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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, DC 20549**

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**FORM 8-K**

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**CURRENT REPORT  
Pursuant to Section 13 or 15(d)  
of the Securities Exchange Act of 1934**

**Date of Report (Date of earliest event reported): September 18, 2017**

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**ULTRAGENYX PHARMACEUTICAL INC.**

(Exact name of registrant as specified in charter)

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**Delaware**  
(State or other jurisdiction  
of incorporation)

**001-36276**  
(Commission  
File Number)

**27-2546083**  
(IRS Employer  
Identification No.)

**60 Leveroni Court, Novato, California**  
(Address of principal executive offices)

**94949**  
(Zip Code)

**Registrant's telephone number, including area code: (415) 483-8800**

**Not Applicable**  
(Former name or former address, if changed since last report)

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Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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**Item 8.01. Other Events.**

On September 18, 2017, Ultragenyx Pharmaceutical Inc. (the “*Company*”) issued a press release (the “*Release*”) announcing that the Company has made a proposal to acquire all of the outstanding shares of common stock of Dimension Therapeutics, Inc. (“*Dimension*”) for \$5.50 per share in cash (the “*Proposal*”). A copy of the Release is filed herewith as Exhibit 99.1.

On September 18, 2017, the Company provided supplemental information (the “*Investor Presentation*”) regarding the Proposal in connection with an investor conference call held to discuss the Proposal. A copy of the Investor Presentation is filed herewith as Exhibit 99.2.

**Item 9.01 Financial Statements and Exhibits**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Press Release, dated September 18, 2017</a>
99.2	<a href="#">Investor Presentation, dated September 18, 2017</a>

\* \* \*

**SIGNATURE**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: September 18, 2017

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp

Name: Shalini Sharp

Title: Executive Vice President, Chief Financial Officer



## Ultragenyx Proposes to Acquire Dimension Therapeutics for \$5.50 Per Share in Cash

*Proposed Combination of Complementary Rare Disease Franchises Maximizes the Ability to Bring Needed New Therapies to Market*

*Proposal Delivers Superior Value To Dimension's Shareholders Relative to the REGENXBIO Transaction; Provides Dimension Shareholders with Immediate Cash Premium Value of Over 358% to Unaffected Share Price*

*Ultragenyx to Host Conference Call Today at 10:30am ET*

**NOVATO, Calif., September 18, 2017** – Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE) (“Ultragenyx” or the “Company”), a biopharmaceutical company focused on the development of novel products for rare and ultra-rare diseases, today announced that it has made a proposal to acquire all of the outstanding shares of common stock of Dimension Therapeutics, Inc. (NASDAQ: DMTX) (“Dimension”) for \$5.50 per share, or approximately \$138 million, in cash at close to be effectuated via a tender offer. The Ultragenyx offer represents a premium of over 358% to Dimension’s unaffected share price as of August 24, 2017 and premiums of 24% and 48% over the implied value of the all-stock consideration to be received by Dimension stockholders pursuant to the announced acquisition of Dimension by REGENXBIO Inc. (traded on NASDAQ under RGNX) (“REGENXBIO”), based on REGENXBIO’s last closing price and trailing 20-trading day volume-weighted average price as of September 15, 2017, respectively. As such, the proposal would provide Dimension stockholders with an immediate and certain return on their investment in Dimension and constitutes a superior alternative to the REGENXBIO transaction.

The proposal has been approved by the Board of Directors of Ultragenyx. Ultragenyx would fund the transaction from cash resources on its balance sheet and anticipates that customary closing conditions to the transaction could be satisfied so that the tender offer could complete as soon as 25 business days after merger agreement signing.

“This transaction provides a compelling opportunity to create value by leveraging Ultragenyx’s advanced clinical and regulatory expertise, as well as its rare metabolic disease commercial infrastructure to advance Dimension’s rare disease focused gene therapies and bring much needed new treatments to market,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “Based on my own experience as a scientific advisor to Dimension, I have the greatest respect for the deep expertise and knowledge of Dimension’s employees in AAV gene

therapy and manufacturing. We share Dimension's vision for bringing transformational new therapies to patients with rare genetic diseases and believe that bringing our two companies together would accelerate the process of bringing important new therapies to market for patients."

Dr. Kakkis continued, "Our all-cash offer provides meaningfully greater value and certainty to Dimension shareholders compared to the proposed all-stock acquisition by REGENXBIO. We believe Ultragenyx and its product candidates are highly complementary to Dimension's and present no competitive overlap, giving us confidence that we could combine our two companies quickly and seamlessly."

