Ultragenyx and Solid Biosciences Announce Strategic Collaboration to Develop and Commercialize New Gene Therapies for Duchenne Muscular Dystrophy

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-Collaboration combines Solid's differentiated microdystrophin construct and Ultragenyx's HeLa PCL manufacturing platform for use with AAV8 and variants-

-Solid receives $40 million upfront via equity investment at a premium; up to $255 million in milestones plus royalty payments-

-Solid retains exclusive rights to all other uses of its microdystrophins, including its existing SGT-001 program

Novato, Calif. and Cambridge, Mass. — October 23, 2020 —Ultragenyx Pharmaceutical Inc. (Nasdaq: RARE), a biopharmaceutical company focused on the development and commercialization of novel products for serious rare and ultra-rare diseases, and Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today announced a strategic collaboration and license agreement to focus on the development and commercialization of new gene therapies for Duchenne. The parties will collaborate to develop products that combine Solid's differentiated microdystrophin construct, Ultragenyx's HeLa producer cell line (PCL) manufacturing platform, and AAV8 variants. The collaboration also brings together Solid's expertise in muscle biology and Ultragenyx's expertise in bringing novel therapies to patients with rare diseases.

Under the terms of the collaboration, Solid granted Ultragenyx an exclusive license for any pharmaceutical product that expresses Solid's proprietary microdystrophin construct from AAV8 and variants thereof in clade E for use in the treatment of Duchenne and other diseases resulting from lack of functional dystrophin, including Becker muscular dystrophy. Ultragenyx has made a $40 million investment in Solid and has agreed to pay up to $255 million in cumulative milestone payments per product upon achievement of specified milestone events, and tiered royalties on worldwide net sales at low double digit to mid-teens percentages. Upon achievement of proof-of-concept, Solid has the right to opt-in to co-fund collaboration programs in return for participation in a profit share or increased royalty payments.

"We believe that Solid's microdystrophin is best-in-class with its unique neuronal nitric oxide synthase binding domain," said Emil D. Kakkis, MD, PhD, Chief Executive Officer and President of Ultragenyx. "By using an AAV8 variant validated in prior human and other studies combined with our scalable, efficient HeLa producer cell line platform, we believe we can leverage our mutual strengths to develop a high-quality AAV-based treatment alternative for Duchenne."

"Ultragenyx has a demonstrated track record of success in developing and commercializing innovative therapies for rare diseases," said Ilan Ganot, Co-Founder, President and Chief Executive Officer at Solid Biosciences. "We believe it is the partner of choice for exploring new gene therapy opportunities for patients with Duchenne."

Solid's proprietary microdystrophin construct has exhibited functional benefit in preclinical models. In preclinical studies, animals expressing a microdystrophin capable of restoring neuronal nitric oxide synthase (nNOS) resisted fatigue better than those expressing a microdystrophin that does not. Patients dosed with Solid's proprietary microdystrophin construct at the 2E14 vg/kg dose in Solid's ongoing IGNITE DMD clinical trial have also preliminarily demonstrated nNOS activity and function, further validating these preclinical results. Solid expects to dose the next patient in the IGNITE DMD clinical trial, using SGT-001 produced using its improved HSV manufacturing process, in the first quarter of 2021.

Ultragenyx intends to use its AAV-based HeLa PCL platform including HeLa 3.0 improvements for the development of product candidates. The platform enables large 2,000 liter commercial-scale AAV-based gene therapy product manufacturing. The PCL platform yields high-quality product from a highly reproducible, highly scalable, and less expensive process – a distinct vantage in higher dose indications like Duchenne. The capsid planned is an AAV8 variant with a favorable immunological profile that has been used successfully in the large scale 2,000 liter production process.

About Ultragenyx
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 and ensuring majority access to its therapies for patients who can benefit.

Ultragenyx currently has three AAV gene therapies in clinical development, including DTX201 that uses an AAV8 variant in the HeLa PCL platform and that is partnered with Bayer, who has released positive Phase 1/2 data in Hemophilia A. The company's other clinical AAV8 gene therapies, DTX301 and DTX401, are in Phase 1/2 studies for ornithine transcarbamylase (OTC) deficiency and glycogen storage disease type Ia (GSDIa), respectively. An investigational new drug (IND) application is expected by the end of 2020 for a fourth AAV gene therapy for Wilson disease, which will also use the HeLa PCL AAV manufacturing platform.

For more information on Ultragenyx, please visit the company's website at www.ultragenyx.com.

About Solid Biosciences
Solid Biosciences is a life sciences company focused on advancing transformative treatments to improve the lives of patients living with Duchenne. Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001.

Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne. Duchenne is caused by mutations in the dystrophin gene that result in the absence or near absence of dystrophin protein. SGT-001 is a systemically administered candidate that delivers a synthetic dystrophin gene, called microdystrophin, to the body. This microdystrophin encodes for a

functional protein surrogate that is expressed in muscles and stabilizes essential associated proteins, including nNOS. Data from Solid's preclinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, regardless of genetic mutation or disease stage.

SGT-001 is based on pioneering research in dystrophin biology by Dr. Jeffrey Chamberlain of the University of Washington and Dr. Dongsheng Duan of the University of Missouri. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and European Union.

For more information, please visit www.solidbio.com.

Ultragenyx 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, the effects from the COVID-19 pandemic on the company's clinical activities, business and operating results, uncertainty and potential delays related to clinical drug development, 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 on July 30, 2020, and its subsequent periodic reports filed with the Securities and Exchange Commission.

Solid Biosciences Forward-Looking Statements
This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding whether the collaboration will yield any viable product candidates, potential milestone payments or royalty payments in connection with the collaboration, the potential benefits of the collaboration, the safety or potential efficacy of SGT-001 and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," "working" and similar expressions. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with each party's ability to perform its obligations under the collaboration, the Company's ability to resume and/or continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approval from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll patients in IGNITE DMD; continue to advance SGT-001 in clinical trials; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop DMD/Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the Company's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views as of the date hereof and should not be relied upon as representing the Company's views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

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