Praluent® (alirocumab) Injection Receives FDA Approval to Treat Children with Genetic Form of High Cholesterol

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Approval extends treatment of Praluent to children aged 8 and older with heterozygous familial hypercholesterolemia (HeFH)

TARRYTOWN, N.Y., March 11, 2024 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) announced the U.S. Food & Drug Administration (FDA) has extended the approval of Praluent® (alirocumab) as an adjunct to diet and other low-density lipoprotein cholesterol (LDL-C) lowering therapies to include pediatric patients aged 8 and older with heterozygous familial hypercholesterolemia (HeFH).

“Many children with heterozygous familial hypercholesterolemia (HeFH) are able to substantially improve their LDL-C (bad cholesterol) with currently available therapies. But for those children whose LDL-C remains dangerously high, this approval is an important milestone as it gives these children and their families an additional option to help reduce and manage their LDL-C levels much earlier in their lives,” said Mary P. McGowan, M.D., Chief Medical Officer of the Family Heart Foundation.

Familial hypercholesterolemia (FH) is an inherited condition caused by mutations in one of several genes that control how the body processes cholesterol, which can lead to very high levels of LDL-C (bad cholesterol). FH can come in two forms: HeFH, which develops when one mutated gene is inherited from one parent; and homozygous familial hypercholesterolemia (HoFH), which develops when a mutated gene is inherited from both parents. Praluent is approved to treat both children and adults with HeFH and adults with HoFH.

The approval is based on a Phase 3, randomized multicenter trial evaluating pediatric patients aged 8 to 17 with HeFH, who had LDL-C levels of 130mg/dL or greater and were already being treated with lipid-lowering medications. Patients were randomized to receive Praluent (N=101) or placebo (N=52) every two or four weeks in two consecutive cohorts. Patients who received Praluent every four weeks had 31% lower LDL-C than placebo at 24 weeks (97.5% Confidence Interval: -45.0% to -17.9%; p<0.0001). Improvements in additional key lipid parameters were also observed. Results from the trial were recently published in the Journal of the American Medical Association Pediatrics.

No new adverse reactions were identified in this trial, and the safety profile was consistent with the safety profile observed in adults with HeFH. Across Praluent trials in patients with primary hyperlipidemia (N=2,476), the most common adverse reactions (≥5%) more frequently observed with Praluent than placebo have been injection site reactions (7%), and influenza (6%) and diarrhea (5%).

“The approval of Praluent for the treatment of high cholesterol was a historic landmark achievement, as it was the first approved therapy targeting the genetically-validated PCSK9 target for heart disease,” said George D. Yancopoulos, M.D., Ph.D., Board co-Chair, President and Chief Scientific Officer at Regeneron, and a principal inventor of Praluent. “Praluent has made a meaningful impact in the treatment of adults with familial hypercholesterolemia, and we are proud that our innovation will now be able to help appropriate children with the heterozygous form of this disease manage their dangerously high levels of LDL-C.”

About the Praluent HeFH Pediatric Trial
The randomized multicenter Phase 3 trial consisted of a 24-week double-blind, placebo-controlled evaluating the efficacy and safety of Praluent in pediatric patients aged 8 to 17 years with HeFH (N=79). The primary endpoint was the percent change in LDL-C from baseline to week 24 in the Praluent and placebo treated patients. At baseline, patients were on a low-fat diet and being treated with background lipid-lowering therapy. In the trial, patients were randomized 2:1 to receive Praluent or placebo every 2 or 4 weeks. The Praluent dose was based on body weight.

About Praluent
Praluent inhibits the binding of PCSK9 to the LDL receptor and thereby increases the number of available LDL receptors on the surface of liver cells to clear LDL, which lowers LDL-C levels in the blood. Praluent was developed by Regeneron and Sanofi under a global collaboration agreement and invented by Regeneron using the company's proprietary *VelocImmune*® technology that yields optimized fully-human monoclonal antibodies.

