Garetosmab Biologics License Application Accepted for FDA Priority Review for the Treatment of Fibrodysplasia Ossificans Progressiva (FOP)

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FOP is an ultra-rare genetic disorder characterized by abnormal bone formation that infiltrates muscles, tendons, ligaments and other connective tissues, resulting in significant disability

If approved, garetosmab would be the first and only available treatment shown to reduce the number and volume of new heterotopic bone lesions in adults with FOP

TARRYTOWN, N.Y., Feb. 19, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced that the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the Biologics License Application (BLA) for garetosmab for the treatment of adults with fibrodysplasia ossificans progressiva (FOP). Garetosmab is a monoclonal antibody that blocks Activin A, a protein that Regeneron scientists discovered to be critical in the development of heterotopic ossification (HO) lesions in people with FOP. The target action date for the FDA decision is August 2026.

FOP is a relentless, ultra-rare genetic disorder in which muscles, tendons, ligaments and other connective tissues are progressively infiltrated by abnormal bone formation, a process known as HO, which results in significant disfunction of these structures and skeletal deformity. HO of the jaw, spine, hip and rib cage can make it difficult to speak, eat, walk or breathe, leading to weight loss and escalating loss of mobility. Most people with FOP are wheelchair bound by 30 years old, and the median age of survival is approximately 56 years. Approximately 900 people are diagnosed with FOP worldwide, with many others thought to remain undiagnosed or misdiagnosed.

The BLA is supported by efficacy and safety data from the positive Phase 3 OPTIMA trial evaluating garetosmab in adults with FOP. Both garetosmab doses (3 mg/kg and 10 mg/kg) evaluated in the trial were highly efficacious in reducing the total number and volume of new HO lesions at 56 weeks, compared to placebo. Regarding the primary endpoint analysis of reduction in total number of new HO lesions compared to placebo (n=21), those receiving the 3 mg/kg dose (n=19) experienced a 94% reduction (1 lesion vs. 19 lesions; p=0.0274), while those receiving the 10 mg/kg dose (n=23) experienced a 90% reduction (2 lesions vs. 19 lesions; p=0.0260). A post-hoc analysis also found both doses of garetosmab demonstrated a greater than 99% reduction in mean total volume (cm^3) of new HO lesions compared to placebo (3 mg/kg: 0.01 cm^3 vs. 10.45 cm^3; nominal p=0.0013; 10 mg/kg: 0.02 cm^3 vs. 10.45 cm^3; nominal p=.0005).

At 56 weeks, among all 63 people with FOP aged 18 years and older who participated in the OPTIMA trial, serious treatment-emergent adverse events occurred in 1 patient treated with 3 mg/kg garetosmab, 2 patients treated with 10 mg/kg garetosmab and 2 patients treated with placebo. The most common adverse reactions (incidence ≥30%) are epistaxis, increased hair growth, abscess and acne.

Priority Review is granted to regulatory applications seeking approval for therapies that have the potential to provide significant improvements in the treatment, diagnosis or prevention of serious conditions. The FDA previously granted Fast Track designation and Orphan Drug Designation for garetosmab for the prevention of HO in patients with FOP. Garetosmab has also been granted Orphan Designation in the European Union, and additional garetosmab regulatory submissions are planned in countries around the world.

The safety and efficacy of garetosmab, as well as its potential use for the treatment of FOP, are investigational and have not been fully evaluated or approved by any regulatory authority.

About the OPTIMA Clinical Trial
OPTIMA is a Phase 3, multi-center, multinational trial to assess the efficacy of garetosmab on the reduction of heterotopic bone formation, as well as its safety, tolerability, and pharmacokinetics, in patients with active FOP.

The trial enrolled 63 participants aged 18 years and older who have any FOP-causing variant of type I Activin A receptor (ACVR1), exhibited FOP disease activity or progression of HO lesions, and had a cumulative analogue joint involvement scale (CAJIS) score at screening of ≤19. CAJIS is a clinician-assessed tool, with higher scores representing greater disease severity (scale: 0 to 30). Eligible participants were randomized to intravenously receive 3 mg/kg garetosmab, 10 mg/kg garetosmab, or placebo once every four weeks for 56 weeks. Following this, participants could elect to extend their treatment for at least 84 weeks or discontinue treatment and enter an observation-only arm.

During the treatment period, efficacy was evaluated through whole body computed tomography (CT) scans for HO lesions; physician and patient assessment of flare-ups; utilization of CAJIS to rate joint functionality; and observances of change in disease

severity. Safety assessment includes reports of adverse events, measurement of vital signs, physical examination, and coagulation testing.

