Pasatru™ (garetosmab-grts) First and Only FDA-approved Treatment Demonstrating Reduction in New Heterotopic Ossification (HO) Lesions and Clinician-Assessed Flare-ups in a Placebo-controlled Trial in Adults with Fibrodysplasia Ossificans Progressiva (FOP)

August 19, 2026 at 1:58 PM EDT

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

Approval based on results from the OPTIMA trial demonstrating a 90% or greater reduction in new HO lesions at 56 weeks with a dramatic reduction in clinician-assessed flare-ups in adults with FOP

TARRYTOWN, N.Y., Aug. 19, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced the U.S. Food and Drug Administration (FDA) has granted approval for Pasatru ™ (garetosmab-grts) to reduce formation of new heterotopic ossification (HO) lesions and clinician-assessed flare-ups in adults with fibrodysplasia ossificans progressiva (FOP). Recognizing the significant mobility challenges faced by many people with FOP, Pasatru can be administered across a range of care settings, including home infusion where appropriate. The recommended starting dosage is based on weight at 10 mg/kg and is given intravenously over 60 minutes once monthly (every four weeks) but may be decreased to a 3 mg/kg infusion over 60 minutes once monthly if not tolerated. Pasatru is a *VelocImmune*®-derived, fully human monoclonal antibody that blocks Activin A, a protein that Regeneron scientists discovered to be critical in the development of HO lesions in people with FOP.

FOP is an ultra-rare genetic disorder in which muscles, tendons, ligaments and other connective tissues are progressively infiltrated by rogue bone formation, a process known as HO. HO of the jaw, spine, hip and rib cage can make basic actions such as speaking, eating, walking or breathing difficult, and the dysfunction of these structures can lead to escalating loss of mobility. Worldwide, approximately 900 people are diagnosed with FOP, and most patients are wheelchair-bound by age 30 with the median age of survival being 56.

“For people living with FOP, every irregular new bone formation is a step toward disability and potential loss of mobility,” said Dr. Kathryn Dahir, Professor in the Department of Internal Medicine, Division of Endocrinology, Diabetes, and Metabolism at Vanderbilt University, and a primary investigator for the OPTIMA trial. “With the ability to reduce the number of new bone lesions and flare-ups, we now have a new treatment that can positively affect patients.”

“FOP is a relentless, restrictive condition that severely impacts the lives of those diagnosed and their families,” said Michelle Davis, Executive Director of the International FOP Association. “This approval is monumental for our community, providing a vital new therapy that can have a significant impact on the life of someone with FOP."

Pasatru was granted approval based on efficacy and safety data from the positive Phase 3 OPTIMA trial evaluating Pasatru in adults with FOP. At 56 weeks, both doses of Pasatru, 10 mg/kg (n=23) and 3 mg/kg (n=19), met the primary endpoint and were highly efficacious in reducing the total number of new HO lesions as compared to placebo (n=21), demonstrating a 90% (2 lesions vs. 19 lesions) and 94% (1 lesion vs. 19 lesions) reduction, respectively, as assessed by computed tomography (CT) scan. The number of clinician-assessed flare-ups during this time, a key secondary endpoint, were 9 for Pasatru 10 mg/kg (88% reduction compared to placebo), 53 for Pasatru 3 mg/kg (15% reduction compared to placebo), and 66 for placebo. Changes in the proportion of patients with patient-reported flare-ups through week 56 were not significantly different between placebo and Pasatru treatment groups. At 56 weeks, among all 63 people who participated in the trial, serious treatment-emergent adverse events occurred in 2 patients treated with 10 mg/kg Pasatru, 1 patient treated with 3 mg/kg Pasatru, and 2 patients treated with placebo. The most common adverse reactions occurring in ≥10% of adults with FOP treated with Pasatru 10 mg/kg or 3 mg/kg were abscess, acne, increased hair growth, madarosis (loss of eyebrows), oral ulcers, epistaxis (nosebleeds), folliculitis, paronychia (nail infection), and rash.

“The approval of Pasatru is the culmination of decades of pioneering research that Regeneron has pursued alongside the FOP community, rooted in our discovery of the role that Activin A plays in driving this disease,” said George D. Yancopoulos, M.D., Ph.D., Board co-Chair, President and Chief Scientific Officer at Regeneron. “People living with FOP have needed a new treatment option for this devastating condition far too long, much like many others living with a rare disease. This unprecedented milestone embodies our broader mission to continue delivering new options for those who have long faced limited treatment options and our relentless commitment to bringing scientific breakthroughs to families, no matter the prevalence of the condition they manage.”

Regeneron is committed to supporting people living with rare diseases. Regeneron’s myRARE™ program offers resources to help

patients and healthcare providers, including product information, insurance benefit verification, and information about potential financial support. To learn about myRARE, visit myRARE.com or call 1-833-4my-RARE (1-833-469-7273).

