UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): August 14, 2019

ULTRAGENYX PHARMACEUTICAL INC.

(Exact name of registrant as specified in charter)

Delaware	**001-36276**	**27-2546083**
(State or other jurisdiction of incorporation)	(Commission File Number)	(IRS Employer Identification No.)

60 Leveroni Court, Novato, California	**94949**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (415) 483-8800

Not Applicable
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol	Name of each exchange on which registered
Common Stock, $0.001 par value	**RARE**	**The Nasdaq Global Select Market**

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01 Regulation FD Disclosure

On August 14, 2019, Ultragenyx Pharmaceutical Inc. (the "***Company***") posted a presentation (the "***Presentation***") to its website at www.ultragenyx.com in the "*Events and Presentations*" subsection of the "Investors" tab.

The information set forth under Item 7.01 and in the Presentation shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, except as shall be expressly set forth by specific reference in any such filing.

Item 8.01 Other Events.

On August 14, 2019, the Company issued a press release announcing its partnership with GeneTx Biotherapeutics LLC ("***GeneTx***") to develop GeneTx's GTX-102, an antisense oligonucleotide for the treatment of Angelman syndrome (the "***Press Release***"). A copy of the Press Release is filed as Exhibit 99.1

Item 9.01 Financial Statements and Exhibits

(d) **Exhibits**

Exhibit No.	**Description**
99.1	Press Release, dated August 14, 2019.
104	The cover page from the Company's Current Report on Form 8-K dated August 14, 2019 formatted in Inline XBRL.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: August 14, 2019

Ultragenyx Pharmaceutical Inc.

By: /s/ Shalini Sharp
Shalini Sharp
Executive Vice President, Chief Financial Officer

Ultragenyx Announces Partnership with GeneTx to Advance Treatment for Angelman Syndrome

Program aims to be first disease-modifying treatment for this serious neurogenetic disorder

Ultragenyx receives an exclusive option to acquire GeneTx

Novato, Calif. and Downers Grove, Ill. — August 14, 2019 — Ultragenyx Pharmaceutical Inc. (NASDAQ: RARE), a biopharmaceutical company focused on the development of novel products for serious rare and ultra-rare diseases, and GeneTx Biotherapeutics LLC, today announced a partnership to develop GeneTx's GTX-102, an antisense oligonucleotide (ASO) for the treatment of Angelman syndrome, a serious, debilitating, rare neurogenetic disorder that affects approximately 1 in 15,000 people worldwide. GTX-102 is currently in late preclinical development with an investigational new drug (IND) application expected to be filed with the U.S. Food and Drug Administration (FDA) in the first half of 2020.

"Angelman syndrome is a devastating neurological condition with no treatment options that represents a very significant unmet medical need," said Emil D. Kakkis, M.D., Ph.D., Chief Executive Officer and President of Ultragenyx. "GTX-102 is a novel and promising potential therapy specific to the disease mechanism in these patients, and we are pleased that GeneTx chose to partner with us based on our track-record of rapid, innovative development in rare diseases."

"GeneTx was formed by the Foundation for Angelman Syndrome Therapeutics (FAST), the largest patient organization in the Angelman community. Donations to FAST, from family and friends, supported the preclinical research on this therapeutic strategy," stated Paula Evans, Chief Executive Officer of GeneTx. "It is entirely fitting that GeneTx would partner with a company that not only has proven expertise in the development and commercialization of rare disease therapeutics, but truly puts the patient first in each and every step of the treatment process."

Under the terms of the agreement, Ultragenyx will make an upfront payment of $20 million for an exclusive option to acquire GeneTx. This option may be exercised any time prior to 30 days following FDA acceptance of the IND for GTX-102. Ultragenyx may extend the option period by paying an option extension payment of $25 million. Ultragenyx may exercise this extended option any time until the earlier of 30 months from the first dosing of a patient in a planned Phase 1/2 study (subject to extensions) or 90 days after results are available from that study.

During the exclusive option period, GeneTx will provide regulatory and scientific expertise as well as fund all development activities, while Ultragenyx will provide staff support, including strategic guidance and clinical expertise. The parties will collaborate on the submission of the IND and management of the Phase 1/2 study in patients with Angelman syndrome. If Ultragenyx acquires GeneTx, Ultragenyx will then be responsible for all development and

commercialization activities. If Ultragenyx decides to exercise its option, it will purchase GeneTx for an initial purchase price and contingent milestones and royalties.

About Angelman Syndrome
Angelman syndrome is a rare, neurogenetic disorder caused by loss-of-function of the maternally inherited allele of the *UBE3A* gene. The maternal-specific inheritance pattern of Angelman syndrome is due to genomic imprinting of *UBE3A* in neurons of the central nervous system, a naturally occurring phenomenon in which the maternal *UBE3A* allele is expressed and the paternal *UBE3A* is not. Silencing of the paternal *UBE3A* allele is regulated by the *UBE3A* antisense transcript (*UBE3A-AS*), the target of GTX-102. In almost all cases of Angelman syndrome the maternal UBE3A allele is either missing or mutated, resulting in limited to no protein expression. This condition is typically not inherited but instead occurs spontaneously.

Individuals with Angelman syndrome have developmental delay, balance issues, motor impairment, and debilitating seizures. Some individuals with Angelman syndrome are unable to walk and most do not speak. Anxiety and disturbed sleep can be serious challenges in individuals with Angelman syndrome. While individuals with Angelman syndrome have a normal lifespan, they require continuous care and are unable to live independently. Angelman syndrome is not a degenerative disorder, but the loss of the UBE3A protein expression in neurons results in abnormal communications between neurons. Angelman syndrome is often misdiagnosed as autism or cerebral palsy. There are no currently approved therapies for Angelman syndrome; however, several symptoms of this disorder can be reversed in adult animal models of Angelman syndrome suggesting that improvement of symptoms can potentially be achieved at any age.

About GTX-102
GTX-102 is an investigational antisense oligonucleotide designed to target and inhibit expression *UBE3A-AS*. Studies show that GTX-102 reduces the levels of *UBE3A-AS* and reactivates expression of the paternal *UBE3A* allele in neurons of the CNS, and that reactivation of paternal *UBE3A* expression in animal models of Angelman syndrome improves some of the neurological symptoms associated with the condition. A *UBE3A-AS* targeted ASO has been granted orphan-drug designation from the U.S. FDA.

About GeneTx Biotherapeutics
GeneTx Biotherapeutics LLC is a startup biotechnology company singularly focused on developing and commercializing a safe and effective antisense therapeutic for the treatment of Angelman syndrome. GeneTx was launched by FAST, a patient advocacy organization and the largest non-governmental funder of Angelman syndrome research. GeneTx licensed the rights to antisense technology intellectual property from The Texas A&M University System in December 2017.

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.

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 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, including our partnership with GeneTx, 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), 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 Quarterly Report filed on Form 10-Q with the Securities and Exchange Commission on August 2 2019, and its subsequent periodic reports filed with the Securities and Exchange Commission.

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Contacts:
Ultragenyx
Investors & Media
Danielle Keatley
415-475-6876
dkeatley@ultragenyx.com

GeneTx

Paula Evans
630-639-7271
Paula.Evans@GeneTxBio.com