A Phase 3 trial of garetosmab in adolescents and children with FOP, OPTIMA 2, is planned to begin later this year. For more information, visit the Regeneron clinical trials website, contact clinicaltrials@regeneron.com, or call +1 844-734-6643.

About Garetosmab
Regeneron has been engaged in FOP research for decades and helped to provide fundamental insights into the biology and natural history of the disease. Regeneron scientists discovered that Activin A plays a key role in FOP by driving HO, the main pathology of FOP. Garetosmab is a *VelocImmune*-derived, fully-human monoclonal antibody that binds and neutralizes Activin A, which is involved in the development of heterotopic bone in people with FOP.

About Regeneron's *VelocImmune* Technology
Regeneron's *VelocImmune* technology utilizes a proprietary genetically engineered mouse platform endowed with a genetically humanized immune system to produce fully optimized human antibodies. When Regeneron's Co-Founder, President and Chief Scientific Officer George D. Yancopoulos was a graduate student with his mentor Frederick W. Alt in 1985, they were the first to envision making such a genetically humanized mouse, and Regeneron has spent decades inventing and developing *VelocImmune* and related *VelociSuite*® technologies. Dr. Yancopoulos and his team have used *VelocImmune* technology to create a substantial proportion of all original, FDA-approved fully human monoclonal antibodies. This includes Dupixent® (dupilumab), Libtayo® (cemiplimab-rwlc), Praluent® (alirocumab), Kevzara® (sarilumab), Evkeeza® (evinacumab-dgnb), Inmazeb® (atoltivimab, maftivimab and odesivimab-ebgn) and Veopoz® (pozelimab-bbfg). In addition, REGEN-COV® (casirivimab and imdevimab) had been authorized by the FDA during the COVID-19 pandemic until 2024. Garetosmab was also created using Regeneron's *VelocImmune* technology.

About Regeneron
Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents, develops and commercializes life-transforming medicines for people with serious diseases. Founded and led by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to numerous approved treatments and product candidates in development, most 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, neurological diseases, hematologic conditions, infectious diseases, and rare diseases.

Regeneron pushes the boundaries of scientific discovery and accelerates drug development using our proprietary technologies, such as *VelociSuite*, which produces optimized fully human antibodies and new classes of bispecific antibodies. We are shaping the next frontier of medicine with data-powered insights from the Regeneron Genetics Center® and pioneering genetic medicine platforms, enabling us to identify innovative targets and complementary approaches to potentially treat or cure diseases.

For more information, please visit www.Regeneron.com or follow Regeneron on LinkedIn, Instagram, Facebook or X.

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 nature, timing, and possible success and therapeutic applications of products marketed or otherwise commercialized by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Products") and product candidates being developed by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Product Candidates") and research and clinical programs now underway or planned, including without limitation garetosmab; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products, including garetosmab for the treatment of adults with fibrodysplasia ossificans progressiva as discussed in this press release; uncertainty of the utilization, market acceptance, and commercial success of Regeneron's Products and Regeneron's Product Candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary), including the studies discussed or referenced in this press release, on any of the foregoing or any potential regulatory approval of Regeneron's Products and Regeneron's Product Candidates (such as garetosmab); the ability of Regeneron's collaborators, licensees, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and Regeneron's Product Candidates; the ability of Regeneron to manage supply chains for multiple products and product candidates and risks associated with tariffs and other trade restrictions; safety issues resulting from the administration of Regeneron's Products and Regeneron's Product Candidates (such as garetosmab) in patients, including serious complications or side effects in connection with the use of Regeneron's Products and Regeneron's 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 Regeneron's Product Candidates; 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 or copay assistance for Regeneron's Products from third-party payors and other third parties, including private payor 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 payors and other third parties and new policies and procedures adopted by such payors and other third parties; changes to drug pricing regulations and requirements and Regeneron's pricing strategy; other changes in laws, regulations, and policies affecting the healthcare industry; competing products and product candidates (including biosimilar products) that may be superior to, or more cost effective than, Regeneron's Products and Regeneron's Product Candidates; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; 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, collaboration, or supply agreement, including Regeneron's agreements with Sanofi and Bayer (or their respective affiliated companies, as applicable), to be cancelled or terminated; the impact of public health outbreaks, epidemics, or pandemics on Regeneron's business; and risks associated with litigation and other proceedings and government investigations relating to the Company and/or its operations (including the pending civil proceedings initiated or joined by the U.S. Department of Justice and the U.S. Attorney's Office for the District of Massachusetts), 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® (aflibercept) Injection), 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, 2025. 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 or otherwise) 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 (https://investor.regeneron.com) and its LinkedIn page (https://www.linkedin.com/company/regeneron-pharmaceuticals).

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