In the European Union, a regulatory submission for Pasatru is currently under review by the European Medicines Agency. Additional regulatory submissions are planned in countries around the world, including Japan. Pasatru previously received Fast Track designation and Orphan Drug Designation from the FDA, as well as Orphan Designation by the European Medicines Agency in the European Union and the Ministry of Health, Labour and Welfare in Japan.

About the OPTIMA Clinical Trial
OPTIMA is a Phase 3, multi-center, multinational trial to assess the efficacy of Pasatru on the reduction of heterotopic bone formation and flares, 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 scoring tool used by clinicians to assess the degree of joint involvement, with higher scores representing a greater degree of disease severity (scale: 0 to 30). Eligible participants were randomized to receive intravenously administered Pasatru 10 mg/kg, Pasatru 3 mg/kg, or placebo once every four weeks for 56 weeks. Following this, participants could elect to then continue their originally assigned treatment in a double-blind extension phase for at least 84 weeks or discontinue treatment and enter an observation-only arm.

During the treatment period, efficacy was evaluated through whole body 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 Pasatru 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 Pasatru
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. Pasatru 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.

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about PASATRU?

PASATRU can cause serious side effects, including:

- **Harm to your unborn baby including serious birth defects if taken during pregnancy. Females who are pregnant must not take PASATRU.**
  - **Females who can become pregnant:**
    - Your healthcare provider will ask you to take a pregnancy test to verify that you are not pregnant before starting treatment with PASATRU.
    - Use effective birth control (contraception) during treatment with PASATRU and for 6 months after the last dose of PASATRU. Talk to your healthcare provider about birth control methods that may be right for you.
    - If you become pregnant or think you may be pregnant during treatment with PASATRU, **stop taking PASATRU immediately and call your healthcare provider right away**.
- **Infections of the skin and tissue under the skin** requiring treatment or hospitalization, such as infected lumps (abscesses) and bacterial skin infections (cellulitis), may happen while you are taking PASATRU. Call your healthcare provider right away if you have signs or symptoms of skin infection, such as:
  - redness
  - skin feels warm to the touch
  - swelling
  - pain or tenderness
  - fever
  - feeling generally unwell
- **Nosebleeds (epistaxis)** including serious nosebleeds that require medical care, can happen while taking PASATRU. **Tell your healthcare provider right away if you have a nosebleed that:**
  - is severe or heavy
  - does not stop with basic first-aid measures, such as pinching your nose with continuous firm pressure
  - lasts more than 20 minutes

Do not receive PASATRU if you are pregnant.

Before you receive PASATRU, tell your healthcare provider about all your medical conditions, including if you:

- are breastfeeding. It is not known whether PASATRU passes into your breastmilk. Breastfeeding is not recommended during treatment with PASATRU and for 6 months after the last dose of PASATRU. Talk to your healthcare provider about the best way to feed your baby during this time.
- are concerned about male fertility. PASATRU may affect your ability to father a child. Talk to your healthcare provider if this is concern for you.

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

The most common side effects of PASATRU include:

- infected lumps (abscess)
- acne
- increased hair growth
- hair loss of eyebrows or lashes (madarosis)
- mouth sores (oral ulcers)
- nosebleeds (epistaxis)

These are not all of the possible side effects of PASATRU. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

What is PASATRU?

PASATRU ™ (garetosmab-grts) is a prescription medicine used to reduce the formation of new abnormal bone growth outside of the skeleton (heterotopic ossification) and flare-ups in adults with fibrodysplasia ossificans progressiva (FOP).

It is not known if PASATRU is safe and effective in children.

Please click here for full Prescribing Information, including Medication Guide.

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), Veopoz® (pozelimab-bbfg) and Pasatru ™ (garetosmab-grts). In addition, REGEN-COV® (casirivimab and imdevimab) had been authorized by the FDA during the COVID-19 pandemic until 2024. A total of 11 antibody and bispecific products have been approved or authorized worldwide that were invented or derived using *VelocImmune* technologies.

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 Pasatru™ (garetosmab-grts) to reduce the formation of new heterotopic ossification lesions and clinician-assessed flare-ups in adults with fibrodysplasia ossificans progressiva; uncertainty of the utilization, market acceptance, and/or commercial success of Regeneron's Products (such as Pasatru) 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 and risks associated with tariffs and other trade restrictions; safety issues resulting from the administration of Regeneron's Products (such as Pasatru) 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 to drug pricing regulations and requirements and Regeneron's pricing strategy, including in connection with Regeneron's April 2026 agreements with the U.S. government; 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 and its Form 10-Q for the quarterly period ended June 30, 2026. 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

REGENERON

Source: Regeneron Pharmaceuticals, Inc.