Below is the text of a letter that has been sent concurrent with this announcement to Dr. Annalisa Jenkins, President and Chief Executive Officer of Dimension:

September 18, 2017  
Dimension Therapeutics, Inc.  
840 Memorial Drive, 4<sup>th</sup> Floor  
Cambridge, Massachusetts 02139

Attention: Dr. Annalisa Jenkins, Chief Executive Officer

Dear Annalisa:

We at Ultragenyx Pharmaceutical Inc. have followed the progress of Dimension Therapeutics, Inc. ("Dimension" or the "Company") with great interest and are impressed by the advances you have made with your product candidates. The Dimension team has built an innovative and valuable business with a strong portfolio of assets and an advanced manufacturing platform that are poised to make significant advances in the treatment of patients with rare genetic diseases.

We share your vision to expand treatment options and bring transformational therapies to patients in areas with significant unmet need and we have a strong track record of advancing rare disease focused product candidates through the clinical and regulatory processes, including submission of marketing applications for two products in both the US and EU during this last year. As we head to commercialization for two products in 2018, we are best prepared to support the advancement and eventual filing for any products successfully developed from your portfolio. As experts in the metabolic disease space, our scientific, clinical, regulatory, and commercial skills would be complementary with your technology, programs and people. As such, we believe that a combination of our respective organizations will maximize the impact we can have for patients by bringing much-needed new therapies to market.

Our vision would be to leverage our significant clinical and regulatory expertise, as well as our growing rare metabolic disease commercial infrastructure, to advance Dimension's rare disease focused gene therapies through the clinic and to maximize their reach with patients. Furthermore, we recognize and value the deep expertise and knowledge that Dimension's employees have developed in AAV gene therapy and manufacturing, and we believe that their collective talents would be an impressive addition to Ultragenyx. We would envision maintaining a gene therapy development unit and manufacturing development team at Dimension's facilities in Massachusetts to continue to retain your strong team's significant institutional knowledge and efficiently progress your critical manufacturing there.

We are pleased to submit this non-binding proposal ("Proposal") to acquire Dimension for a value, consideration and structure that we believe represents a compelling opportunity for Dimension shareholders. In addition, this offer provides meaningfully greater value and certainty than the agreement recently reached with REGENXBIO Inc. ("REGENXBIO").

- 1) **Price, Consideration and Structure.** We are prepared to acquire 100% of the outstanding common stock of Dimension for \$5.50 per share ("Purchase Price") in cash to be effectuated via a tender offer.

This represents a premium of 358% to Dimension's unaffected share price as of August 24, 2017, and a 24% premium to the implied offer value of the REGENXBIO transaction, based upon REGENXBIO's closing price on September 15, 2017. This also represents a 48% premium to the implied offer value of the REGENXBIO transaction, based upon REGENXBIO's 20 trading day volume weighted average price of \$23.68 per share (per Bloomberg) as of September 15, 2017.

The \$5.50 per share offer implies an equity purchase price of approximately \$138 million in cash at close.

- 2) **Financing.** We have sufficient cash resources to fund this transaction with cash currently on our balance sheet, and our offer is not subject to any financing condition.
- 3) **Due Diligence and Timing.** This Proposal is based on our current knowledge of Dimension from the Company's public filings and disclosures and is subject to confirmatory due diligence that we expect can be addressed quickly and efficiently if

we are afforded access to a customary data room and appropriate Company personnel. We are prepared to move expeditiously to complete diligence with your assistance in a two-week period.

- 4) **Merger Agreement.** Concurrently with our due diligence review, we would anticipate working with you to negotiate a definitive agreement. We are prepared to accept identical or more favorable terms for Dimension than your existing merger agreement with REGENXBIO, as you will see in the enclosed version of our draft merger agreement showing changes from the existing agreement with REGENXBIO. Our draft merger agreement in fact offers greater speed and deal certainty than the pending transaction with REGENXBIO, in particular:
- a. We are proposing an all-cash transaction structured as a tender offer, which would expire as soon as 25 business days following entry into the merger agreement. We propose to close the deal on the business day after expiration of the tender offer. In contrast, the existing merger agreement with REGENXBIO is conditioned on SEC clearance of a registration statement by REGENXBIO and a Company shareholder approval.
  - b. We are willing to agree to an absolute “hell or high water” covenant to demonstrate our comfort and commitment in securing antitrust clearance for our acquisition of the Company. We believe Ultragenyx and its product candidates present no competitive overlap with the Company. In contrast, the existing merger agreement with REGENXBIO specifically provides that REGENXBIO will not be obligated to sell, dispose of or hold separate any assets of REGENXBIO or the Company in order to secure antitrust clearance.
  - c. We are proposing to bear any risk related to clinical data coming out of the Company’s ongoing trials before closing of the transaction, as reflected in our changes to the definition of “Company Material Adverse Effect” in our draft merger agreement.

