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# EDITED TRANSCRIPT

RGNX - Regenxbio Inc to Acquire Dimension Therapeutics Inc

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## Important Additional Information Will Be Filed With the SEC

This communication does not constitute an offer to sell or the solicitation of an offer to buy any securities or a solicitation of any vote or approval with respect to the proposed acquisition by REGENXBIO of Dimension. No offer of securities shall be made except by means of a prospectus meeting the requirements of Section 10 of the Securities Act of 1933, as amended, and no offer to sell or solicitation of an offer to buy shall be made in any jurisdiction in which such offer, solicitation or sale would be unlawful.

In connection with the proposed transaction, REGENXBIO plans to file with the SEC a Registration Statement on Form S-4 containing a proxy statement of Dimension and a prospectus of REGENXBIO, and each of REGENXBIO and Dimension may file with the SEC other documents regarding the proposed transaction. The definitive proxy statement/prospectus will be mailed to stockholders of Dimension. STOCKHOLDERS OF DIMENSION ARE URGED TO READ THE REGISTRATION STATEMENT AND THE PROXY STATEMENT/PROSPECTUS REGARDING THE ACQUISITION CAREFULLY AND IN THEIR ENTIRETY WHEN THEY BECOME AVAILABLE AND ANY OTHER DOCUMENTS FILED WITH THE SEC BY REGENXBIO AND DIMENSION BECAUSE THEY WILL CONTAIN IMPORTANT INFORMATION ABOUT THE PROPOSED TRANSACTION. Investors will be able to obtain free copies of the Registration Statement and the proxy statement/prospectus (when available) and other documents filed with the SEC by REGENXBIO and Dimension through the website maintained by the SEC at [www.sec.gov](http://www.sec.gov). Free copies of the Registration Statement and the proxy statement/prospectus (when available) and other documents filed with the SEC can also be obtained by directing a request to REGENXBIO Inc., 9600 Blackwell Road, Suite 210, Rockville, Maryland 20850, or by directing a request to Dimension Therapeutics, Inc., 840 Memorial Drive, Cambridge, Massachusetts 02139.

### **Participants in the Solicitation**

REGENXBIO, Dimension and their respective directors and certain of their executive officers and employees may be deemed to be participants in the solicitation of proxies in respect of the proposed transaction. Information regarding REGENXBIO's directors and executive officers is available in its proxy statement for its 2017 annual meeting of stockholders, which was filed with the SEC on April 13, 2017, and information regarding Dimension's directors and executive officers is available in its proxy statement for its 2017 annual meeting of stockholders, which was filed with the SEC on April 14, 2017. Information regarding the persons who may, under the rules of the SEC, be deemed participants in the proxy solicitation and a description of their direct and indirect interests, by security holdings or otherwise, will be contained in the proxy statement/prospectus and other relevant materials to be filed with the SEC when they become available.

## CORPORATE PARTICIPANTS

**Kenneth T. Mills** *Regenxbio Inc. - CEO, President and Director*

**Patrick J. Christmas** *Regenxbio Inc. - SVP and General Counsel*

**Vittal K. Vasista** *Regenxbio Inc. - CFO*

## CONFERENCE CALL PARTICIPANTS

**Gbolahan Amusa** *Chardan Capital Markets, LLC, Research Division - Director of Research and Head of Healthcare Research*

**Joshua Elliott Schimmer** *Evercore ISI, Research Division - Senior MD & Equity Analyst*

**Ying Huang** *BofA Merrill Lynch, Research Division - Director in Equity Research*

## PRESENTATION

### Operator

Good day, ladies and gentlemen, and welcome to the REGENXBIO Conference Call. (Operator Instructions) As a reminder, this conference call may be recorded.

I would now like to introduce your host for today's conference, Mr. Patrick Christmas, Senior Vice President and General Counsel of REGENXBIO. You may begin.

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**Patrick J. Christmas** - *Regenxbio Inc. - SVP and General Counsel*

Good morning, and thank you for joining us today. With us are Ken Mills, REGENXBIO's President and Chief Executive Officer; and Vit Vasista, our Chief Financial Officer. Earlier this morning, REGENXBIO announced that it has entered into a definitive agreement to acquire Dimension Therapeutics. This press release is available on our website at [www.regenxbio.com](http://www.regenxbio.com).

