Regeneron Presents Positive Interim Data from Phase 2 Proof-of-Concept Study of Evinacumab in Patients with Homozygous Familial Hypercholesterolemia

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TARRYTOWN, N.Y., May 31, 2016 /PRNewswire/ -- Regeneron Pharmaceuticals, Inc. (NASDAQ: **REGN**) today announced that positive preliminary results from an ongoing proof-of-concept study of evinacumab (REGN1500) in patients with Homozygous Familial Hypercholestrolemia (HoFH) were presented at the 84th European Atherosclerosis Society (EAS) Congress in Innsbruck, Austria. The interim data from the first 4 patients treated in this study showed that evinacumab added to current lipid-lowering therapy reduced low-density lipoprotein cholesterol (LDL-C) levels by an additional 55 percent (mean reduction; range 25 to 90 percent) at week 4 compared to baseline. Evinacumab is an investigational monoclonal antibody to angiopoietin-like protein 3 (ANGPTL3). ANGPTL3 acts as an inhibitor of lipoprotein lipase and endothelial lipase, and appears to play a central role in lipoprotein metabolism.

HoFH is the most severe form of hypercholesterolemia. While rare, occurring in approximately 1 to 2 people per million, untreated patients can have "bad cholesterol" or LDL-C levels ranging from 500 to 1000 mg/dL, compared to normal LDL-C levels of less than 130 mg/dL. Due to these high levels of LDL-C, patients with HoFH are at an extreme risk of premature cardiovascular disease. Without treatment, patients typically present with signs and symptoms of atherosclerotic cardiovascular disease before the age of 20.

"HoFH patients are not as responsive to traditional lipid lowering therapies like statins, and use of some new treatment options can be limited by safety and tolerability concerns," said Bill Sasiela, Ph.D., Vice President, Program Direction, Regeneron. "Preliminary data from this ongoing study show that evinacumab reduced LDL-C when combined with other lipid-lowering therapies, and suggests that ANGPTL3 is a new and exciting target that we look forward to exploring further in additional clinical trials."

The ongoing single-arm, open label, proof-of-concept study is targeting to enroll up to 8 patients with HoFH. Study patients' diagnosis is confirmed both genetically and phenotypically. Current lipid-lowering therapy was maintained from 4 weeks before baseline through the 26-week treatment and observation period. Patients on LDL apheresis within 4 weeks prior to screening were excluded. For the 4 patients reported here, all were receiving maximum doses of statin plus ezetimibe with one patient additionally receiving lomitapide 20 mg. Evinacumab was dosed as a single 250 mg subcutaneous injection at baseline and subsequently, at week 2, as a 15 mg/kg intravenous (IV) infusion. The primary endpoint of the study was the mean percent change in LDL-C levels from baseline to week 4.

After 4 weeks of treatment and 2 weeks after administration of the 15 mg/kg IV dose, the mean reduction in LDL-C in the 4 patients was 55 percent (primary endpoint of the study). Among these 4 patients, the percent reductions in LDL-C ranged from 25 to 90 percent. Overall, the patients enrolled in the study saw their mean LDL-C levels fall from 331mg/dL at baseline to 175mg/dL at week 4.

Evinacumab was generally well tolerated and there were no adverse events leading to discontinuation. The most common drug-related adverse events were injection-site reactions, which were mild in severity.

About Regeneron Pharmaceuticals, Inc.
Regeneron (NASDAQ: REGN) 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 high LDL cholesterol, eye diseases, 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.

Regeneron 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 evinacumab (REGN1500); unforeseen safety issues resulting from the administration of products and product candidates in patients, including serious complications or side effects in connection with the use of Regeneron's product candidates in clinical trials, such as the clinical development program evaluating evinacumab; the likelihood and timing of possible regulatory approval and commercial launch of Regeneron's product candidates, such as evinacumab in patients with Homozygous Familial Hypercholesterolemia or other indications; 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, such as evinacumab; ongoing regulatory obligations and oversight impacting Regeneron's marketed products, research and clinical programs, and business, including those relating to patient privacy; 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, 2015 and its Form 10-Q for the quarterly period ended March 31, 2016. 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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