The Ultragenyx board of directors has approved this Proposal. Subject to completing our due diligence and negotiating a mutually satisfactory definitive agreement to be executed upon your termination of the existing merger agreement with REGENXBIO, we will require final approval by our board of directors. No additional Ultragenyx internal approvals or shareholder approvals would be needed to consummate the transaction. Based on our current knowledge of Dimension from publicly available information, we do not believe that any other material approvals would be required for us to consummate the transaction, other than the expiration or early termination of the waiting period under the Hart-Scott-Rodino Act and, if applicable, any approvals under foreign antitrust laws.

This Proposal is not legally binding upon Ultragenyx, and no binding obligation shall arise for either party unless and until a definitive agreement has been duly executed between Ultragenyx and Dimension.

We believe that our Proposal constitutes a Superior Proposal (as defined in your existing merger agreement with REGENXBIO) and that your board of directors can and should, consistent with its fiduciary duties, make a determination to that effect. We urge you and your Board of Directors promptly to take those actions necessary under the existing merger agreement with REGENXBIO in order to afford us the opportunity to complete our due diligence and commence discussions with management and your advisors. The form of Acceptable Confidentiality Agreement (as defined in your existing merger agreement with REGENXBIO) can be provided to our General Counsel, Karah Parschauer, by email at [KParschauer@ultragenyx.com](mailto:KParschauer@ultragenyx.com). We have engaged Centerview Partners LLC and Skadden, Arps, Slate, Meagher & Flom LLP as financial and legal advisors, respectively, to assist us in this transaction and are prepared to move quickly to complete our diligence and negotiate a definitive agreement.

We have publicly disclosed this letter, simultaneously with sending it to you. We look forward to hearing from you and please do not hesitate to contact me or our advisors with any questions.

Sincerely,  
Dr. Emil D. Kakkis  
Chief Executive Officer and President  
Ultragenyx Pharmaceutical Inc.

#### **Advisors**

Centerview Partners LLC is serving as financial advisor to Ultragenyx, and Skadden, Arps, Slate, Meagher & Flom LLP is serving as Ultragenyx's legal advisor.

#### **Conference Call**

Ultragenyx will host a conference call today at 10:30 a.m. Eastern (7:30 a.m. Pacific). The live and replayed webcast of the call will be available through the company's website at [www.ultragenyx.com](http://www.ultragenyx.com). To participate in the live call by phone, dial (855) 797-6910 (USA) or (262) 912-6260 (international) and enter the passcode 86650959. The replay of the call will be available for one year.

#### **About Ultragenyx Pharmaceutical Inc.**

Ultragenyx is a biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. The Company has rapidly built and advanced a diverse portfolio of product candidates with the potential to address diseases for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

The Company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit the Company's website at [www.ultragenyx.com](http://www.ultragenyx.com).

#### **Forward Looking Statements / Additional Information**

Except for the historical information contained herein, the matters set forth in this communication, including statements of anticipated changes in the business environment in which Ultragenyx operates and in Ultragenyx's future prospects or results, statements relating to Ultragenyx's intentions, plans, hopes, beliefs, anticipations, expectations or predictions of its future, or statements relating to Ultragenyx's offer and the potential benefits of a transaction with Dimension Therapeutics, Inc. ("Dimension"), are forward-looking statements. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term

investments to fund operations and the availability or commercial potential of our drug candidates. There is no assurance that the potential transaction will be consummated, and it is important to note that actual results could differ materially from those projected in such forward-looking statements. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (the "SEC") on July 28, 2017, and its subsequent periodic reports filed with the SEC.

