



FDA Accepts Evinacumab Biologics License Application for Priority Review as a Treatment for Patients with HoFH, an Ultra-rare Inherited Form of High Cholesterol

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Homozygous familial hypercholesterolemia affects approximately 1,300 patients in the U.S., the vast majority of whom are unable to reach target LDL-C levels with currently available therapies

[Regeneron Pharmaceuticals, Inc.](#) (NASDAQ: REGN) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review a Biologics License Application (BLA) for evinacumab as an adjunct to other lipid-lowering therapies in patients with homozygous familial hypercholesterolemia (HoFH). Evinacumab is the first investigational medicine of its kind to show efficacy in patients with HoFH – including patients with little to no low-density lipoprotein (LDL) receptor function – by binding to and blocking the function of angiotensin-like 3 (ANGPTL3).

The target action date for the FDA decision is February 11, 2021. The FDA granted evinacumab Breakthrough Therapy designation in 2017 for the treatment of hypercholesterolemia in patients with HoFH.

HoFH is an ultra-rare inherited disease that affects approximately 1,300 patients in the U.S. Patients with HoFH have severely elevated levels of bad cholesterol (low-density lipoprotein cholesterol, or LDL-C), which increases their risk for premature atherosclerotic disease and cardiac events as early as their teenage years. Treatment guidelines recommend early and intensive LDL-C lowering, but patients with HoFH are less responsive (or unresponsive) to standard lipid-lowering therapies, including statins and PCSK9 (proprotein convertase subtilisin/kexin type 9) inhibitors.

The BLA was supported by a pivotal Phase 3 trial evaluating the efficacy and safety of evinacumab in patients with HoFH. Results from this trial were [presented](#) as a late-breaking presentation at the American College of Cardiology's Annual Scientific Session together with World Congress of Cardiology (ACC.20) in March 2020.

Previous research [published](#) in NEJM in 2017 by the Regeneron Genetics Center found that patients with loss-of-function mutations in their ANGPTL3 gene have significantly lower levels of key blood lipids, including LDL-C. By blocking the ANGPTL3 protein, evinacumab was designed to replicate this loss-of-function mutation effect to lower LDL-C in patients with HoFH.

Regulatory submissions for evinacumab are also progressing in the European Union. In June 2020, the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) recommended an accelerated assessment for evinacumab based on the high unmet medical need and therapeutic innovation demonstrated by the product. The safety and efficacy of evinacumab have not been fully evaluated by any regulatory authority.

About evinacumab and the ELIPSE HoFH Trial

Regeneron invented evinacumab using the company's *VelocImmune*[®] technology, a proprietary genetically-engineered mouse platform endowed with a genetically-humanized immune system to produce optimized fully-human monoclonal antibodies. *VelocImmune* technology has been used to create multiple FDA-approved antibodies including Praluent[®] (alirocumab), Dupixent[®] (dupilumab), Libtayo[®] (cemiplimab-rwlc) and Kevzara[®] (sarilumab). Regeneron previously used these technologies to rapidly develop a [treatment](#) for Ebola virus infection, which is currently under review by the FDA, and is now being used in efforts to create prophylactic and treatment medicines for COVID-19.

Evinacumab is an investigational fully-human monoclonal antibody that binds to and blocks the function of ANGPTL3 and is currently being studied in patients with HoFH (ongoing Phase 3 extension trial), refractory hypercholesterolemia (Phase 2) and severe hypertriglyceridemia (Phase 2).

ELIPSE (Evinacumab **L**IPid **S**tudi**E**s) HoFH was a multi-national Phase 3 randomized, double-blind, placebo-controlled, parallel-group trial evaluating the efficacy and safety of evinacumab 15 mg/kg administered intravenously every four weeks in 65 patients aged 12 years or older with HoFH (43 evinacumab, 22 placebo). The primary endpoint was reduction of LDL-C from baseline with evinacumab compared to placebo at 24 weeks.

About Regeneron

Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents life-transforming medicines for people with serious diseases. Founded and led for over 30 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to seven FDA-approved treatments and numerous product candidates in development, all of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, pain, infectious diseases and rare diseases. Regeneron is accelerating and improving the traditional drug development process through our proprietary *VelociSuite*[®] technologies, such as *VelocImmune*, which uses unique genetically-humanized mice to produce optimized fully-human antibodies and bispecific antibodies, and through ambitious research initiatives such as the Regeneron Genetics Center, which is conducting one of the largest genetics sequencing efforts in the world. 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 press 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 impact of SARS-CoV-2 (the virus that has caused the COVID-19 pandemic) on Regeneron's business and its employees, collaborators, and suppliers and other third parties on which Regeneron relies, Regeneron's and its

collaborators' ability to continue to conduct research and clinical programs, Regeneron's ability to manage its supply chain, net product sales of products marketed by Regeneron and/or its collaborators (collectively, "Regeneron's Products"), and the global economy; the nature, timing, and possible success and therapeutic applications of Regeneron's Products and Regeneron's product candidates and research and clinical programs now underway or planned, including without limitation evinacumab; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's product candidates and new indications for Regeneron's Products, such as evinacumab for the treatment of patients with homozygous familial hypercholesterolemia, refractory hypercholesterolemia, or severe hypertriglyceridemia; 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; safety issues resulting from the administration of Regeneron's Products and product candidates (such as evinacumab) in patients, including serious complications or side effects in connection with the use of Regeneron's Products and product candidates in clinical trials; 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, including without limitation evinacumab; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement of Regeneron's Products from third-party payers, including private payer healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payers and new policies and procedures adopted by such payers; competing drugs and product candidates that may be superior to, or more cost effective than, Regeneron's Products and product candidates; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; the ability of Regeneron to manufacture and manage supply chains for multiple products and product candidates; the ability of Regeneron's collaborators, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and product candidates; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its 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, Bayer, and Teva Pharmaceutical Industries Ltd. (or their respective affiliated companies, as applicable), 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 (including without limitation the patent litigation and other related proceedings relating to EYLEA® (afibercept) Injection, Dupixent® (dupilumab), and Praluent® (alirocumab)), other litigation and other proceedings and government investigations relating to the Company and/or its operations, the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2019 and its Form 10-Q for the quarterly period ended June 30, 2020. 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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