Evkeeza® (evinacumab-dgnb) ANGPTL3 Antibody Approved in the U.S. for Children as Young as 1 Year Old with Ultra-Rare Form of High Cholesterol

September 26, 2025 at 7:00 AM EDT

FDA approval extends the indication of Evkeeza to treat younger patients with homozygous hypercholesterolemia (HoFH)

HoFH is an inherited condition characterized by extremely high levels of low-density lipoprotein cholesterol (LDL-C)

Initial Evkeeza approval based on placebo-controlled trial showing Evkeeza, when added to standard lipid-lowering therapies, could lower LDL-C by about 50% compared to placebo in this high unmet need population

TARRYTOWN, N.Y., Sept. 26, 2025 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced that the U.S. Food and Drug Administration (FDA) has approved Evkeeza® (evinacumab-dgnb) ANGPTL3 antibody as an adjunct to diet and exercise and other lipid-lowering therapies for the treatment of children from age 1 to less than 5 years old with homozygous familial hypercholesterolemia (HoFH). Evkeeza was initially approved in 2021 for adults and adolescents aged 12 years and older with HoFH based on a placebo-controlled trial showing Evkeeza, when added to standard lipid-lowering therapies, could lower LDL-C by about 50% compared to placebo in this high unmet need population. It was then approved for children aged 5 to 11 in 2023. All Evkeeza FDA submissions were evaluated under Priority Review, which is reserved for medicines that represent potentially significant improvements in efficacy or safety in treating serious conditions.

HoFH is the most severe form of familial hypercholesterolemia (FH), and affects approximately 1,300 people in the U.S. It occurs when two copies of the FH-causing genes are inherited, one from each parent, resulting in dangerously high levels (usually >400 mg/dL) of low-density lipoprotein cholesterol (LDL-C). Those living with HoFH are at risk for premature atherosclerotic disease and cardiac events even in their teenage years. Many patients are not diagnosed or are only diagnosed later in life.

“The approval of Evkeeza for children as young as 1 year of age addresses a critical unmet need for those with homozygous familial hypercholesterolemia, a life-threatening condition that causes extraordinarily high LDL-C levels from birth,” said Katherine Wilemon, Founder and CEO of the Family Heart Foundation. “Families and their medical teams will now have an effective treatment option for these very young children who are at risk of serious complications from diseased arteries and calcified valves without timely and sufficient LDL-C lowering. This development underscores the importance of, and urgency needed in, identifying children with FH through pediatric screenings in accordance with guidelines.”

The extended indication for Evkeeza was supported by clinical efficacy and safety data among 6 children living with HoFH (including pharmacokinetic data among 4 of these patients) who took part in either the U.S. expanded access program or ex-U.S. compassionate use program for Evkeeza. No new safety concerns have been identified in the compassionate use program. The most common adverse reactions (≥5%) of Evkeeza include nasopharyngitis, influenza-like illness, dizziness, rhinorrhea, nausea, and fatigue.

“Evkeeza is a testament to the power of Regeneron’s science and proprietary technologies in developing first-in-class, lifechanging medicines that become standard-of-care,” said George D. Yancopoulos, M.D., Ph.D., Board co-Chair, President and Chief Scientific Officer at Regeneron. “This label extension adds to our growing commitment to the rare disease space, which includes diverse clinical development programs in neuromuscular and genetic diseases – such as myasthenia gravis, otoferlin hearing loss, and fibrodysplasia ossificans progressiva – for which we’ve shared data.”

Regeneron is committed to helping patients who have been prescribed Evkeeza access their medication through its myRARE® patient support program. myRARE offers financial assistance to eligible patients, as well as resources to help patients and healthcare providers get started with Evkeeza including product information, insurance benefit verification, community connections, and appointment reminders. For more information, call 1-833-EVKEEZA (833-385-3392) or visit www.EVKEEZA.com.

About Evkeeza® (evinacumab-dgnb) ANGPTL3 antibody

Evkeeza was invented using Regeneron’s *VelocImmune*® technology and is a fully human monoclonal antibody that binds to and blocks the function of angiopoietin-like 3 (ANGPTL3), a protein that inhibits lipoprotein lipase (LPL) and endothelial lipase (EL) and regulates circulating lipids, including LDL-C.