In the U.S., Praluent is currently indicated:

- to reduce the risk of myocardial infarction, stroke, and unstable angina requiring hospitalization in adults with established cardiovascular disease
- as an adjunct to diet, alone or in combination with other low-density lipoprotein cholesterol (LDL-C) lowering therapies in adults with primary hyperlipidemia including HeFH to reduce LDL-C
- as an adjunct to other LDL-C-lowering therapies in adults with HoFH to reduce LDL-C
- along with diet and other LDL-C lowering treatments in children aged 8 years and older with HeFH to reduce LDL-C

In addition to the U.S., Praluent is approved in 60 countries, including the European Union, Japan, Canada, Switzerland and Brazil.

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 optimized fully human antibodies. When Regeneron's 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 currently available.

This includes Evkeeza® (evinacumab-dgnb), REGEN-COV® (casirivimab and imdevimab), Dupixent® (dupilumab), Libtayo® (cemiplimab-rwlc), Praluent, Kevzara® (sarilumab), Inmazeb® (atoltivimab, maftivimab and odesivimab-ebgn) and Veopoz® (pozelimab-bbfg).

IMPORTANT SAFETY INFORMATION AND INDICATIONS

INDICATIONS
PRALUENT is an injectable prescription medicine used:

- in adults with cardiovascular disease to reduce the risk of heart attack, stroke, and certain types of chest pain conditions (unstable angina) requiring hospitalization.
- along with diet, alone or together with other cholesterol-lowering medicines in adults with high blood cholesterol levels called primary hyperlipidemia (including a type of high cholesterol called heterozygous familial hypercholesterolemia [HeFH]), to reduce low-density lipoprotein cholesterol (LDL-C) or bad cholesterol.
- along with other LDL-lowering treatments in adults with a type of high cholesterol called homozygous familial hypercholesterolemia, who need additional lowering of LDL-C.
- along with diet and other LDL-C lowering treatments in children aged 8 years and older with HeFH to reduce LDL-C.

It is not known if PRALUENT is safe and effective in children who are younger than 8 years of age or in children with other types of high cholesterol (hyperlipemias).

IMPORTANT SAFETY INFORMATION

Do not use PRALUENT if you are allergic to alirocumab or to any of the ingredients in PRALUENT.

Before you start using PRALUENT, tell your healthcare provider about all of your medical conditions, including allergies, and if you are pregnant or plan to become pregnant or if you are breastfeeding or plan to breastfeed.

Tell your healthcare provider or pharmacist about any medicines you take, including prescription and over-the-counter medicines, vitamins, or herbal supplements.

PRALUENT can cause serious side effects, including allergic reactions that can be severe and require treatment in a hospital. Stop using PRALUENT and call your healthcare provider or go to the nearest hospital emergency room right away if you have any symptoms of an allergic reaction including a severe rash, redness, hives, severe itching, trouble breathing, or swelling of the face, lips, throat, or tongue.

The common side effects of PRALUENT include: redness, itching, swelling, or pain/tenderness at the injection site, flu or flu-like symptoms, diarrhea, muscle pain, muscle spasms and bruising. Tell your healthcare provider if you have any side effect that bothers you or that does not go away.

Talk to your doctor about the right way to prepare and give yourself a PRALUENT injection and follow the “Instructions For Use” that comes with PRALUENT. In children aged 12 to 17 years, it is recommended that PRALUENT be given by or under the supervision of an adult. In children aged 8 to 11 years, PRALUENT should be given by a caregiver.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088.

Please click here for full Prescribing Information.

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 for over 35 years by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to numerous FDA-approved treatments and product candidates in development, almost 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, hematologic conditions, 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 more information, please visit www.Regeneron.com or follow Regeneron on LinkedIn.

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 Praluent® (alirocumab) for the treatment of pediatric patients aged 8 to 17 with heterozygous familial hypercholesterolemia; 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; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron’s Product Candidates and new indications for Regeneron’s Products; 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; safety issues resulting from the administration of Regeneron's Products (such as Praluent) and Regeneron's Product Candidates 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 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 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 (such as the COVID-19 pandemic) on Regeneron's business; 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® (aflibercept) Injection), 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, 2023. 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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