Today's conference call will include forward-looking statements, including without limitation statements relating to the impact REGENXBIO expects its proposed acquisition of Dimension Therapeutics to have on the combined entity's operations and regarding our financial outlook, in addition to regulatory and product development plans. These forward-looking statements are subject to risks and uncertainties that may cause actual results or developments to differ from those projected in the forward-looking statements. Any such forward-looking statements are not guarantees of future performance and involve certain risks and uncertainties. These risks are described in the Risk Factors and Management's Discussion and Analysis section of REGENXBIO's annual report on Form 10-K for the year ended December 31, 2016, and comparable risk factor sections of REGENXBIO's quarterly report on Form 10-Q for the quarter ended June 30, 2017, which are on file with the Securities and Exchange Commission and available on the SEC's website and other filings we may make with the SEC.

Any information we provide on this conference call is provided only as of the date of this call, August 25, 2017, and we undertake no obligation to update any forward-looking statements we may make on this call, whether as a result of new information, future events or otherwise. Please be advised that today's call is being recorded and webcast. In addition, any unaudited or pro forma financial information that may be provided is preliminary and does not purport to project financial positions or operating results of the company. Actual results may differ materially.

I would now like to turn the call over to Ken Mills, President and Chief Executive Officer of REGENXBIO.

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**Kenneth T. Mills** - *Regenxbio Inc. - CEO, President and Director*

Thank you, Patrick, and good morning, everyone. Thanks for joining us. We were very pleased to announce earlier this morning that we signed a merger agreement to acquire Dimension Therapeutics in an all-stock transaction. We believe this acquisition will enhance our leadership position

in the field of AAV gene therapy and will further our mission of improving the lives of patients through the curative potential of gene therapy. Dimension's assets are a strong strategic fit with our mission and with our current development efforts, and we believe this transaction creates substantial value for shareholders of both companies.

Once closed, the transaction will immediately expand REGENXBIO's metabolic disease franchise, one of our 3 internal focus areas, with the addition of a clinical stage product candidate, DTX301, and a product candidate nearing IND filing, DTX401. All Dimension candidates, including DTX301 and DTX401, utilize REGENXBIO's NAV Technology and have been developed under exclusive licenses previously granted by REGENXBIO to Dimension.

Our current pipeline has 3 clinical stage product candidates in RGX-314 for wet AMD, RGX-501 for treatment of HoFH, and RGX-111 recently approved for clinical trial for the treatment of MPS I. Dimension's 2 lead product candidates coupled with our existing pipeline will strengthen our position as a leading AAV gene therapy company and deliver one of the industry's broadest clinical stage pipelines in 2018 and beyond.

Following the closing of the acquisition of Dimension, we would have 4 clinical stage product candidates and 2 additional INDs expected to be filed in the next 6 to 12 months. For those not familiar with the 2 lead product candidates that we will add as part of the acquisition of Dimension, I will now provide an overview of the diseases and their development status.

Starting with DTX301 for the treatment of ornithine transcarbamylase deficiency, or OTC deficiency. OTC deficiency is a rare X-linked disorder that creates an accumulation of ammonia in the blood. OTC deficiency is the most common of the class of diseases called the urea cycle disorders. It's caused by mutations in the OTC gene itself, which is responsible for producing the ornithine transcarbamylase enzyme. The defective gene and resulting enzyme deficiency interrupts the urea cycle, a sequence of reactions that occur in liver cells and results in nitrogen accumulation in the bloodstream, which takes the form of excesses in ammonia. This ammonia accumulation causes severe adverse cognitive and neurological effects and can ultimately lead to coma and death.

The urea cycle disorder's consortium estimates that approximately 10,000 patients are affected by OTC deficiency worldwide, although many cases may go misdiagnosed or undiagnosed. Current treatment options are limited. OTC deficiency is managed through special daily amino acid formulas that provide a portion of dietary protein allowance. Poor compliance with this regimen, which is common, can be life-threatening.

While these treatments may help, the patient still experience hyperammonemic crises. Liver transplant is also an option for severe patients, but this approach is associated with significant morbidity and mortality, creating a significant unmet need for patients suffering with the disease. As such, a gene therapy approach has the potential to significantly and positively impact the treatment paradigm of the OTC deficiency patient population.

DTX301 uses the NAV AAV8 vector to deliver a healthy copy of the OTC gene to liver cells in a onetime intravenous administration. The Phase I and II clinical trial for DTX301, a global multicenter open label multi-cohort dose escalation trial, is currently recruiting participants; 10 sites currently opened in the United States, U.K., Spain and Canada. This product candidate is supported by strong in vivo data, which shows stable gene expression mediated by NAV Technology, enhanced survival and prevention and reduction of hyperammonemia in animals.

