Otarmeni™ (lunsotogene parvec-cwha) Approved by FDA as First and Only Gene Therapy for Genetic Hearing Loss; Regeneron to Provide Otarmeni for Free in the U.S.

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Approval in severe-to-profound and profound *OTOF*-related hearing loss is based on pivotal results of the CHORD trial demonstrating 80% of participants achieved or surpassed a hearing level meeting the primary endpoint, and with longer follow-up, 42% achieved normal hearing that included whispers

First FDA-approved example of a gene therapy to restore a neurosensory function to normal levels

Otarmeni is Regeneron's first approved genetic medicine, showcasing its ability to advance new therapeutic approaches to address conditions with great unmet medical need

TARRYTOWN, N.Y., April 23, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced the U.S. Food and Drug Administration (FDA) has granted accelerated approval for Otarmeni™ (lunsotogene parvec-cwha), the first gene therapy and second new molecular entity approved under the FDA Commissioner's National Priority Voucher program. Otarmeni is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric and adult patients with severe-to-profound and profound sensorineural hearing loss (any frequency >90 decibel hearing level [dB HL]) associated with molecularly confirmed biallelic variants in the *OTOF* gene, preserved outer hair cell function, and no prior cochlear implant in the same ear. Otarmeni, formerly known as DB-OTO, is the first and only *in vivo* gene therapy for *OTOF*-related hearing loss and will be made available by Regeneron for free in the U.S.

Otarmeni was granted accelerated approval based on the improvement of hearing sensitivity by average pure tone audiometry (PTA) at week 24. Continued approval for this indication may be contingent upon verification and description of clinical benefit in the confirmatory portion of the CHORD clinical trial. Otarmeni is not recommended in patients in whom preoperative imaging demonstrates that access to the inner ear is not feasible, including those with abnormal mastoid pneumatization or clinically significant anatomic variations of the middle ear and inner ear.

"The FDA approval of Otarmeni signals a new era in the treatment of genetic forms of hearing loss, where reinstating 24/7 natural hearing is now possible," said A. Eliot Shearer, M.D., Ph.D., otolaryngologist in the department of Otolaryngology and Communication Enhancement at Boston Children's Hospital, Associate Professor of Otolaryngology-Head and Neck Surgery at Harvard Medical School and a CHORD trial investigator. "In the pivotal trial, the one-time gene therapy demonstrated rapid, meaningful and consistent hearing responses, with most children achieving remarkable hearing improvements. I've witnessed firsthand my trial participant responding to their mother's voice, dancing to music and interacting with the world, and these moments are now possible for more children born with this specific form of hearing loss."

"Otarmeni is a huge scientific leap and is representative of Regeneron's approaches to continually push the boundaries of science to benefit humanity," said George D. Yancopoulos, M.D., Ph.D., Board co-Chair, President and Chief Scientific Officer of Regeneron. "This unprecedented breakthrough in gene therapy has already proven to be life-changing for many of the children in our clinical trial and their families. We are honored to be in the position to be the first company to ever offer such a gene therapy advance for free to those in the U.S. and serves to highlight our belief that the biopharmaceutical industry can be a genuine force for good in the world."

OTOF-related hearing loss is an ultra-rare condition, affecting about 50 newborns per year in the U.S. Though all structures within the ear are intact, variants in the *OTOF* gene cause a lack of a functional otoferlin protein, which is critical for communication between the sensory cells of the inner ear and the auditory nerve. Historically, genetic *OTOF*-related hearing loss was considered permanent and managed with life-long use of devices. While these devices can amplify sound to improve hearing for individuals with a range of hearing loss, they do not currently restore the full spectrum of sound.

"Connection and communication are at the heart of how we experience the world – whether that happens through listening and spoken language, sign language, the use of technology, or a combination of approaches," said Janet DesGeorges, Executive Director of Hands & Voices. "Families deserve access to balanced information and a range of options when navigating genetic hearing loss. As new treatments and innovations emerge, families can assess available options and choose the approach best suited to their unique circumstances."

The FDA approval is based on results from the pivotal CHORD trial, in which 20 participants (aged 10 months to 16 years) received a single dose of Otarmeni via intracochlear infusion, either unilaterally (in one ear; n=10) or bilaterally (in both ears; n=10). Among the trial participants, efficacy results found:

- **80% (16 of 20) experienced hearing improvements per pure tone audiometry assessments** at a threshold of ≤70 dB HL at 24 weeks, achieving the trial's primary endpoint; one additional participant achieved this threshold by week 48. This threshold corresponds to a clinical standard that enables natural hearing and typically does not require cochlear implantation.
- **70% (14 of 20) demonstrated an auditory brainstem response** (ABR) at ≤90 decibels at 24 weeks, achieving the trial's key secondary endpoint. ABR is an objective confirmation of hearing function, as measured by recording electrical brainstem signals in response to sound.
- **For those followed to 48 weeks, all prior responders maintained a response to therapy, and 42% of all participants (5 of 12) achieved normal hearing that included whispers** (≤25 dB HL).

