



Pasatru™ (garetosmab-grts) Demonstrates Zero New Heterotopic Ossification (HO) Lesions in Adult Fibrodysplasia Ossificans Progressiva (FOP) Patients During OPTIMA Trial Extension

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Pasatru is the first and only FDA-approved treatment demonstrating reductions in both new HO lesions and clinician-assessed flare-ups, per results of a 56-week placebo-controlled trial in adults with FOP

Late-breaking data presented at ASBMR show sustained efficacy of Pasatru and a consistent safety profile from weeks 56 to 84, supporting long-term disease control

TARRYTOWN, N.Y., Oct. 12, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced positive 84-week results from the Phase 3 OPTIMA trial evaluating Pasatru™ (garetosmab-grts) in adults with fibrodysplasia ossificans progressiva (FOP). These longer-term findings for Pasatru demonstrated sustained efficacy in halting new, rogue bone growth and in significantly reducing disease flare-ups, and were presented during a late-breaking oral session at the American Society for Bone and Mineral Research (ASBMR) Annual Meeting. Pasatru was recently approved by the U.S. Food and Drug Administration (FDA) based on a 56-week, placebo-controlled trial to reduce the formation of new heterotopic ossification (HO) lesions and clinician-assessed flare-ups in adults with FOP. Pasatru is a *VelocImmune*®-derived, fully human monoclonal antibody that binds to and inhibits signaling by the Activin A protein, which Regeneron scientists [discovered](#) to be a critical protein involved in the development of HO lesions in people with FOP.

"FOP is a devastating, relentless condition in which every flare-up can bring anxiety around potential new, abnormal bone growth and resulting loss of mobility," said Professor Richard Keen, Director of the Metabolic Bone Disease Centre, Royal National Orthopaedic Hospital, London, and global primary investigator of the OPTIMA trial. "What patients urgently need is long-term control over new HO lesions and flare-ups to help bring peace of mind. The 84-week data from the Phase 3 OPTIMA trial demonstrate sustained disease-modifying control alongside a generally well-tolerated safety profile, which is important for the adult FOP community."

OPTIMA, a global, multi-center, randomized, double-blind, placebo-controlled trial, enrolled 63 people with FOP aged 18 years and older. Trial 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, 58 participants completed the double-blind extension phase to at least 84 weeks.

At 56 weeks, OPTIMA met its primary endpoint, demonstrating a 90% or greater reduction in new HO lesions, with a dramatic reduction in clinician-assessed flare-ups in adults with FOP. Detailed results on these 56-week data were recently published in [The Lancet](#). The endpoints were then re-assessed at 84 weeks. From week 56, there were no new HO lesions observed at week 84 for Pasatru compared to 9 new lesions for placebo as assessed by computed tomography (CT) scan. Additionally, one new HO lesion previously detected at week 56 for Pasatru 3 mg/kg was no longer detectable at week 84. The number of new clinician-assessed flare-ups was also reduced compared to placebo, notably with 1 observed for Pasatru 10 mg/kg, 14 for Pasatru 3 mg/kg, and 17 for placebo.

Through week 84, both doses of Pasatru had a generally well-tolerated safety profile, consistent with that observed at 56 weeks. At 56 weeks, the most common adverse reactions occurring in ≥10% of adults with FOP treated with Pasatru were abscess, acne, increased hair growth, madarosis (hair loss of eyebrows or lashes), oral ulcers, epistaxis (nosebleeds), folliculitis, paronychia (nail infection), and rash. At 84 weeks, the most common treatment-emergent adverse events were acne in participants treated with Pasatru 10 mg/kg and epistaxis (all non-severe) in participants treated with Pasatru 3 mg/kg. Treatment-emergent bleeding events were lower in participants treated with Pasatru 10 mg/kg than those treated with Pasatru 3 mg/kg or placebo. There was a dose-dependent increase in skin and soft tissue infections (33% on placebo, 53% on Pasatru 3 mg/kg, and 78% on Pasatru 10 mg/kg) that were all resolved with further management, with no patients discontinuing treatment as a result of infection. No deaths were reported in the trial.

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 FOP

FOP is an ultra-rare genetic disorder in which muscle, ligaments, and connective tissue 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, leading to skeletal deformity, weight loss, and escalating loss of mobility. Worldwide, approximately 900 people are known to have been diagnosed with FOP, and most patients become wheelchair users by age 30, with the median

age of survival being approximately 56.

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 then elect to 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. The safety assessment includes reports of adverse events, measurement of vital signs, physical examination, and coagulation testing.

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 to and inhibits signaling by the Activin A protein, which is involved in the development of heterotopic bone in people with FOP.

Pasatru is FDA-approved in the U.S. to reduce formation of new HO lesions and clinician-assessed flare-ups in adults with FOP. 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.

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).

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)
- hair loss of eyebrows or lashes (madarosis)
- acne
- mouth sores (oral ulcers)
- increased hair growth
- 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), Inmazed® (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™ (garetoismab-grts) for the treatment of adults with fibrodysplasia ossificans progressiva ("FOP"); 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, including Pasatru for the treatment of adolescents and children with FOP; 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>).

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