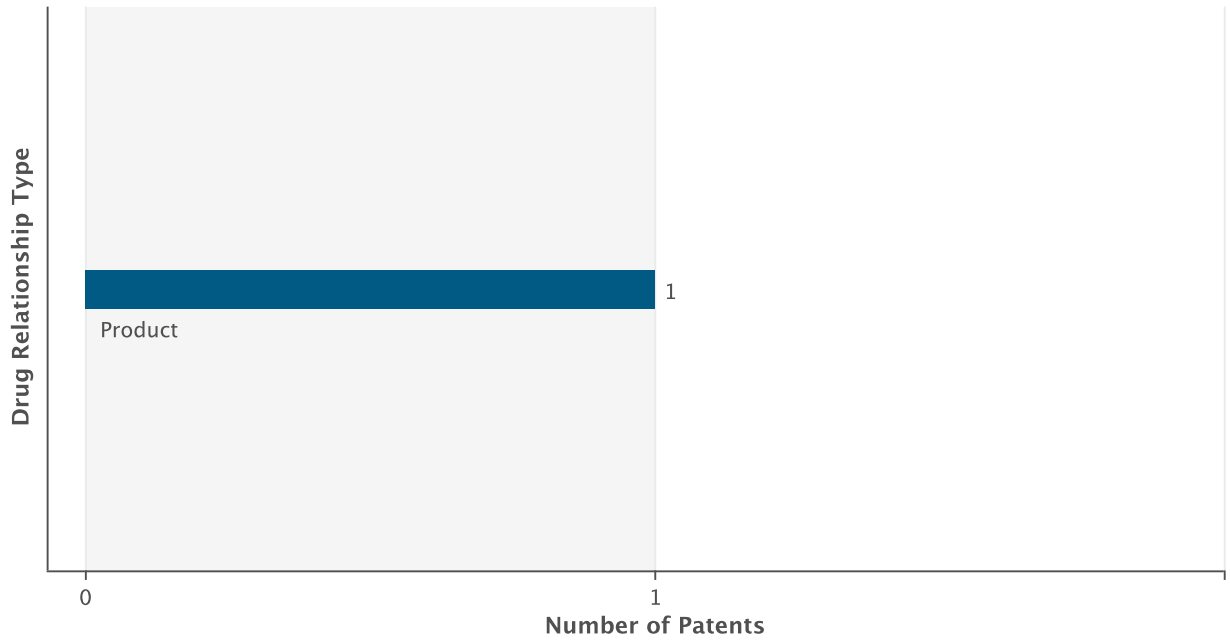


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
arGEN-X NV	0	1	1
Kirin Holdings Co Ltd	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

UX-001

UX-001 SNAPSHOT

Drug Name	UX-001
Key Synonyms	
Originator Company	Ultragenyx Pharmaceutical Inc
Active Companies	Ultragenyx Pharmaceutical Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Myopathy
Target-based Actions	
Other Actions	Unspecified drug target;Muscle system agent
Technologies	Tablet formulation;Oral formulation;Small molecule therapeutic;Sustained release formulation
Last Change Date	14-Jan-2015

UX-001 DEVELOPMENT PROFILE

SUMMARY

Ultragenyx is developing UX-001 (SA-ER), an extended-release oral tablet formulation of sialic acid, for the potential treatment of hereditary inclusion body myopathy (HIBM, also known as acetylglucosamine 2-epimerase/N-acetylmannosamine kinase gene/GNE myopathy),,. In May 2012, a phase II trial began ; in July 2013, interim 24-week data were reported ; in December 2013, 48-week data were reported and by that time, an extension study had been initiated. In October 2014, interim data from the phase II extension study were presented . In November 2014, the company planned to initiate a phase III trial by mid-2015,.

