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Ultragenyx Pharmaceutical Inc

COMPANY AND PIPELINE OVERVIEW REPORT

A comprehensive coverage of the company and a summary of the drug pipeline portfolio.

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ABOUT COMPANY AND PIPELINE OVERVIEW 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.

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

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GLOSSARY

Number of Drugs in Active Development

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

Number of Inactive Drugs

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i.e. where the development status for the drug(s) is one of the following: No Development Reported, Discontinued, or Withdrawn.

Number of Patents as Owner

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

Number of Patents as Third Party

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

Patents summary table

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

Number of Deals

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

Key Indications

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

Key Target-based Actions

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

Key Technologies

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

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PLEASE NOTE: the financials section where present in the report includes: Share Information, Stock Performance chart (including the consensus recommendation), and Major Shareholders. Financial Performance presents graphical and tabular data on Worldwide Sales, Operating Income and Net Income over time, together with a Quarterly earnings update. Balance Sheet lists Assets, Liabilities and Stockholders Equity, and Forecasts includes: EPS Forecast and Other Forecasts. The data reported in all sections (except share information and major stock holders) are correct as of the publication date of the report (and not the download date). For share information and major stock holders the data is correct for the date shown with these sections

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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	12
Key Indications	Lysosome storage disease,Myopathy,Carbohydrate metabolism disorder,Fatty acid oxidation disorder,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
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 million Series A financing round.

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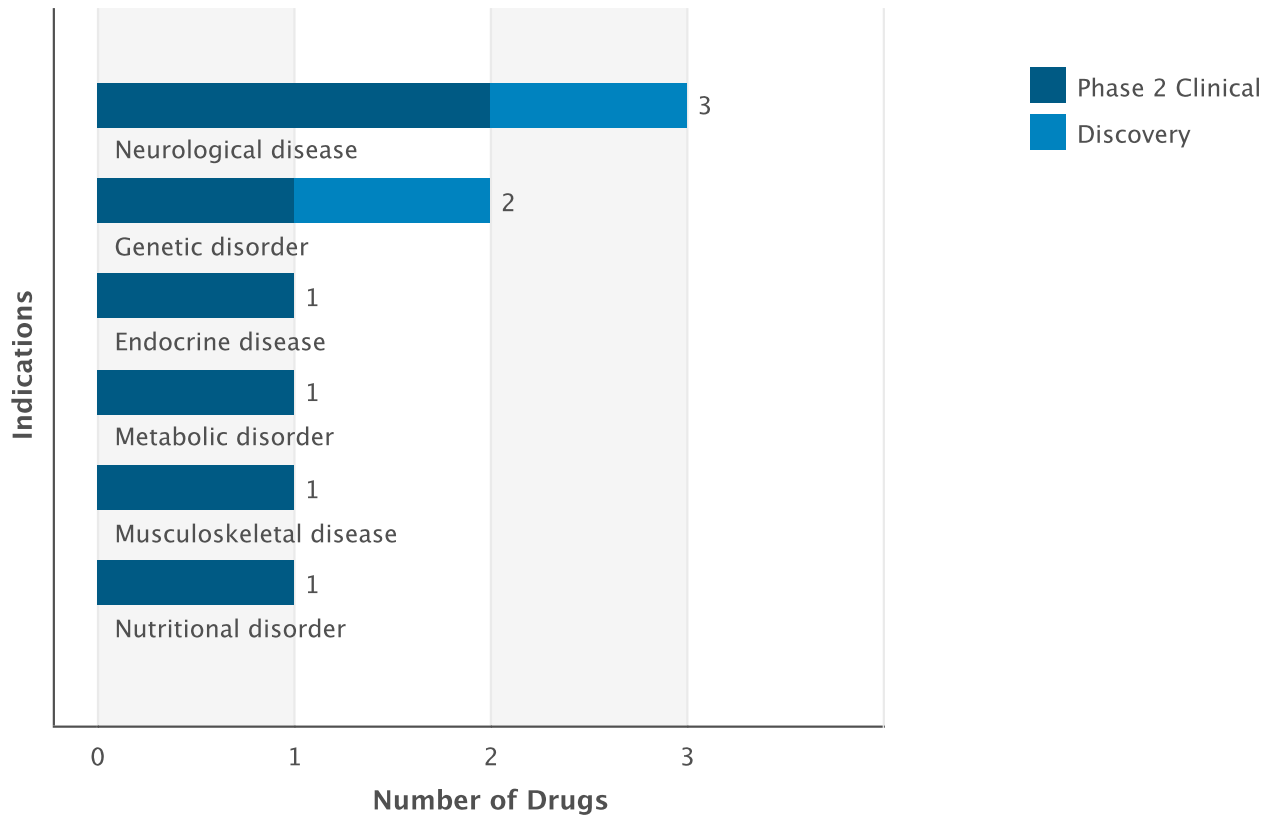
In June 2011, the company closed a $45 million Series A financing round.

