



## **REGENXBIO to Receive \$80.0 Million Milestone Payment from Novartis AG**

October 27, 2020 11:00 AM EDT

**- Milestone payment based on achievement of \$1.0 billion in cumulative net sales of Zolgensma® -**

**- Zolgensma is the first gene therapy based on REGENXBIO's NAV® Technology Platform approved by the FDA and other regulatory authorities -**

ROCKVILLE, Md., Oct. 27, 2020 /PRNewswire/ -- REGENXBIO Inc. (Nasdaq: RGNX), a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy based on its proprietary NAV® Technology Platform, today announced that it will receive a milestone payment of \$80.0 million from Novartis AG based upon the achievement of \$1.0 billion in cumulative net sales of Zolgensma. REGENXBIO expects to recognize this revenue in the third quarter of 2020.

"Zolgensma is a transformative, one-time gene therapy for the treatment of pediatric patients with SMA, a debilitating and potentially deadly disease, and the first approved gene therapy based on REGENXBIO's NAV Technology. We believe that this achievement provides further validation of the broad potential of NAV Technology to improve the lives of patients in need," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "We are pleased to have contributed to a therapy that has impacted the lives of over 700 patients with SMA and their families around the world."

Zolgensma uses REGENXBIO's proprietary NAV AAV9 vector and was designed and developed by AveXis, Inc., now known as Novartis Gene Therapies. In May 2019, Zolgensma was granted U.S. Food and Drug Administration (FDA) approval for the treatment of spinal muscular atrophy (SMA) in pediatric patients who are less than two years of age, and has since been approved in Europe, Japan and other countries. REGENXBIO continues to receive tiered royalties on sales of Zolgensma. Since FDA approval in 2019, REGENXBIO has earned more than \$140.0 million in revenues from royalties and commercial milestone payments related to Zolgensma through the third quarter of 2020.

### **About REGENXBIO Inc.**

REGENXBIO is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. REGENXBIO's NAV Technology Platform, a proprietary adeno-associated virus (AAV) gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO and its third-party NAV Technology Platform Licensees are applying the NAV Technology Platform in the development of a broad pipeline of candidates in multiple therapeutic areas.

### **Forward-Looking Statements**

This press release includes "forward-looking statements," within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue," "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations of such words or by similar expressions. The forward-looking statements include statements relating to, among other things, REGENXBIO's cash flow. REGENXBIO has based these forward-looking statements on its current expectations and assumptions and analyses made by REGENXBIO in light of its experience and its perception of historical trends, current conditions and expected future developments, as well as other factors REGENXBIO believes are appropriate under the circumstances. However, whether actual results and developments will conform with REGENXBIO's expectations and predictions is subject to a number of risks and uncertainties, including the timing of enrollment, commencement and completion and the success of clinical trials conducted by REGENXBIO, its licensees and its partners, the timing of commencement and completion and the success of preclinical studies conducted by REGENXBIO and its development partners, the timely development and launch of new products, the ability to obtain and maintain regulatory approval of product candidates, the ability to obtain and maintain intellectual property protection for product candidates and technology, trends and challenges in the business and markets in which REGENXBIO operates, the size and growth of potential markets for product candidates and the ability to serve those markets, the rate and degree of acceptance of product candidates, the impact of the COVID-19 pandemic or similar public health crises on REGENXBIO's business, and other factors, many of which are beyond the control of REGENXBIO. Refer to the "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of REGENXBIO's Annual Report on Form 10-K for the year ended December 31, 2019, and comparable "risk factors" sections of REGENXBIO's Quarterly Reports on Form 10-Q and other filings, which have been filed with the U.S. Securities and Exchange Commission (SEC) and are available on the SEC's website at [www.sec.gov](http://www.sec.gov). All of the forward-looking statements made in this press release are expressly qualified by the cautionary statements contained or referred to herein. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on REGENXBIO or its businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially from those projected in the forward-looking statements. Readers are cautioned not to rely too heavily on the forward-looking statements contained in this press release. These forward-looking statements speak only as of the date of this press release. REGENXBIO does not undertake any obligation, and specifically declines any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Zolgensma® is a registered trademark of Novartis Gene Therapies. All other trademarks referenced herein are registered trademarks of REGENXBIO.

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