UX-001 DEVELOPMENT STATUS

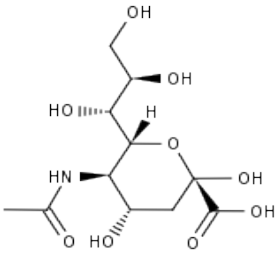
CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Ultragenyx Pharmaceutical Inc	Myopathy	Israel	Phase 2 Clinical	18-May-2012
Ultragenyx Pharmaceutical Inc	Myopathy	US	Phase 2 Clinical	18-May-2012

UX-001 CHEMICAL STRUCTURES

Return to Table of Contents

CAS Registry Number:	Confidence Level:
	2



Name	Type
sialic acid	
N-acetylneuraminic acid	

UX-001 DRUG NAMES

Names	Type
UX-001	
SA-ER	
sialic acid (extended release formulation/oral/tablet, HIBM), Ultragenyx	
sialic acid	
sialic acid (extended release/oral tablet formulation, GNE myopathy), Ultragenyx Pharmaceutical	
sialic acid (extended release formulation, hereditary inclusion body myopathies), Ultragenyx	

UX-001 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
Myopathy											
0	0	0	0	1	2	0	1	0	0	1	3

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	1	2	0	1	0	0	1	3

Phase Definitions

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

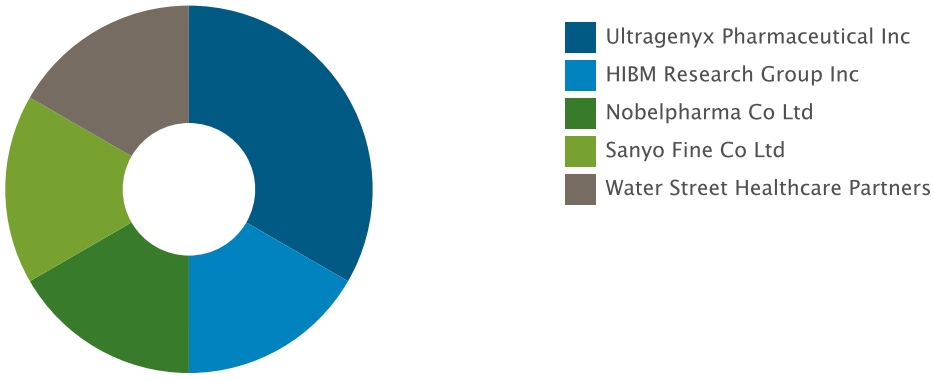
Phase 2 Clinical
Includes Phase 2, Phase 2a, Phase 2b, Phase 1/2 (where enrolment count is 100 or over), Phase 2/3 (where enrolment count is under 300 or not specified)

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

UX-001 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart



Active Deals
Total: 3

Return to Table of Contents

Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Ultragenyx Pharmaceutical Inc	0	0	2	0	2
HIBM Research Group Inc	1	0	0	0	1
Water Street Healthcare Partners	1	0	0	0	1
Nobelpharma Co Ltd	0	0	1	0	1
Sanyo Fine Co Ltd	1	0	0	0	1