Now I will turn to DTX401, which is for the treatment of glycogen storage disease type Ia. GSDIa, which is the most common genetically inherited glycogen storage disease, is a rare autosomal recessive disorder caused by mutations in the G6PC gene that results in a deficiency of glucose-6-phosphatase, or G6Pase. G6Pase is necessary for breaking down sugar molecule glucose-6-phosphatase. When glucose-6-phosphate is not broken down, it is converted to glycogen or fat storage within cells and this can cause hypoglycemia or low blood sugar. The hypoglycemia in GSDIa patients is severe, however, and can be life-threatening, while the accumulation of glycogen in certain organs and tissues can impair the ability of the tissues to function normally.

The Children's Fund for GSD Research estimates that 6,000 or more patients are affected by this disease worldwide. Affected children must be fed every 1 to 4 hours in order to maintain blood glucose at an appropriate level. Many affected children [require] a gastric or nasal gastric tube in place to allow for overnight feedings. While most patients progress to adulthood, individuals with GSDIa must maintain strict diets to ensure maintenance of normal glucose levels. Patients who are chronically untreated develop severe lactic acidosis, which can progress to renal failure.

Reduced quality of life experienced by GSDIa due to the need for a strict diet and frequent feedings represents a significant unmet need. The only FDA-approved therapy is a dietary product, which does not address the underlying disease. An effective gene therapy with the potential to replace the defective G6PC gene could significantly improve the prognosis for affected children and adults suffering from the disease who must regularly confront the burden, the standard of care treatment and the overhang of the significant risks which emerge from noncompliance with the treatment.

DTX401 also uses the NAV AAV8 vector, in this case to deliver a healthy copy of the G6PC gene to liver cells. As with DTX301, DTX401 is designed as a onetime intravenous administration. Preclinical animal models have demonstrated that an AAV gene therapy may be able to produce stable gene expression, enhance survival and the prevention of hypoglycemia. An IND application is anticipated to be filed for DTX401 in early 2018.

In addition, once closed, the proposed acquisition of Dimension will add the product candidate DTX201 for the treatment of hemophilia A to our pipeline. DTX201 is designed to use REGENXBIO's NAV Technology to deliver a copy of the Factor VIII gene to liver cells, and is partnered through a global development and commercialization collaboration with Bayer.

Finally, once closed, the proposed acquisition also brings to REGENXBIO preclinical product candidates for PKU, Wilson disease and citrullinemia type I, manufacturing technologies and other intellectual properties developed by Dimension.

I would now like to turn the call over to Vit to just briefly describe the terms of the transaction.

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**Vittal K. Vasista** - *Regenxbio Inc. - CFO*

Thank you, Ken. As outlined in our press release, we intend to acquire Dimension in an all-stock transaction for an implied value of approximately \$3.41 per share. Under the terms of the agreement, Dimension will become a wholly-owned subsidiary of REGENXBIO. Dimension shareholders will receive 0.1573 shares of REGENXBIO in exchange for each of their shares in Dimension and are expected to own approximately 10.9% of REGENXBIO at closing.

It is anticipated that the transaction will close by year-end 2017, pending approval by Dimension shareholders, receipt of any required customary regulatory approvals and the satisfaction of other customary closing conditions. We expect to provide guidance for the combined company in early 2018 after the close of the transaction.

I will now turn the call back to Ken for some closing comments.

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**Kenneth T. Mills** - *Regenxbio Inc. - CEO, President and Director*

Thanks, Vit. So we believe the proposed acquisition announced today will create significant value for REGENXBIO and Dimension shareholders, as we confirm REGENXBIO's leadership position in the AAV gene therapy space. The proposed acquisition allows REGENXBIO to acquire 2 leading product candidates that use our NAV AAV8 vector and expand our metabolic disease franchise.

DTX301 and DTX401 are product candidates that address diseases with high unmet need and will become an important part of a strong internal pipeline at REGENXBIO that has the potential to achieve multiple milestones through the end of 2018 and beyond. These milestones will start with our expected interim updates anticipated by the end of this year for RGX-314 for wet AMD and RGX-501 for HoFH.

Along with DTX301 and DTX401, the transaction provides REGENXBIO with a collaboration with a leading biopharmaceutical company in Bayer, additional preclinical assets, technology, capabilities and intellectual property. The acquisition of Dimension is another meaningful step in building a robust clinical pipeline of gene therapy product candidates, with the goal of improving treatment options for patients and families in many diseases. The proposed acquisition fits squarely within our mission of improving the lives of patients through the curative potential of gene therapy based on our proprietary NAV Technology Platform, and we look forward to sharing our progress on each of these important product candidates with you all in the future.

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With that, I would like to open up the call now for any questions. Operator?