The tender offer referred to in this communication (an "Offer") has not yet commenced. Accordingly, this communication is for informational purposes only and does not constitute an offer to purchase or a solicitation of an offer to sell any shares of Dimension common stock or any other securities. On the commencement date of any Offer, a tender offer statement on Schedule TO, including an offer to purchase, a letter of transmittal and related materials, will be filed with the SEC by Ultragenyx and a wholly owned subsidiary. The offer to purchase shares of Dimension common stock will only be made pursuant to the offer to purchase, letter of transmittal and related materials filed with the SEC by Ultragenyx as part of its Schedule TO. Investors and security holders are urged to read both the tender offer statement and any solicitation/recommendation statement filed by Dimension regarding the Offer, as they may be amended from time to time, when they become available, because they will contain important information about the Offer, including its terms and conditions, and should be read carefully before any decision is made with respect to the Offer. Investors and security holders may obtain free copies of these statements (when available) and other materials filed with the SEC at the website maintained by the SEC at [www.sec.gov](http://www.sec.gov), or by directing requests for such materials to the information agent for the Offer, which will be named in the tender offer statement.

## **Contacts**

Investor Relations:  
Ryan Martins  
415-483-8257

Media Relations:

Joele Frank, Wilkinson Brimmer Katcher  
Tim Lynch / Trevor Gibbons / Leigh Parrish  
212-355-4449



# Proposal for Ultragenyx to Acquire Dimension Therapeutics

September 18, 2017

[www.ultragenyx.com](http://www.ultragenyx.com)

# Forward Looking Statements / Additional Information

Except for the historical information contained herein, the matters set forth in this communication, including statements of anticipated changes in the business environment in which Ultragenyx operates and in Ultragenyx's future prospects or results, statements relating to Ultragenyx's intentions, plans, hopes, beliefs, anticipations, expectations or predictions of its future, or statements relating to Ultragenyx's offer and the potential benefits of a transaction with Dimension Therapeutics, Inc. ("Dimension"), are forward-looking statements. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of our regulatory filings and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our drug candidates. There is no assurance that the potential transaction will be consummated, and it is important to note that actual results could differ materially from those projected in such forward-looking statements. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (the "SEC") on July 28, 2017, and its subsequent periodic reports filed with the SEC.

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- **Ultragenyx has submitted a proposal to acquire Dimension Therapeutics for \$5.50 per share in cash**
  - Total equity purchase price of approximately \$138mm<sup>(1)</sup> in cash at close
  - Premium of 358% to Dimension's unaffected share price as of August 24, 2017
  - Premiums of 24% and 48% to implied value of REGENXBIO's all stock agreement assuming latest closing stock price<sup>(2)</sup> and 20-day volume-weighted average trading price<sup>(3)</sup>, respectively
  
- **Offer provides both greater value and certainty for Dimension shareholders**
  - Provides Dimension shareholders with substantial premium in cash and certain value at close
  - No financing contingency – funded with Ultragenyx current cash on the balance sheet
  - Proposed merger agreement and tender offer provides greater certainty of and speed to close
  - Proposal has been approved by the Board of Directors of Ultragenyx

(1) Assumes 25.0mm basic shares outstanding based on REGENXBIO-Dimension merger agreement filed on 8/25/17.

(2) REGENXBIO closing price of \$28.25 per share as of 9/15/17.

(3) REGENXBIO 20 trading day VWAP of \$23.68 per share (per Bloomberg) as of 9/15/17 and 8/25/17 announced exchange ratio of 0.1573 REGENXBIO shares per Dimension share.

# Our Strategy

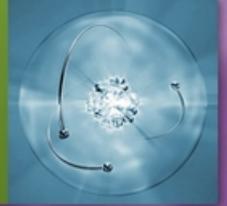
Transforming good science into great medicine for rare genetic diseases



HOW  
HAVE WE  
DONE IT?

HOW DO WE  
CONTINUE  
DOING IT?

CLEAR BIOLOGY



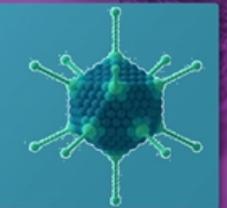
RAPID DEVELOPMENT



GLOBAL VISION



NEW MODALITIES



## 5 things we look for in an opportunity:

- ✓ Rare and ultra-rare disease
- ✓ Genetic disease with clear biology
- ✓ Potential for meaningful clinical benefit
- ✓ Translating existing science to clinic rapidly
- ✓ Global rights to key assets