Deals by Type Chart

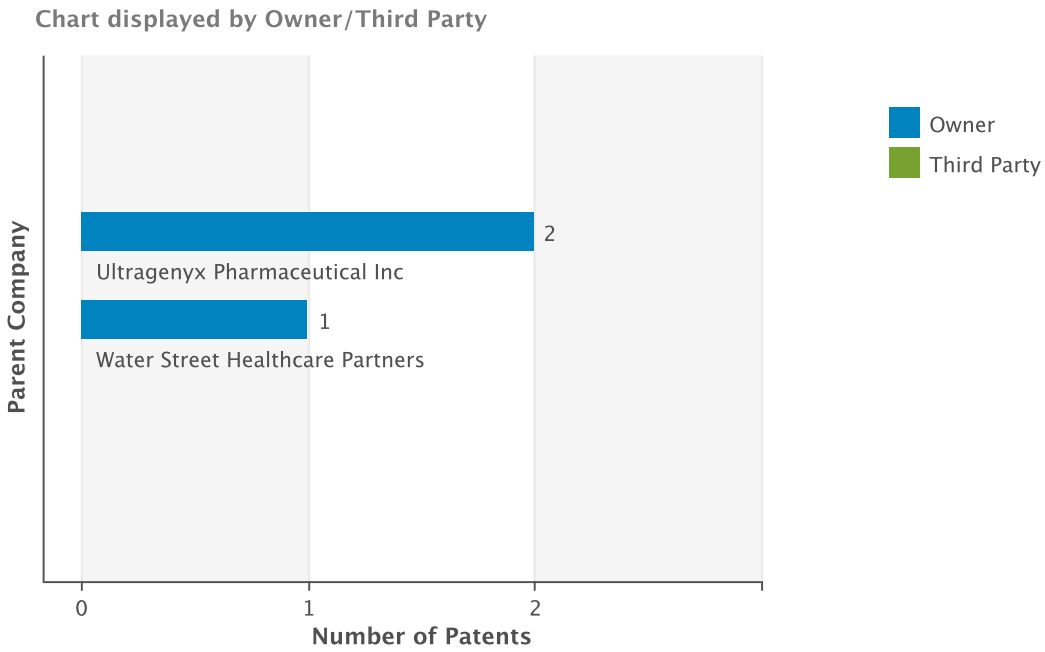
Deals by Type Table

Deal Type	Active	Inactive	Total
Patent - Exclusive Rights	1	0	1
Drug - Manufacturing/Supply	1	0	1
Technology - Delivery/Formulation	1	0	1

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

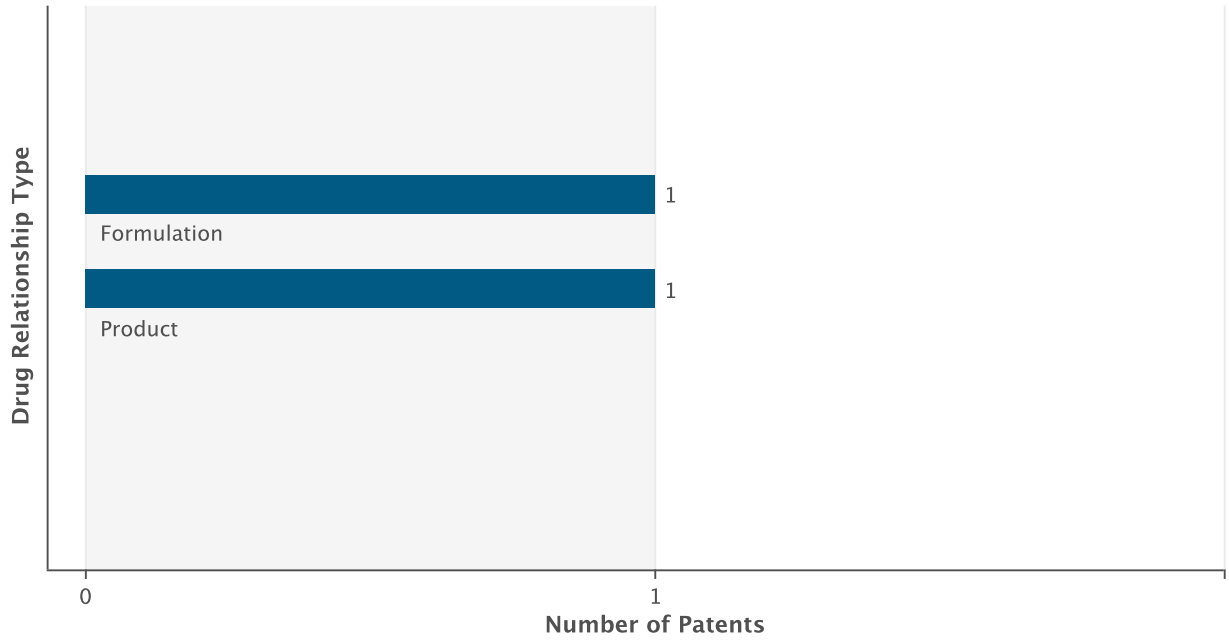


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
Ultragenyx Pharmaceutical Inc	2	0	2
Water Street Healthcare Partners	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
Formulation	1

