C-Path and Ultragenyx Announce Data Sharing Agreement to Support Rare Disease Treatment and Novel Therapies

TUCSON, Ariz., Dec. 1, 2022 — Critical Path Institute (C-Path) and Ultragenyx Pharmaceutical, Inc., (NASDAQ: RARE), a biopharmaceutical company focused on the development and commercialization of novel therapies for serious rare and ultra-rare genetic diseases, today announced a data sharing agreement to incorporate rare disease patient data into C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP®).

RDCA-DAP provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted for clinical development. Additionally, the platform advances best practices to support the rigorous conduct of natural history studies, with attention to established data quality standards, in order to be most useful to clinical trial design and regulatory review. It includes a robust, integrated database and analytics hub that allows for the aggregation of rare disease data from various sources and the efficient and effective interrogation of that data.

Ultragenyx will contribute GNE myopathy data from its completed disease monitoring program: A Registry and Prospective Observational Natural History Study to Assess Hereditary Inclusion Body Myopathy- (HIBM).

"We applaud Ultragenyx for sharing this data," said RDCA-DAP Scientific Director Alexandre Betourne, Pharm.D., Ph.D. "This is RDCA-DAP's second dataset for this disease, which will increase potential discoveries for GNE myopathy and will open opportunities in related disorders." RDCA-DAP continues to enrich its database including muscular and neuromuscular disorders with more than 66 datasets spanning 25 different diseases to date.

Ultragenyx and RDCA-DAP have a shared mission to accelerate the development of medical products and effective treatments for rare diseases — offering patients and families hope for the future.

"From the beginning of Ultragenyx, we have committed to sharing natural history data as the right approach to responsible drug development," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "We are pleased that a more formalized platform at C-Path will allow for more systematic access to all natural history data."

Groups interested in contributing data to RDCA-DAP may visit c-path.org/rdca-dap or email rdcadap@c-path.org. The platform is now OPEN and accepting applications for use; visit portal.rdca.c-path.org to apply and learn more.

About Ultragenyx Pharmaceutical Inc.
Ultragenyx is a biopharmaceutical company committed to bringing novel products to patients 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: ultragenyx.com.

Ultragenyx Forward-Looking Statements and Use of Digital Media
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 operations 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 the company's future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. 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 on November 3, 2022, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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 (ir.ultragenyx.com) and LinkedIn website (linkedin.com/company/ultragenyx-pharmaceutical-inc-/mycompany).

About Critical Path Institute
Critical Path Institute (C-Path) is an independent, nonprofit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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