Regeneron scientists discovered the angiopoietin gene family more than two decades ago. Human genetics research published in *New England Journal of Medicine* in 2017 by scientists from the Regeneron Genetics Center® found that patients whose ANGPTL3 gene did not function properly (called a “loss-of function mutation”) have significantly lower levels of key blood lipids, including LDL-C, and that this is associated with a significantly lower risk of coronary artery disease.

The generic name for Evkeeza in its approved U.S. indications is evinacumab-dgnb, with dgnb the suffix designated in accordance with Nonproprietary Naming of Biological Products Guidance for Industry issued by the U.S. FDA.

Regeneron is responsible for the development and distribution of Evkeeza in the U.S. and is collaborating with Ultragenyx to clinically develop, commercialize and distribute Evkeeza outside of the U.S.

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 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.

U.S. INDICATION

EVKEEZA is an injectable prescription medicine used along with diet and exercise and other low-density lipoprotein-cholesterol (LDL-C) lowering medicines to lower LDL-C in adults and children 1 year of age and older with a type of high cholesterol called homozygous familial hypercholesterolemia (HoFH).

It is not known if EVKEEZA is safe and effective in children under 1 year of age.

IMPORTANT SAFETY INFORMATION

Do not use EVKEEZA if you are allergic to evinacumab-dgnb or to any of the ingredients in EVKEEZA.

Before receiving EVKEEZA, tell your healthcare provider about all of your medical conditions, including if you:

- Are pregnant or plan to become pregnant. EVKEEZA may harm your unborn baby. Tell your healthcare provider if you become pregnant while using EVKEEZA. **People who are able to become pregnant:**
  - Your healthcare provider may do a pregnancy test before you start treatment with EVKEEZA.
  - You should use an effective method of birth control during treatment and for at least **5 months** after the last dose of EVKEEZA. Talk with your healthcare provider about birth control methods that you can use during this time.
- Are breastfeeding or plan to breastfeed. It is not known if EVKEEZA passes into your breast milk. You and your healthcare provider should decide if you will receive EVKEEZA or breastfeed.

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

EVKEEZA can cause serious side effects, including:

- **Allergic reactions (hypersensitivity), including a severe reaction known as anaphylaxis.** Tell your healthcare provider right away if you get any of the following symptoms: swelling (mainly of the lips, tongue or throat which makes it difficult to swallow or breathe), breathing problems or wheezing, feeling dizzy or fainting, rash, hives, and itching.

The most common side effects of EVKEEZA include symptoms of the common cold, flu-like symptoms, dizziness, runny nose, nausea, and feeling tired or weak.

Tell your healthcare provider if you have any side effect that bothers you or does not go away. These are not all the possible side effects of EVKEEZA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see accompanying full Prescribing Information, including Patient 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 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 Evkeeza® (evinacumab-dgnb) as an adjunct to other lipid-lowering therapies for the treatment of children from age 1 to less than 5 years old with homozygous familial hypercholesterolemia; uncertainty of the utilization, market acceptance, and commercial success of Regeneron's Products (such as Evkeeza) 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, including Regeneron's Product Candidates for the treatment of neuromuscular and genetic diseases (such as myasthenia gravis, otoferlin hearing loss, and fibrodysplasia ossificans progressiva) as referenced in this press release; 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 (such as Evkeeza) 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 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 in laws, regulations, and policies affecting the healthcare industry; competing drugs and product candidates that may be superior to, or more cost effective than, Regeneron's Products and Regeneron's Product Candidates (including biosimilar versions of Regeneron's Products); 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), as well as Regeneron's collaboration with Ultragenyx Pharmaceutical Inc. referenced in this press release, 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, 2024 and its Form 10-Q for the quarterly period ended June 30, 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).

Contacts:

Media Relations
Ilana Yellen
Tel: +1 914-330-9618
Ilana.Yellen@regeneron.com

Investor Relations
Mark Hudson
Tel: +1 914-847-3482
Mark.Hudson@regeneron.com

Source: Regeneron Pharmaceuticals, Inc.