Return to Table of Contents

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx SNAPSHOT

Drug Name	triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx
Key Synonyms	
Originator Company	Baylor Research Institute
Active Companies	Baylor Research Institute;Ultragenyx Pharmaceutical Inc
Inactive Companies	
Highest Status	Phase 2 Clinical
Active Indications	Huntingtons chorea;Fatty acid oxidation disorder;Carbohydrate metabolism disorder
Target-based Actions	Facilitated glucose transporter-1 stimulator
Other Actions	Lipid metabolism modulator
Technologies	Oral liquid formulation;Oral formulation;Lipid;Small molecule therapeutic
Last Change Date	14-Jan-2015

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx DEVELOPMENT PROFILE

SUMMARY

Baylor Research Institute and licensee Ultragenyx Pharmaceutical are codeveloping UX-007 (triheptanoin), a synthetic compound, for the potential oral treatment of long-chain fatty acid oxidation disorders (FAOD) . Ultragenyx is also developing the drug as UX-007G, for the potential treatment of glucose transporter type-1 deficiency syndrome (Glut-1 DS),. Ultragenyx is also investigating the compound for the potential treatment of Huntington's disease (HD)under licence from INSERM. In February 2014, a phase II trial was initiated in Glut-1 DS patients; in March 2014, data were expected in 2015 ; in January 2015, interim data were expected in the second half of 2015. In September 2013, a phase II study for long-chain FAOD was initiated; at that time, the study was expected to complete in July 2015. In June 2014, pilot study data in HD was reported ; in January 2015, first data were presented. In January 2015, a second clinical trial in HD was planned.

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

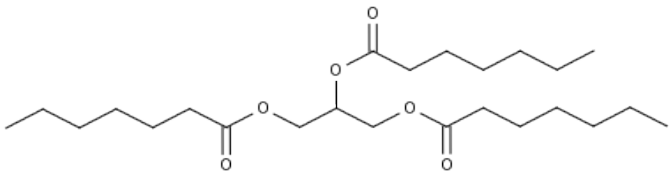
Company	Indication	Country	Development Status	Date
Baylor Research Institute	Fatty acid oxidation disorder	US	Phase 2 Clinical	17-Sep-2013
Ultragenyx Pharmaceutical Inc	Carbohydrate metabolism disorder	Italy	Phase 2 Clinical	04-Feb-2014
Ultragenyx Pharmaceutical Inc	Carbohydrate metabolism disorder	US	Phase 2 Clinical	04-Feb-2014
Ultragenyx Pharmaceutical Inc	Fatty acid oxidation disorder	Europe	Phase 2 Clinical	11-Feb-2014

Return to Table of Contents

Company	Indication	Country	Development Status	Date
Ultragenyx Pharmaceutical Inc	Fatty acid oxidation disorder	US	Phase 2 Clinical	17-Sep-2013
Ultragenyx Pharmaceutical Inc	Huntingtons chorea	US	Clinical	04-Jun-2014

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx CHEMICAL STRUCTURES

CAS Registry Number:	Confidence Level:
620-67-7	3



Name	Type
UX-007G	Research Code
UX-007	Research Code
triheptanoin	

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx DRUG NAMES

Names	Type
triheptanoin	
UX-007G	Research Code
triheptanoin (long-chain fatty acid oxidation disorders), Baylor Research Institute/Ultragenyx Pharmaceutical	
UX-007	Research Code
triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx	
triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency syndrome), Baylor Research Institute/Ultragenyx	
triheptanoin (long-chain fatty acid oxidation disorders), Baylor Research Institute/Ultragenyx	

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx CLINICAL TRIALS

Trials by Phase and Condition Studied

Return to Table of Contents

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
Fatty acid oxidation disorder											
0	0	0	0	1	2	0	0	1	1	2	3
Fanconi-Bickel disease											
0	0	0	0	1	2	0	0	0	0	1	2
Autosomal disorder											
0	0	0	0	1	1	0	0	0	0	1	1
Glycogen storage disease											
0	0	0	0	0	0	0	0	1	1	1	1
Inherited disorder											
0	0	0	0	0	1	0	0	0	0	0	1
Huntingtons chorea											
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	3	7	0	0	1	1	4	8

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)