***Satisfies all of  
these criteria***

# Expanding into New Modalities & Capabilities:

Optimizing solutions to genetic diseases with clear biology



## Deep Understanding of Genetic Disease Drug Development



### Advantages

- ✓ Oral delivery
- ✓ Simple manufacturing

- ✓ High specificity targeting
- ✓ Long half life

- ✓ Ability to target "undruggable" targets
- ✓ Genomics-based discovery process

- ✓ *Potential for one-time treatment for monogenic diseases*

### Current Capabilities

- ✓ In late-stage clinical development:
  - Substrate replacement

- ✓ In late-stage clinical development:
  - Fully human monoclonal antibodies
  - Enzyme replacement therapies

- ✓ In preclinical development:
  - 2 programs using mRNA / lipid nanoparticle delivery

- ✓ *In early clinical development:*
  - *1 AAV gene transfer program in the clinic*
  - *2 more entering the clinic in the next 6-12 months*

## Company Overview

- Clinical-stage biopharmaceutical company developing AAV-based gene therapies for rare metabolic diseases associated with the liver
- Holds exclusive license to various AAV vectors from REGENXBIO
  - Option to license for additional indication not yet determined
  - Cannot be terminated by REGENXBIO due to a change of control of Dimension
- Manufacturing capabilities and expertise:
  - Up to 250L bioreactor capacity at Woburn, MA site
  - Partnering with CMOs with up to 1000L bioreactor capacity
- Founded in 2013

## Lead Metabolic Product Candidates

### DTX301 – *OTC Deficiency (OTC Gene – AAV8)*

- X-linked, urea cycle disorder (UCD)
- Genetic defect in ammonia detoxification
- Adverse cognitive & neurological effects, coma, death
- Treatments limited, non-curative
- ~10,000 patients WW
- First patient now treated

### DTX401 – *GSD1a (G6Pase Gene – AAV8)*

- Autosomal recessive, inborn error of glucose metabolism
- Deficient glucose-6-phosphatase (G6Pase)
- Severe hypoglycemia and hepatomegaly caused by the accumulation of glycogen
- Strict diet and frequent feedings of uncooked corn starch or Glycosade®
- ~6,000 patients WW diagnosed at birth

# Proposed Acquisition of Dimension Would Diversify Ultragenyx's Clinical and Preclinical Pipeline



Candidate	Description	Stage -2 to 0	IND	Phase 1	Phase 2	Phase 3	Filed	Patients Worldwide
<b>Burosumab (KRN23)</b> <b>KYOWA KIRIN</b>	Anti-FGF23 monoclonal antibody	XLH						~48,000
		TIO						~2,000-4,000
<b>rhGUS (UX003)</b>	Enzyme replacement	MPS 7						~200
<b>UX007</b>	Substrate replacement	LC-FAOD						~8,000-14,000
		Glut1 DS						~12,000-28,000
<b>DTX301</b>	AAV8-OTC Gene Transfer	OTCD						~10,000
<b>DTX401</b>	AAV8-G6Pase Gene Transfer	GSD1a						~6,000
<b>DTX201</b>	AAV-FVIII Gene Transfer	Hemophilia A						~144,000
<b>UX004</b>	rhPPCA ERT	Galactosialidosis						~200
<b>DTX701</b>	AAV-ATP7B Gene Transfer	Wilson Disease						>50,000
<b>UX034</b>	mRNA/Lipid							>20,000
<b>DTX501</b>	AAV8-PAH Gene Transfer							~50,000
<b>DTX601</b>	AAV8-ASS1 Gene Transfer							~2,000
<b>UX068</b>	Substrate replacement							>20,000
<b>UX001P</b>	Substrate replacement							~2,000
<b>TAK418 Takeda</b>	Undisclosed							>50,000
<b>UX053</b>	mRNA/Lipid							>10,000
<b>Undisclosed</b>	New mechanism							>50,000



Note: Based on Dimension corporate presentations.