PRODUCT PORTFOLIO SUMMARY

DRUGS

Drugs by Indication

Active Drugs by Indication Chart



Drugs by Indication Table

Indication	Active	Inactive	Total
Neurological disease	3	2	5
Genetic disorder	2	2	4
Nutritional disorder	1	0	1
Metabolic disorder	1	0	1
Endocrine disease	1	0	1
Musculoskeletal disease	1	0	1

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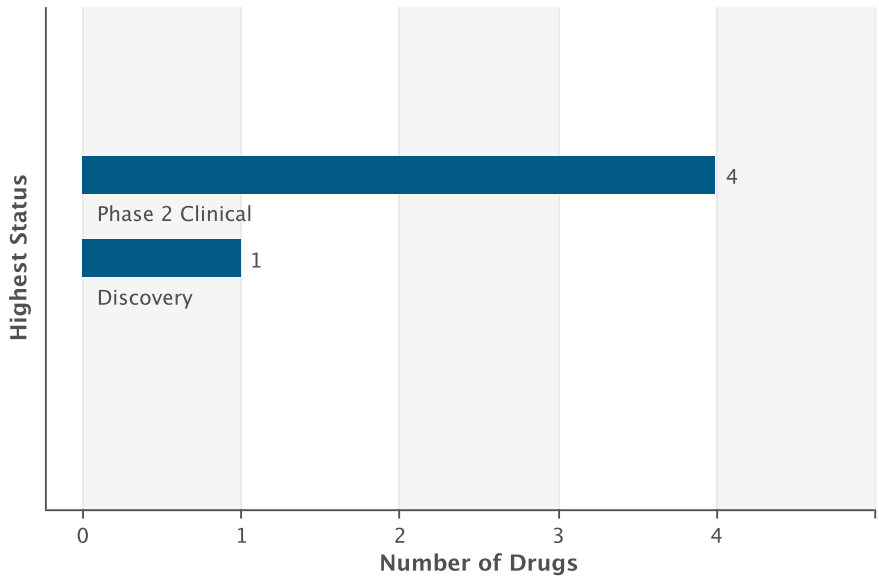
Drugs by Highest Status

Active Drugs by Highest Status Chart



Drugs by Highest Status Table

Development Status	Number of Drugs
Phase 2 Clinical	4
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	2	0	2
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

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CLINICAL TRIALS

Trials by Condition Studied

Condition Studied	Ongoing	All
Metabolic disorder	4	6
Neurological disease	2	5
Genetic disorder	2	3

Trials by Phase

Phase	Ongoing	All
Phase 3	1	1
Phase 2	6	9
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
Metabolic disorder	2	0	2
Neurological disease	8	1	9
Infectious disease	1	0	1
Inflammatory disease	3	2	5

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

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PRODUCT PORTFOLIO DRUGS

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

KRN-23

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
Target-based Actions	Fibroblast growth factor 23 ligand inhibitor
Other Actions	Immunomodulator, Phosphate raising agent
Technologies	Monoclonal antibody human, Immunoglobulin-G, Subcutaneous formulation, Intravenous formulation, Biological therapeutic, Protein recombinant
Last Change Date	11-Nov-2014

UX-001

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	Muscle system agent, Unspecified drug target
Technologies	Oral formulation, Sustained release formulation, Tablet formulation, Small molecule therapeutic
Last Change Date	11-Nov-2014

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UX-003

Drug Name	UX-003
Key Synonyms	
Originator Company	St Louis University
Active Companies	Ultragenyx Pharmaceutical Inc
Inactive Companies	St Louis University
Highest Status	Phase 2 Clinical
Active Indications	Lysosome storage disease, Sly syndrome
Target-based Actions	Beta-glucuronidase stimulator
Other Actions	
Technologies	Intravenous formulation, Infusion, Biological therapeutic, Recombinant enzyme
Last Change Date	09-Sep-2014

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

Drug Name	triheptanoin (oral, long-chain fatty acid oxidation disorders/glucose transporter type-1 deficiency syndrome), Baylor Research Institute/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	Fatty acid oxidation disorder, Carbohydrate metabolism disorder
Target-based Actions	Facilitated glucose transporter-1 stimulator
Other Actions	Lipid metabolism modulator
Technologies	Lipid, Oral formulation, Oral liquid formulation, Small molecule therapeutic
Last Change Date	11-Nov-2014

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UX-004

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

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