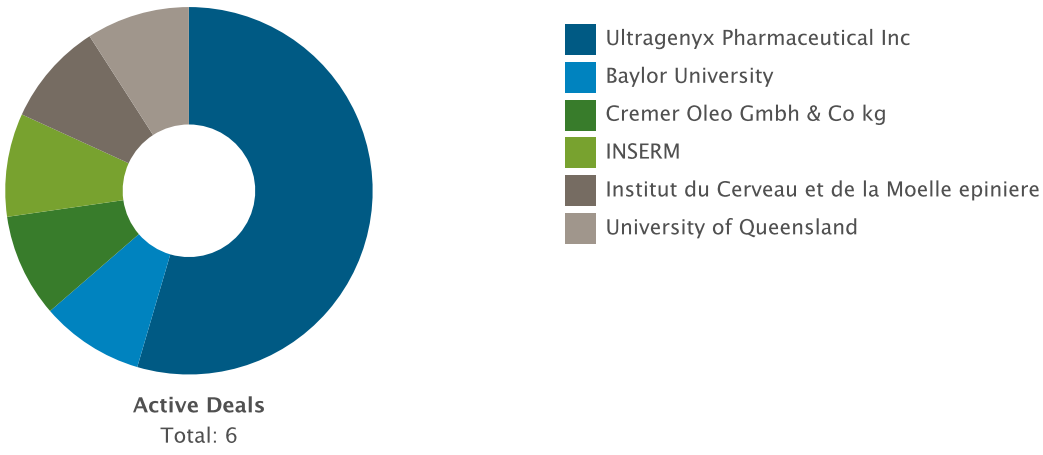
Phase 1 Clinical
Includes Phase 1, Phase 1a, Phase 1, Phase 1/2 (where enrolment count is under 100 or not specified), Phase 0

Return to Table of Contents

triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency, Huntingtons), Baylor/Ultragenyx DEALS AND PATENTS

DEALS

Deals by Parent Company Chart

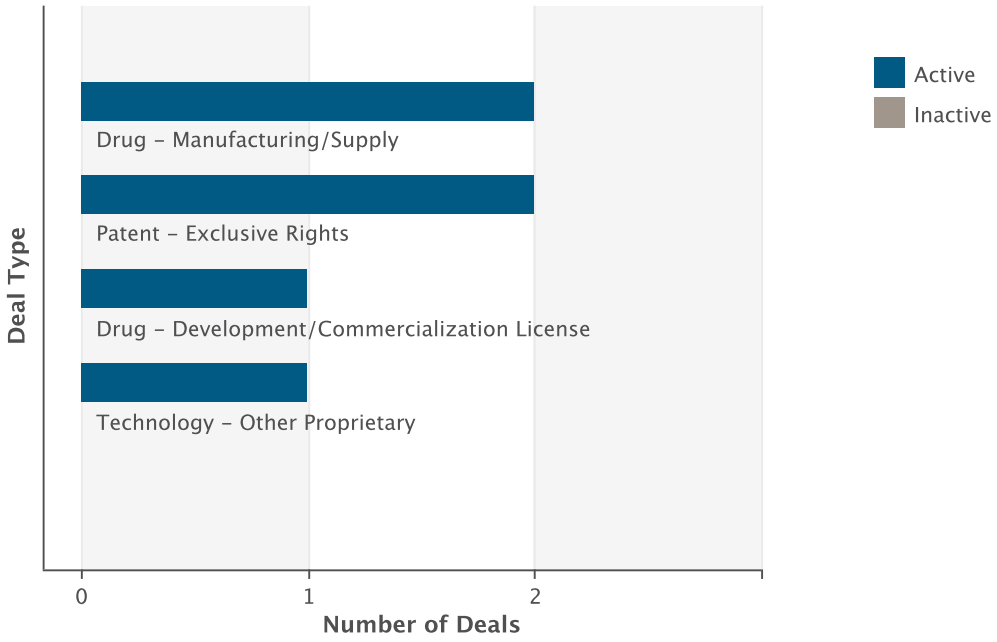


Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
Ultragenyx Pharmaceutical Inc	0	0	6	0	6
Cremer Oleo Gmbh & Co kg	1	0	0	0	1
INSERM	1	0	0	0	1
Baylor University	1	0	0	0	1
University of Queensland	1	0	0	0	1
Institut du Cerveau et de la Moelle epiniere	1	0	0	0	1