# Proposed Acquisition of Dimension Would Add Significant Upcoming Milestones

Candidate	Disease	2H17	2018
<b>Burosumab</b> (UX023) <b>KYOWA KIRIN</b>	XLH	<b>CHMP Opinion</b> (end 2017)	<b>BLA Result</b> Peds Ph 3 Data
	TIO	Phase 2 Bone Data	
<b>rhGUS</b> (UX003)	MPS7	<b>PDUFA:</b> Nov 16, 2017	<b>CHMP Opinion</b>
<b>UX007</b>	LC-FAOD	Phase 3 study design/Reg. path	
	Glut1 DS	Phase 3 Movement Disorder study ongoing	Ph 3 Data
<b>DTX301</b>	OTC Deficiency	Phase 1/2 Data	
<b>DTX401</b>	GSD1a		IND Filing
<b>DTX201</b>	Hem A		IND Filing



# Ultragenyx's Rare Disease Expertise

Ultragenyx's integrated rare disease capabilities could accelerate the development of Dimension's programs



**Innovation in rare disease drug development, study & endpoint design, patient diagnosis**

**Experience partnering with rare disease patient communities through Patient Advocacy function**

**Senior leadership with track record of success in development and commercialization of multiple rare diseases**

**Global regulatory expertise in registration strategy for first ever treatments, biometrics analyses, global submissions and accelerated time to market**

**Burosumab and rhGUS global commercial organization will be leveraged to support potential Dimension candidate launches**

*Ultragenyx plans to maintain a gene therapy development unit and manufacturing team at Dimension's facilities in Massachusetts to retain the team's significant institutional gene therapy knowledge and efficiently progress critical manufacturing*

## Acquisition Terms:

Ultragenyx offer constitutes a superior proposal for Dimension shareholders



### Compared to REGENXBIO's Proposal, Ultragenyx Proposal Offers Superior...

- ✓ Value
  - 24% premium to implied value assuming REGENXBIO's latest closing stock price<sup>(1)</sup>
  - 48% premium over 20-Day VWAP<sup>(2)</sup>
- ✓ Certainty
  - All cash tender offer
- ✓ Speed to close
- ✓ Proposed merger agreement increases deal certainty for Dimension

### Ultragenyx Balance Sheet:

- Cash<sup>(3)</sup> (as of June 30, 2017):  
**\$457.5 million**
- **No debt**



(1) Based on closing price of \$28.25 on 9/15/17.

(2) Based on REGENXBIO 20 trading day VWAP of \$23.68 per share (per Bloomberg) as of 9/15/17.

(3) Cash, cash equivalents, and short-term investments.

# Conclusion & Expected Next Steps

Aligns with Ultragenyx's focus on **patients** and meeting the challenges of diseases with **high unmet need**

Provides Ultragenyx with **new modalities and capabilities** and expands further in rare genetic diseases

Ultragenyx brings global capabilities and resources to accelerate **development and commercialization** of Dimension's assets

Superior proposal in terms of both **value and certainty** to Dimension's shareholders

## Expected Next Steps

- Ultragenyx executes CDA and conducts expedited due diligence after Dimension decides to engage with Ultragenyx
- Ultragenyx and Dimension negotiate a merger agreement and Ultragenyx submits a binding proposal informed by due diligence
- Dimension's Board of Directors makes "Superior Proposal" determination as defined by the merger agreement executed with REGENXBIO
- Dimension terminates merger agreement with REGENXBIO in favor of a transaction with Ultragenyx
- Dimension and Ultragenyx enter into a merger agreement
- Launch tender offer and obtain customary approvals
- Potential for tender offer to complete as soon as 25 business days after merger agreement signing



## Appendix

[www.ultragenyx.com](http://www.ultragenyx.com)

# Overview of Dimension's Additional Inherited Metabolic Disease Portfolio

## Wilson Disease

- Autosomal recessive copper metabolism disorder
- Deficiency in copper transporter P-type ATPase, *ATP7B*
- Copper in liver, brain, kidney, cornea; chelation & strict diet
- WW prevalence >50K patients; incidence 1 in 30K

## PKU

- Autosomal recessive disorder, inability to metabolize phenylalanine (Phe)
- Deficiency in phenylalanine hydrolase (PAH)
- Reduce Phe to prevent cognitive decline; strict diet low in Phe & protein
- ~50K prevalent patients developed world; incidence 1 in 10K – 40K

## Citrullinemia Type 1

- Autosomal recessive UCD due to inability to metabolize ammonia
- Deficiency in argininosuccinate synthetase (ASS1)
- Decompensation risk, neurological effects; nitrogen scavengers, strict diet
- 14% of all UCDs; WW prevalence ~2K; incidence ~1 in 60K+

- IND filing expected in early 2018
- Dimension responsible for preclinical activities and Phase 1/2 clinical trial, funded by Bayer
- Bayer to fund subsequent trials and commercial activities at clinical POC

- 
- R&D expense reimbursement
  - Up to \$232M in milestones
  - High single-digit to low double-digit royalties not exceeding the mid-teens

