



REGENXBIO Joins Organizations Worldwide to Recognize Rare Disease Day® 2016

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ROCKVILLE, Md., February 29, 2016 -- REGENXBIO Inc. (Nasdaq: RGNX), a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy based on its proprietary NAV® Technology Platform, today announced that it will join organizations worldwide to recognize Rare Disease Day® 2016, an annual event that seeks to raise awareness among the general public and stakeholders about rare diseases and their impact on patients' lives. This year's theme, "Patient Voice," highlights the role patients play in voicing their needs and implementing change that improves their lives and the lives of their families and caregivers.

"REGENXBIO is pleased to unite with the global rare disease community to support Rare Disease Day and increase awareness of the significant unmet need for improved treatment options for patients," said Kenneth T. Mills, President and Chief Executive Officer of REGENXBIO. "At REGENXBIO, we are committed to developing gene therapy product candidates with the potential to transform the lives of patients suffering from rare diseases and their families. We look forward to continuing to work with the rare disease community to advance meaningful treatment options."

In recognition of Rare Disease Day, REGENXBIO employees will be participating in the following activities:

- Rare Disease Day at the National Institutes of Health (NIH), to be held Monday, February 29, 2016, in Bethesda, Maryland. The event, sponsored by the National Center for Advancing Translational Sciences (NCATS) and the NIH Clinical Center, aims to raise awareness about rare diseases, the patients they affect and the research collaborations that are addressing rare disease challenges.
- 12th Annual *WORLDSymposium*™ 2016, to be held February 29 through March 4 in San Diego, California. The *WORLDSymposium* gathers researchers, clinicians, patient organizations and industry participants, providing an interdisciplinary forum to explore and discuss specific areas of interest, research and clinical applicability related to lysosomal diseases.
- Rare Disease Legislative Advocates' Rare Disease Legislative Conference, to be held Tuesday, March 1, in Washington, D.C.

REGENXBIO is leveraging its proprietary NAV Technology Platform to advance the development of gene therapy treatments that address serious unmet needs in metabolic, neurodegenerative and retinal diseases. REGENXBIO's lead gene therapy product candidates include programs for the treatment of two severe and rare genetic diseases: homozygous familial hypercholesterolemia (HoFH) and mucopolysaccharidosis Type I (MPS I).

About Rare Disease Day

Rare Disease Day takes place every year on the last day of February to underscore the nature of rare diseases and what patients face. Rare Disease Day was established in Europe in 2008 by the European Organization for Rare Diseases (EURORDIS), the organization representing rare disease patients in Europe, and is now observed in more than 80 nations. Rare Disease Day is sponsored in the U.S. by the National Organization for Rare Disorders (NORD), a leading independent, non-profit organization committed to the identification, treatment and cure of rare diseases. Organizations around the world will hold a variety of activities to raise rare and genetic disease awareness.

For additional information on Rare Disease Day in the U.S., please visit: www.rarediseaseday.us. For global activities, please visit: www.rarediseaseday.org.

About REGENXBIO

REGENXBIO is a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. REGENXBIO's NAV® Technology Platform, a proprietary AAV gene delivery platform, consists of exclusive rights to more than 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. REGENXBIO's mission is to transform the lives of patients suffering from severe diseases with significant unmet medical need by developing and commercializing in vivo gene therapy products based on REGENXBIO's NAV Technology Platform. REGENXBIO seeks to accomplish this mission through a combination of internal development efforts and third-party NAV Technology Platform licensees. As of December 31, 2015, REGENXBIO's NAV Technology Platform is being applied in the development of 28 product candidates for a variety of diseases, including five internally developed candidates and 23 partnered candidates developed by REGENXBIO's licensees.

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