Return to Table of Contents

Deals by Type Chart



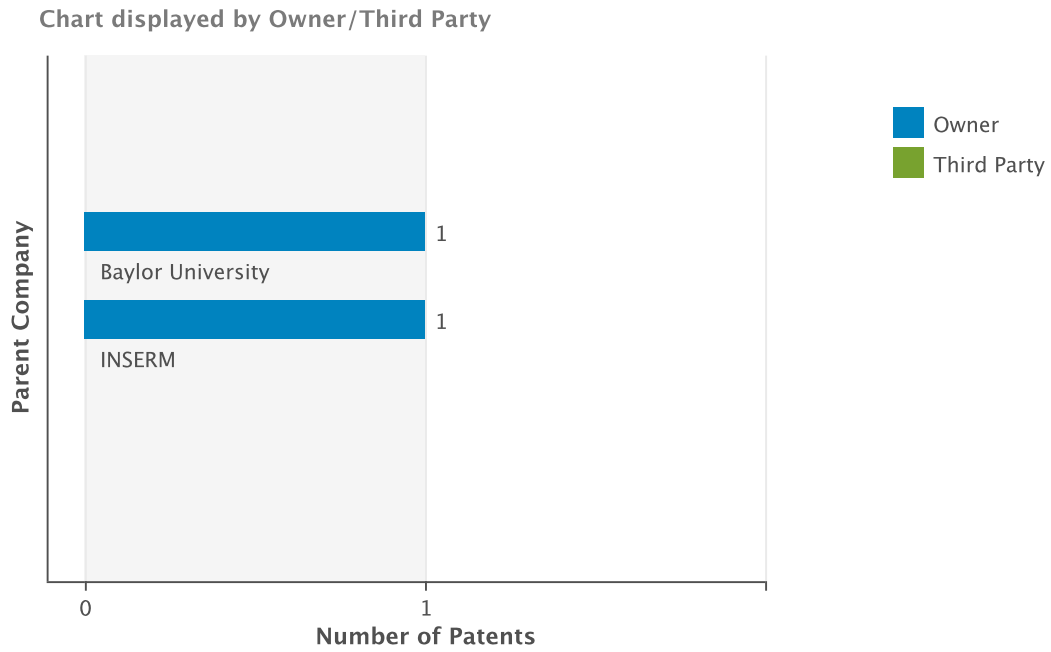
Deals by Type Table

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

Return to Table of Contents

PATENTS

Patents by Parent Company Chart

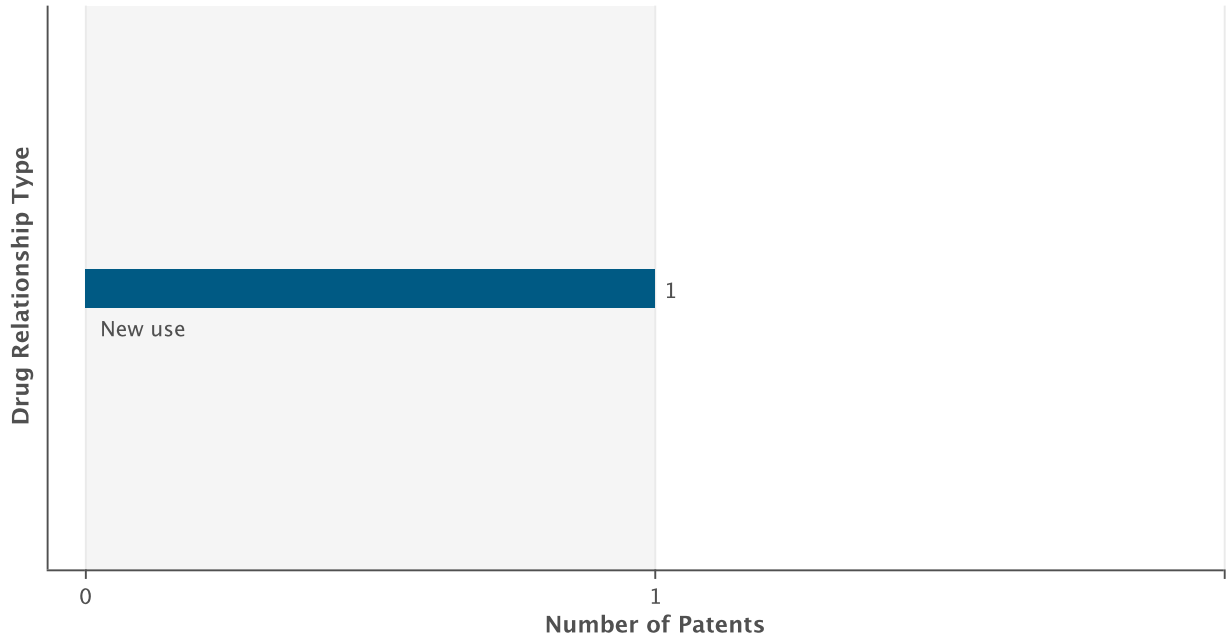


Patents by Parent Company Table

Company Name	As Owner	As Third Party	Total
INSERM	1	0	1
Baylor University	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	1

Return to Table of Contents

UX-004

UX-004 SNAPSHOT

Drug Name	UX-004
Key Synonyms	
Originator Company	Ultragenyx Pharmaceutical Inc
Active Companies	Ultragenyx Pharmaceutical Inc
Inactive Companies	
Highest Status	Discovery
Active Indications	Lysosome storage disease
Target-based Actions	Lysosomal protective protein stimulator
Other Actions	
Technologies	Biological therapeutic;Parenteral formulation unspecified;Protein recombinant
Last Change Date	13-Jun-2014

UX-004 DEVELOPMENT PROFILE

SUMMARY

Ultragenyx is investigating UX-004 (rhPPCA), a recombinant human protein protective cathepsin-A as an enzyme replacement therapy, for the potential treatment of galactosialidosis. In June 2011, the therapy was listed as being in preclinical development ; in June 2014, this was still the case.

UX-004 DEVELOPMENT STATUS

