



Regeneron Discovery on Cause of Rare Genetic Bone Disease Detailed in Science Translational Medicine Publication

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Tarrytown, New York (September 2, 2015) - Regeneron Pharmaceuticals, Inc. (NASDAQ: **REGN**) today announced that *Science Translational Medicine* has published a paper describing the discovery and preclinical validation of a key biologic mechanism that drives the pathophysiology of the rare genetic disorder Fibrodysplasia Ossificans Progressiva (FOP). FOP is a progressive, severely disabling and ultimately fatal disease in which muscles, ligaments, tendons and other connective tissues are transformed into bone.

FOP is caused by mutations in *ACVR1*, a gene that encodes for the ACVR1 receptor protein. When a set of ligands (proteins that help regulate cell behavior) known as Bone Morphogenetic Proteins (BMPs) bind to the ACVR1 receptor, a cascade of intracellular events (referred to as "signaling") is induced. This signaling is critical in controlling the formation of the skeleton and ensuring normal bone growth.

Regeneron scientists found that another ligand, Activin-A, in combination with the ACVR1 protein, normally "turns off" signaling by the BMPs, and is thus believed to play a role in regulating the volume of bone growth. However, they also discovered that in the presence of the ACVR1 mutation, Activin-A instead "turns on" BMP signaling, driving the abnormal bone growth that is characteristic of FOP.

People diagnosed with FOP are gradually debilitated and immobilized as the soft tissue throughout their body transforms into bone. Attempts to remove this extra bone through surgery only result in additional episodes of abnormal bone growth. There are approximately 800 confirmed cases of FOP in the world, including around 200 in the United States, and the current lack of effective treatments underscores the need for scientific exploration that may aid in the development of new therapies.

"We are excited and grateful to have this new fundamental insight into FOP," said Betsy Bogard, Global Research Development Director of the International FOP Association (IFOPA). "Regeneron's extraordinary research findings bring new hope to the families who struggle with this devastating disease on a daily basis."

To extend their findings *in vivo*, Regeneron scientists used the Company's proprietary *VelociGene*® technology to develop a genetically humanized and novel mouse model of FOP in which the hypothesized molecular pathophysiology of disease was confirmed. The team also used Regeneron's *VelocImmune*®, a platform that enables the rapid generation of fully human monoclonal antibodies, to generate and validate a potential therapeutic antibody chosen for its ability to potently and selectively block Activin-A. Regeneron continues active preclinical testing on this antibody.

"We have been touched and motivated by the stories of people with FOP, and are grateful to contribute to the understanding of this disease in a way that may lead to new therapeutic options," said Aris N. Economides, Executive Director of Skeletal Diseases Therapeutic Focus Area and Genome Engineering Technologies at Regeneron, and co-founder of the Regeneron Genetics Center. "Gaining insight into the Activin A-related mechanism is a tremendous step forward for researchers, and the knowledge gained about receptor-ligand interactions and signaling in this system may prove relevant in other diseases, as well."

About Regeneron Pharmaceuticals, Inc.

Regeneron is a leading science-based biopharmaceutical company based in Tarrytown, New York that discovers, invents, develops, manufactures, and commercializes medicines for the treatment of serious medical conditions. Regeneron commercializes medicines for eye diseases, high LDL-cholesterol, and a rare inflammatory condition and has product candidates in development in other areas of high unmet medical need, including oncology, rheumatoid arthritis, asthma, atopic dermatitis, pain, and infectious diseases. For additional information about the company, please visit www.regeneron.com or follow @Regeneron on Twitter.

Forward-Looking Statements and Use of Digital Media *This news release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," "variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of Regeneron's products, product candidates, and research and clinical programs now underway or planned, including without limitation the antibody targeting Activin-A discussed in this news release; the extent to which the results from Regeneron's research programs or preclinical testing may lead to advancement of product candidates to clinical trials or therapeutic applications; unforeseen safety issues and possible liability resulting from the administration of products and product candidates in patients; serious complications or side effects in connection with the use of Regeneron's products and product candidates in clinical trials; ongoing regulatory obligations and oversight impacting Regeneron's marketed products, research and clinical programs, and business; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's products and product candidates; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's late-stage product candidates and new indications for marketed products; competing drugs and product candidates that may be superior to Regeneron's products and product candidates; uncertainty of market acceptance and commercial success of Regeneron's products and product candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary) on the commercial success of Regeneron's products and product candidates; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; coverage and reimbursement determinations by third-party payers, including Medicare and Medicaid; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its sales or other financial projections or guidance and changes to the assumptions underlying those projections or guidance; the potential for any license or collaboration agreement, including Regeneron's agreements with Sanofi and Bayer*

HealthCare LLC, to be cancelled or terminated without any further product success; and risks associated with intellectual property of other parties and pending or future litigation relating thereto. A more complete description of these and other material risks can be found in Regeneron's filings with the United States Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2014 and its Form 10-Q for the quarterly period ended June 30, 2015. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update publicly any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (<http://newsroom.regeneron.com>) and its Twitter feed (<http://twitter.com/regeneron>).

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