Regeneron to Highlight Advances in Genetic Medicine Research at American Society of Gene and Cell Therapy (ASGCT)

April 22, 2024 at 5:54 PM EDT

Oral presentations include updated results from clinical study of otoferlin gene therapy DB-OTO demonstrating restoration in children with profound genetic hearing loss

Additional presentations cover progress in novel genetic medicine delivery systems and immune response modulation

TARRYTOWN, N.Y., April 22, 2024 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced that new and updated data across its genetic medicines portfolio will be presented at the American Society of Gene and Cell Therapy (ASGCT) annual conference in Baltimore, Maryland, from May 7 to 11, 2024. Data from 10 abstracts, including six oral presentations, provide insight on Regeneron's approach to overcoming obstacles to clinical implementation of genetic medicines, from pre-dosing to delivery to long-term sustained expression. The company will also present updated data from the Phase 1/2 CHORD trial investigating DB-OTO in children with profound genetic hearing loss due to mutations of the otoferlin gene.

"Genetic medicine approaches including gene therapy, gene editing and gene silencing hold incredible promise for people with serious, genetically driven diseases, but some common barriers to practical implementation remain, such as delivery to tissues beyond the liver and waning efficacy over time," said Christos Kyratsous, Ph.D., Senior Vice President and Co-Head of Regeneron Genetic Medicines. "Regeneron continues to advance methods to overcome these obstacles through our proprietary delivery approaches utilizing next-generation viral vectors, particularly specific retargeting antibodies and innovative payloads. Our data at ASGCT also details efforts to sustain expression of treatment over time and better modulate immune response via adeno-associated virus delivery."

"We are continuing to dose patients in our clinical trial of DB-OTO gene therapy for profound hearing loss due to otoferlin deficiency and are advancing additional gene therapy programs toward the clinic. The ASGCT presentation will build on promising early results in the first patient," said Aris Baras, M.D., Senior Vice President, Co-Head of Regeneron Genetic Medicines and Head, Regeneron Genetics Center®. "These results raise hope and enthusiasm for the field, and we believe that findings from the program will help us unlock paths forward for gene therapies and genetic medicines for more patients and diseases."

Regeneron presentations at ASGCT:

Abstract title	**Abstract**	**Presenting/Lead Author**	**Presentation date/time (ET)**
Oral Presentations			
Intracochlear Administration of DB-OTO Gene Therapy in Pediatric Patients with Profound Hearing Loss Due to Otoferlin Mutations: The CHORD Phase 1/2 Open-Label Trial	10	Lawrence Lustig, Columbia University	Wednesday, May 8, 9:30-9:45AM ET
Antibody-Based AAV Retargeting to Transferrin Receptor Mediates Efficient Blood Brain Barrier Crossing and *In Vivo* Gene Delivery to the CNS in Mice and Non-Human Primates	118	Kalyani Nambiar	Wednesday, May 8, 4:15-4:30PM ET
Targeted Gene Insertion of Vectorized Monoclonal Antibodies in Non-Human Primates Overcomes AAV Genome Silencing in the Liver and Supports High, Sustained *In Vivo* Expression of Functional Antibodies	197	Rachel Sattler	Thursday, May 9, 5:15-5:30pm ET
Retargeting of AAV Using Bispecific Antibodies	218	Sven Moller-Tank	Thursday, May 9, 5:15-5:30PM ET
Tissue De-Targeting Abrogates Hepatotoxicity and Complement-Related Thrombotic Complications Associated with High-Dose AAV Gene Therapies	298	Andrew Baik	Friday, May 10, 4:15-4:30PM ET
Orthogonal B Cell and Plasma Cell Immunosuppression Strategies Prevent and Suppress High-Titer Antibody Immunity to Enable AAV Vector Re-Dosing	353	Nicholas Giovannone	Saturday, May 11, 8:15-8:30AM ET
Poster Presentations			
AAV Conjugated to Antibodies Against $p75^{NTR}$: A New Platform to Deliver Pain Therapeutics to Nociceptive Sensory Neurons	638	Adina Buxbaum	Wednesday, May 8, 12:00-7:00PM ET
A Process for Identifying AAV and Transgene Integrations in Mouse and Human Genomes Using Long Read Oxford Nanopore Sequencing	897	Terrence Turner	Wednesday, May 8, 12:00-7:00PM ET
DNA Leakage of rAAV Under Freeze/Thaw Stress and Analytical Method Development for Free DNA Characterization	898	Shuai Li	Wednesday, May 8, 12:00-7:00PM ET

Identification of Degradation Pathways of rAAV8 to Aid Stable Drug Product Formulation Development	899	Ariel Chen	Wednesday, May 8, 12:00-7:00PM ET
Lectures			
Engineering CAR-T Cells with Novel Receptor Architectures	N/A	Philip Gregory	Thursday, May 9, 10:55-11:25AM ET
Pressing Challenges in Gene Therapy	N/A	Jim Wang	Saturday, May 11, 8:00-9:45AM ET

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, many 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 pushes the boundaries of scientific discovery and accelerates drug development using our proprietary technologies, including *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 the investigational gene therapy DB-OTO as discussed in this press release as well as Regeneron's other genetic medicine programs referenced in this press release; the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products, such as DB-OTO in children with profound genetic hearing loss due to mutations of the otoferlin gene; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees (including those referenced in this press release) may be further replicated and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; the potential of the Company's novel genetic medicine delivery systems and approaches to immune response modulation discussed or referenced in this press release; 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 or any potential regulatory approval of Regeneron's Products and Regeneron's Product Candidates (such as DB-OTO); 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 and Regeneron's Product Candidates (such as DB-OTO) 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; 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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