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## QUESTIONS AND ANSWERS

### Operator

(Operator Instructions) And our first question comes from Ying Huang from Bank of America Merrill Lynch.

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**Ying Huang** - *BofA Merrill Lynch, Research Division - Director in Equity Research*

Maybe, first of all, Ken, can you talk a little bit about the diagnosis of the OTC syndrome? It sounds like it might be a little bit difficult to diagnose compared to the other program? And then secondly, do you guys have sufficient manufacturing capacity to bring both of these programs into the Phase I clinical trial? And then lastly, maybe Vit can talk a little bit about what the implication is for the R&D cost for you guys going forward.

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**Kenneth T. Mills** - *Regenxbio Inc. - CEO, President and Director*

Sure. Thanks, Ying. Those are all great questions and I'll take them in order. So I think there is — in the urea cycle disorder space, I think OTC deficiency is one of the more well understood and more commonly diagnosed of the urea cycle disorders. And I think that there certainly is a spectrum of disease that presents across the natural history of the population. In some cases, diagnosis comes quite early because there's infant crisis. In other cases, certainly, there are less-severe circumstances for patients with OTC deficiency. But I think with sort of a better awareness, including treatments that have emerged over the last several years, and the fact that the natural history of the disease, certainly, can at times create a spectrum of symptoms that, I think, physicians worldwide have become more familiar with, I think the evolution of the diagnosis of the disease is going exactly in the direction that sets up well for the development and support of a treatment for OTC deficiency. With respect to the second question, our plans throughout 2016 and 2017, Ying, have been focused on ensuring that we have the capabilities to support manufacturing for clinical and later stage with respect to our 4 lead product candidates, but — as well as anticipating the growth of the company in terms of adding pipeline programs. So we're right on track to be able to support the addition of programs, including the DTX301 and DTX401. And lastly, Vit, do you want to comment about what sort of guidance we're giving right now on R&D spend?

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**Vittal K. Vasista** - *Regenxbio Inc. - CFO*

Sure, Ken. Thank you, Ying, for the question. As I — as we mentioned earlier, I think that as we go through the process of integration, we'll get a much better handle on our anticipated spending in 2018 as it relates to R&D. But based on the fact that we are adding one clinical stage program and another program that we'll be moving into the clinic, it would be anticipated that R&D spending should decrease in 2018 over what our original plans were.

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### Operator

And our next question comes from Josh Schimmer from Evercore ISI.

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**Joshua Elliott Schimmer** - *Evercore ISI, Research Division - Senior MD & Equity Analyst*

I guess, first, given the relationship with Dimension-REGENX, are there any factors such as contractual or progress obligations to consider that might preclude another bidder from emerging?

**Kenneth T. Mills** - Regenxbio Inc. - CEO, President and Director

Josh, this is Ken. No, the board of both companies unanimously approved the acquisition. And all factors were considered during the process through which the parties came to agreement.

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**Joshua Elliott Schimmer** - Evercore ISI, Research Division - Senior MD & Equity Analyst

Okay. Any reason to believe that the Dimension IP may extend beyond their development stage programs, either into other systemically administered AAV gene therapy approaches or liver-targeted ones?

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**Kenneth T. Mills** - Regenxbio Inc. - CEO, President and Director

I think that there is a portfolio of intellectual property that exists within Dimension associated with its lead product candidates that has claims that have the opportunity to extend beyond literal implementation of the disease-targeted areas. This is intellectual property that as it becomes part of the larger intellectual property portfolio of REGENXBIO, we'll be exercising in as broad and deep a way as possible.

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**Joshua Elliott Schimmer** - Evercore ISI, Research Division - Senior MD & Equity Analyst

Got it. And since you have had separate manufacturing efforts, do you plan to consolidate them and how much effort or time would that take to accomplish?

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**Kenneth T. Mills** - Regenxbio Inc. - CEO, President and Director

Yes. This area of manufacturing between the 2 companies is one where there are actually fantastic synergies and sort of an excellent nucleus of both common understanding of technology, but also common use of technology on both sides. So we view the opportunity of the acquisition to provide a really excellent platform for just continuing to interrogate, basically, our — as you know our million cell culture production processes that we think will support not only the expansion of additional programs, but be able to support us all the way through commercialization, I think. REGENXBIO and Dimension have always been and continue to be very aligned on that, and so it's going to be a great fit post acquisition as well.

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**Joshua Elliott Schimmer** - Evercore ISI, Research Division - Senior MD & Equity Analyst

And then in terms of the timing for data readout for 301 and 401, when might we first get updates?

