Ultragenyx Announces Upcoming Data Presentations at American Society of Gene & Cell Therapy 2020 Virtual Annual Meeting

April 28, 2020

NOVATO, Calif., April 28, 2020 (GLOBE NEWSWIRE) -- Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for rare and ultra-rare 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) 23rd Annual Meeting, which will be held virtually May 12-15, 2020. Information will be available at www.asgct.org.

Nine Ultragenyx abstracts have been accepted for virtual presentation, including:

- An oral presentation of available data from the confirmatory cohort of the Phase 1/2 study of DTX401, an AAV-based gene therapy for the treatment of glycogen storage disease Type Ia (GSDIa) (Abstract #1306)
- An oral presentation of new data from the first three cohorts of the Phase 1/2 study of DTX301, an investigational adeno-associated virus (AAV) gene therapy for the treatment of ornithine transcarbamylase (OTC) deficiency (Abstract #505)
- Multiple presentations, including two orals, highlighting the HeLa producer cell line platform and improvements in the HEK293 triple transfection system (Abstracts #539 and #543)

“We have made meaningful progress across all of our gene therapy programs, including continued improvements to both the HeLa PCL and HEK293 transient transfection manufacturing technology platforms which we believe will enable highly reproducible, more consistent and scalable gene therapy manufacturing,” said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. “In addition to the presentations that support our gene therapy manufacturing approach, we look forward to sharing first-time results from the confirmatory cohort of our GSDIa study and updated clinical data from the OTC study.”

Details of the nine presentations are as follows:

Tuesday, May 12
Oral Presentation
Abstract #98: AAV9/*hCDKL5* delivery to cerebrospinal fluid of juvenile CDKL5-deficient mice improves learning and memory and motor function in adult mice

- Time: 5:15-5:30 PM ET

Poster Presentation
Abstract #172: Characterization and correction of an *in vitro* model of Wilson Disease by recombinant adeno-associated virus (rAAV) delivered *ATP7B* transgene

- Time: 5:30-6:30 PM ET

Poster Presentation
Abstract #451: Rapid CMC development and pre-commercial considerations for rAAV gene therapy products for rare diseases

- Time: 5:30-6:30 PM ET

Wednesday, May 13
Oral Presentation
Abstract #539: HeLa 3.0: CRISPR knockout of genes modulating titer in established rAAV-producing cell lines

- Time: 4:00-4:15 PM ET

Oral Presentation
Abstract #505: AAV8 gene therapy as a potential treatment in adults with late-onset OTC deficiency: results from a Phase 1/2 clinical trial

- Time: 4:15-4:30 PM ET

Oral Presentation
Abstract #543: Characterization of phenotypic and genotypic stability of rAAV producing HeLa cell lines

- Time: 4:45-5:00 PM ET

Poster Presentation
Abstract #586: Gene therapy for Wilson Disease using rAAV to restore ATP7B gene function

- Time: 5:30-6:30 PM ET

Thursday, May 14
Poster Presentation
Abstract #1008: Elongation of the Rep-Cap cassette with a cellular intron reduces reverse-packaged Rep-Cap *trans* plasmid sequences and increases therapeutic vector genome packaging in a HEK293 triple transfection rAAV vector production system

- Time: 5:30-6:30 PM ET

Friday, May 15
Oral Presentation
Abstract #1306: AAV8-mediated liver-directed gene therapy as a potential therapeutic option in adults with glycogen storage disease type Ia (GSDIa): results from a Phase 1/2 clinical trial

- Time: 10:30-10:45 AM ET

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates aimed at addressing diseases with high unmet medical need and clear biology for treatment, 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 the Company's website at 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 regarding 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, the uncertainties inherent in the clinical drug development process, such as the regulatory approval process, the timing of regulatory filings and approvals (including whether such approvals can be obtained), the effects from the COVID-19 pandemic on clinical trial activities and other business operations, and other matters that could affect sufficiency of existing cash, cash equivalents and short-term investments to fund operations and the availability or commercial potential of our 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 Annual Report filed on Form 10-K with the Securities and Exchange Commission on February 14, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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