CURRENT DEVELOPMENT STATUS

Company	Indication	Country	Development Status	Date
Ultragenyx Pharmaceutical Inc	Lysosome storage disease	US	Discovery	07-Jun-2011

UX-004 DRUG NAMES

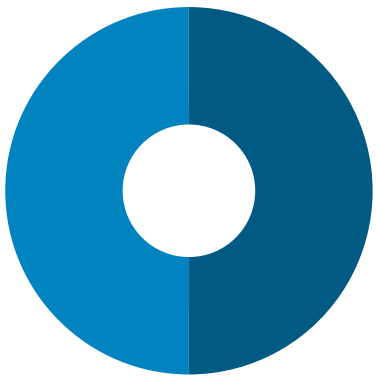
Names	Type
rhPPCA	
UX-004	Research Code

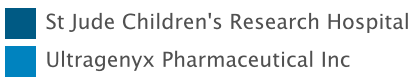
Return to Table of Contents

UX-004 DEALS AND PATENTS

DEALS

Deals by Parent Company Chart





Deals by Parent Company Table

Company Name	Principal		Partner		Total
	Active	Inactive	Active	Inactive	
St Jude Children's Research Hospital	1	0	0	0	1
Ultragenyx Pharmaceutical Inc	0	0	1	0	1

Return to Table of Contents

Deals by Type Chart

Deals by Type Table

Deal Type	Active	Inactive	Total
Patent - Exclusive Rights	1	0	1

Return to Table of Contents

This report was created by Thomson Reuters, using information from *Thomson Reuters Cortellis™ for Competitive Intelligence*; a comprehensive, proven intelligence solution that leverages the most accurate, complete, and widely respected drug pipeline information.

For more information about *Cortellis for Competitive Intelligence*, visit:
http://cortellis.thomsonreuters.com/cortellis_for_you/?cid=thomsonone.

For subscription information, e-mail scientific.lifesciences@thomsonreuters.com.

Return to Table of Contents