The most common adverse reactions (≥5%) in the safety population of CHORD (n=24) associated with Otarmeni include otitis media, vomiting, nausea, dizziness, procedural pain, gait disturbance, and nystagmus. The surgical procedure to administer Otarmeni uses an approach similar to cochlear implantation and allows use in young infants. Otarmeni should be administered by a surgeon experienced in intracochlear surgery and trained in the Otarmeni administration process and should only be administered using the provided Administration Kit for use with Otarmeni.

Regeneron will provide Otarmeni at no cost to clinically eligible individuals in the U.S. This may not necessarily reflect out-of-pocket costs for administration of this free therapy, which would be outside of the control of Regeneron; individuals should consult with their healthcare provider and/or insurance provider. For more information on access, contact Regeneron's OnPath with OTARMENI™ patient support program at 1-866-500-GENE (1-866-500-4363).

Otarmeni received Orphan Drug, Rare Pediatric Disease, Fast Track and Regenerative Medicine Advanced Therapy designations from the FDA. The European Medicines Agency also granted Orphan Drug Designation. Regulatory submissions are planned in additional markets.

About Otarmeni™ (lunsotogene parvec-cwha)
Otarmeni is an adeno-associated virus vector-based gene therapy indicated for the treatment of certain pediatric and adult patients with severe-to-profound and profound sensorineural hearing loss caused by variants of the *OTOF* gene that produce non-functioning otoferlin protein. The treatment is designed to restore durable, physiological hearing to individuals by delivering a working copy of the *OTOF* gene through a modified, non-pathogenic virus that is delivered via an infusion into the cochlea under general anesthesia (similar to the procedure used for cochlear implantation). In this gene therapy, the newly introduced *OTOF* gene is under the control of a proprietary cell-specific *Myo15* promoter, which is intended to restrict expression only to hair cells that normally express the otoferlin protein.

About the CHORD Trial
The CHORD trial is an ongoing, registrational Phase 1/2 multicenter, open-label trial to evaluate the safety, tolerability and efficacy of Otarmeni in infants, children and adolescents with *OTOF*-related hearing loss. The trial is currently enrolling children (<18 years of age) across sites in the U.S., United Kingdom, Spain, Germany and Japan.

CHORD is being conducted in two parts. In the initial dose-escalation cohort (Part A), participants receive a single intracochlear infusion of Otarmeni in one ear. In the expansion cohort (Part B), participants receive Otarmeni in both ears at the selected dose from Part A.

Hearing improvements were assessed by PTA and ABR. PTA is the gold standard measurement of hearing sensitivity and is measured through behavioral responses to sound (e.g., turning head towards sound) that is emitted at different intensity levels and measured in dB. ABR corroborates these behavioral responses, serving as an objective confirmation of hearing function, and is measured through recording electrical brainstem responses to sound emitted at different intensity levels measured in dBs. At baseline, all participants had profound hearing loss (behavioral PTA), and no electrophysiological (ABR) responses at maximum sound levels.

IMPORTANT SAFETY INFORMATION

What is the most important information to know about OTARMENI?

Before receiving OTARMENI:

- it is recommended to receive age-appropriate vaccinations, at least 1 month before the first corticosteroid dose and at least 1 month after the last corticosteroid dose.
- consult with your healthcare provider and surgeon regarding vaccination status against meningitis, since meningitis is a known risk of inner ear surgery.
- pregnancy status should be verified (if you are sexually active and able to become pregnant).

The following serious side effects may occur with the surgery required to administer OTARMENI:

- Vertigo, ringing in ear(s), cerebral spinal fluid leak, partial facial paralysis or weakness, change in taste, meningitis, wound infection, serious infection of the bone behind the ear (mastoiditis), numbness around the ear, blood or fluid collection at surgical site, and inflammation of the inner ear.

The most common side effects that may occur with OTARMENI include middle ear infection, vomiting, nausea, dizziness,

procedural pain, walking disturbance, and rapid involuntary eye movements.

Other clinically significant side effects, each occurring in 1 person in the clinical study, included temporary balance disorder, abnormal otoacoustic emissions, and wound separation.

Because small quantities of OTARMENI may be present in bodily fluids/waste, any materials that may be contaminated should be placed in a sealable bag and disposed of into regular trash for the first two weeks following administration of OTARMENI. Practice proper hand hygiene, such as hand washing, when coming into direct contact with bodily fluids/waste.

Talk to your healthcare provider for medical advice or any questions about side effects. 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. You may also report side effects to Regeneron Pharmaceuticals at 1-866-500-GENE (1-866-500-4363).

Please see accompanying 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 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 Otarmeni™ (lunsotogene parvec-cwha), an adeno-associated virus vector-based gene therapy for the treatment of pediatric and adult patients with severe-to-profound sensorineural hearing loss; whether and to what extent Otarmeni's clinical benefit will be verified in the confirmatory clinical trial referenced in this press release and any impact of the foregoing on the regulatory approval of Otarmeni discussed in this press release or any other potential regulatory approval; uncertainty of the utilization, market acceptance, and/or commercial success of Regeneron's Products (such as Otarmeni) 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 Otarmeni) 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; 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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