Ultragenyx Announces Upcoming Data Presentations at American Society of Gene & Cell Therapy (ASGCT) 2023 Annual Meeting

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Oral presentations include longer term durability data from DTX301 and DTX401 and showcase the Pinnacle PCL™ (AAV vector producer cell line) platform and technology

NOVATO, Calif., May 08, 2023 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for rare and ultrarare diseases, today announced that clinical, preclinical and manufacturing data from its investigational gene therapy programs will be presented at the American Society of Gene & Cell Therapy (ASGCT) 26th Annual Meeting, which will be held May 16-20, 2023 at the Los Angeles Convention Center in Los Angeles, California.

The company will present additional data supporting its gene therapy portfolio and discuss critical topics during the clinical trials spotlight symposium and at pre-meeting workshops.

Clinical and pre-clinical presentations:

- Oral presentation: Efficacy and safety at week 52 and up to four years in adults with glycogen storage disease type Ia (GSDIa): Results from a Phase 1/2 clinical trial and long-term follow-up study of DTX401, an AAV-8-mediated, liver-directed gene therapy (Abstract #4)
  - Date/Time: Thursday, May 18, 8:00 – 8:15 AM PDT
  - Location: Concourse Hall 152 and 153
  - First author: Rebecca Riba-Wolman, M.D., University of Connecticut

- Oral presentation: Long-term Safety and Efficacy of DTX301 in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: A Phase 1/2 Trial (Abstract #128)
  - Date/Time: Thursday, May 18, 2:45 PM - 3:00 PM PDT
  - Location: Room 403 AB
  - Presenter: Cary Harding, M.D., Oregon Health & Science University

- Poster presentation: Immunosuppression to Inhibit Capsid-Specific Humoral Immune Responses in High-dose AAV Gene Therapy in Cynomolgus Macaques (Board No. 1191)
  - Date/Time: Thursday, May 18, 12:00 PM PDT
  - Presenter: Deniz Erturk-Hasdemir, Ph.D., Ultragenyx

Additional presentations including the company's manufacturing capabilities and Pinnacle PCL™ (AAV vector producer cell line) platform and technology:

- Oral presentation: *In Vivo* and *In Vitro* Assessment of Residual DNA Impurity-Derived Transcriptions (Abstract #109)
  - Date/Time: Thursday, May 18, 1:30 – 1:45 PM PDT
  - Location: Concourse Hall 150 and 151
  - Presenter: Hsin-I Jen, Ph.D., Ultragenyx

- Poster presentation: Early Development of *In Vitro* Potency Assays for Rare and Ultrarare Disease Gene Therapy Products (Board No. 782)
  - Date/Time: Wednesday, May 17, 12:00 PM PDT
  - Presenter: Elena Balkanska-Sinclair, Ultragenyx

- Poster presentation: Global Strategy to Improve the Downstream Manufacturing Process of

the Adeno-associated Viral Vector for Glycogen Storage Disease Type Ia Treatment (Board No. 412)

  - Date/Time: Wednesday, May 17, 12:00 PM PDT
  - Presenter: Chao Huang, Ph.D., Ultragenyx

- Poster presentation: Genome-wide analysis of triple-play plasmid integration in Pinnacle PCL™ producer cell lines across multiple rAAV programs (Board No. 1162)

  - Date/Time: Thursday, May 18, 12:00 PM PDT
  - Presenter: Nicholas Richards, M.S., Ultragenyx

- Poster presentation: Identification of novel mutations in PCCA and PCCB genes from Propionic Acidemia patient fibroblasts via long-read sequencing (Board No. 972)

  - Date/Time: Thursday, May 18, 12:00 PM PDT
  - Presenter: Rea Guertler, M.Sc., Ultragenyx

- Poster presentation: Development of innovative, scalable, high productivity manufacturing process to enable Ph1/2 clinical supply of UX810, an investigational AAV gene therapy to treat Duchenne's Muscular Dystrophy (Board No. 1424)

  - Date/Time: Friday, May 19, 12:00 PM PDT
  - Presenter: Wei Xue, Ph.D., Ultragenyx

- Poster presentation: Development of UX055 AAV Gene Therapy for Cyclin-dependent Kinase-like 5 (CDKL5) Deficiency Disorder (CDD), a Rare Neurological Disorder (Board No. 1340)

  - Date/Time: Friday, May 19, 12:00 PM PDT
  - Presenter: Julie Wei, Ph.D., Ultragenyx

- Poster presentation: Rare Disease Patient Advocacy Perspectives on the Promise and Challenges of Gene Therapy (Board No. 1578)

  - Date/Time: Friday, May 19, 12:00 PM PDT
  - Presenter: Kristin Voorhees, Ultragenyx

In addition to the data presentations, Ultragenyx will present at one pre-meeting workshop on Tuesday, May 16:

- Pre-meeting workshop: How to Become a Site for AAV Gene Therapy Trials
  - Time: 8:25-8:50 AM PDT
  - Location: Concourse Hall 152 and 153
  - Presenter: Sandra Nino-Siddens, Pharm.D., Ultragenyx

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel therapies to patients for the treatment of serious rare and ultrarare genetic diseases. The company has built a diverse portfolio of approved medicines and treatment candidates aimed at addressing diseases with high unmet medical need and clear biology, for which there are typically no approved therapies treating the underlying disease.

The company is led by a management team experienced in the development and commercialization of rare disease therapeutics. Ultragenyx's strategy is predicated upon time- and cost-efficient drug development, with the goal of delivering safe and effective therapies to patients with the utmost urgency.

For more information on Ultragenyx, please visit www.ultragenyx.com.

Forward-Looking Statements
Except for the historical information contained herein, the matters set forth in this press release, including statements related to Ultragenyx's expectations and projections regarding its future operating results and financial performance, anticipated cost or expense reductions, the timing, progress and plans for its clinical programs and clinical studies, future regulatory interactions, and the components and timing of regulatory submissions are forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements involve substantial risks and uncertainties that could cause our clinical development programs, collaboration with third parties, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, risks related to reliance on third party partners to conduct certain activities on the company's behalf, uncertainty and potential delays related to clinical drug development, the company's ability to achieve its projected development goals in its expected timeframes, risks related to undesirable or serious side effects from our product candidates, risks and uncertainties related to the regulatory approval process, smaller than anticipated market opportunities for the company's products and product candidates, manufacturing risks, competition from other therapies or products, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations, the company's future operating results and financial performance, the timing of clinical trial activities and reporting results from same, and the availability or commercial potential of Ultragenyx's products and drug candidates. Ultragenyx undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of Ultragenyx in general, see Ultragenyx's Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 5, 2023, and its subsequent periodic reports filed with the SEC.
In addition to its SEC filings, press releases and public conference calls, Ultragenyx uses its investor relations website and social media outlets to publish important information about the company, including information that may be deemed material to investors, and to comply with its disclosure obligations under Regulation FD. Financial and other information about Ultragenyx is routinely posted and is accessible on Ultragenyx's investor relations website (https://ir.ultragenyx.com/) and LinkedIn website (https://www.linkedin.com/company/ultragenyx-pharmaceutical-inc-/).

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