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**Kenneth T. Mills** - Regenxbio Inc. - CEO, President and Director

Yes. I think we, certainly, will be able to recompile the entire new combined pipeline of REGENXBIO post acquisition. Currently, Dimension is in the process of beginning recruitment and enrollment in the 301 study and has given guidance for interim update of data by the end of 2017. I think there's no guidance at this point other than the IND filing for DTX401. And again, I think this is something that, as a team, we'll be able to revisit and support new guidance post closing, typically, around the time that we're beginning our efforts to talk with all of you about our plans for 2018, in any event.

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**Joshua Elliott Schimmer** - Evercore ISI, Research Division - Senior MD & Equity Analyst

Got it. Great to see these assets have an opportunity to move forward with a little bit more speed.

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**Operator**

(Operator Instructions) And our next question comes from Gbola Amusa from Chardan Capital.

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**Gbolahan Amusa - Chardan Capital Markets, LLC, Research Division - Director of Research and Head of Healthcare Research**

It's Gbola from Chardan. So, Ken, you're — you've been a Dimension partner, so you had information that the market doesn't have, heading into this acquisition. And could you talk a little bit about the types of information, not the information itself, but the type of information you had that made you see value in Dimension that perhaps the market wasn't seeing? And then second question is Dimension shares dropped almost precipitously at the beginning of the year due to some data which were questioned by the market in hemophilia B. And so my question there is to what extent are you comfortable with the issues with that product or the fact they were limited and shouldn't be extrapolated to the other programs?

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**Kenneth T. Mills - Regenxbio Inc. - CEO, President and Director**

Sure. Thanks, Gbola. Good to hear from you. So as you alluded to, Dimension Therapeutics is a NAV Technology licensee of REGENXBIO, among 10 other NAV Technology licensees that we have currently. And so in the course of us being license owners of technology, we, of course, have agreements that govern both sort of diligence and communication between the parties. And I think it definitely, under confidential agreements and terms governed under those licenses provides us updates from our NAV Technology licensees on their progress and advancement of programs, of which there are over 20 in development, if we include all of the Dimension programs in that. And so what we are able to see through those contracts and understandings outside of what's publicly available is, I think, important to understanding how the NAV Technology Platform is working in different disease areas, both at the preclinical and clinical stages. And I think we have always said and feel strongly that that's a real benefit and advantage within this sort of nucleus of decision making of REGENXBIO to be able to internalize that information, to make good decisions about how to bring to bear great programs in our pipeline, both our internal programs and ultimately the opportunity to acquire additional programs, those that even may be in existence extramurally from REGENXBIO. And this is absolutely a case of that, where we have a technology, understanding, an understanding of a licensee in the work that they were doing and absolutely saw a fantastic value created from the work that was done at Dimension and feel that we can provide a platform for expanding the value in opportunity space around DTX301 and DTX401, in particular. Alluding back to the beginning of the year and the reporting of data by Dimension with respect to DTX101, that was certainly information that I think was important to share with the field, with respect to AAV gene therapy. We, I think, like many of our licensees, recognized how innovative the development of AAV gene therapy is and how important it is, at this stage, to be communicating clearly about everything that's going on with respect to development of programs across the entire industry. I know as a partner for Dimension, we were incredibly proud of their approach and information sharing that was going on with respect to the details of that program. And in particular, I guess what I can highlight, Gbola, that certainly has become apparent, I think, in the announcement today is that DTX101, an intravenous administration of an AAV vector for the treatment of hemophilia B, used a viral vector capsid called AAVrh10 that is different than the viral vector capsid in the NAV Technology Platform, NAV AAV8, which is what's used for DTX301, DTX401 and also in RGX-501 for HoFH. So our familiarity as a company with the AAV8 vector in terms of its application in the development of treatments via intravenous administration was an important factor in sort of identifying the synergy and qualifying the great science in order to see the value for our DTX301 and DTX401, perhaps in contrast to how the market viewed the DTX101 data earlier in the year.

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**Operator**

(Operator Instructions) And I am showing no further questions from our phone lines. I would now like to turn the conference back over to Ken Mills for any closing remark.

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**Kenneth T. Mills - Regenxbio Inc. - CEO, President and Director**

Thank you very much, operator, and we appreciate, this morning, everyone dialing in and listening. We look forward to speaking with you all again on this topic and importantly on topics of the progress of the advancement of the REGENXBIO pipeline in the future. Thanks for your time this morning.

9

**Operator**

Ladies and gentlemen, thank you for participating in today's conference. This does conclude the program and you may all disconnect. Everyone, have a wonderful day.

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