UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 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): December 6, 2021

Acadia Pharmaceuticals Inc.
(Exact name of Registrant as Specified in Its Charter)

Delaware	**000-50768**	**06-1376651**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS Employer Identification No.)**

12830 El Camino Real, Suite 400 **San Diego, California** **(Address of Principal Executive Offices)**	**92130** **(Zip Code)**

Registrant's Telephone Number, Including Area Code: (858) 558-2871

N/A
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instruction A.2. of Form 8-K):

☐ 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(s)	Name of each exchange on which registered
Common Stock, par value $0.0001 per share	**ACAD**	**The Nasdaq Stock Market LLC**

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 8.01 Other Events.

On December 6, 2021, the Company announced positive top-line results from Lavender™, its pivotal, Phase 3 study of trofinetide in Rett syndrome. Lavender™ was a 12-week, double-blind, randomized, placebo-controlled study evaluating the efficacy and safety of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome. The study demonstrated a statistically significant improvement over placebo for both co-primary endpoints. On the Rett Syndrome Behaviour Questionnaire (RSBQ), change from baseline to week 12 was -5.1 vs. -1.7 (p=0.0175; effect size = 0.37). The Clinical Global Impression–Improvement (CGI-I) score at week 12 was 3.5 vs. 3.8 (p=0.0030; effect size = 0.47). The RSBQ is a caregiver assessment of the core symptoms of Rett syndrome and the CGI-I is a global physician assessment of worsening or improving of Rett syndrome.

Additionally, trofinetide demonstrated a statistically significant separation over placebo on the key secondary endpoint, the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist–Social composite score (CSBS-DP-IT–Social) change from baseline to week 12 (-0.1 vs. -1.1; p=0.0064; effect size = 0.43).

Study treatment discontinuation rates related to treatment emergent adverse events (TEAEs) were 17.2% in the trofinetide group as compared to 2.1% in the placebo group. The most common adverse events were diarrhea (80.6% with trofinetide vs. 19.1% with placebo), of which 97.3% in the trofinetide arm were characterized as mild-to-moderate, and vomiting (26.9% with trofinetide vs. 9.6% with placebo), of which 96% in the trofinetide arm were characterized as mild-to-moderate. Serious adverse events were observed in 3.2% of study participants in both the trofinetide and placebo groups. Patients completing the Lavender study had the opportunity to continue to receive trofinetide in the open-label Lilac and Lilac-2 extension studies. More than 95% of participants who completed the Lavender study elected to roll-over to the Lilac open-label extension study.

Acadia is planning for a pre-NDA meeting with the U.S. Food and Drug Administration (FDA) in the first quarter of 2022 and plans to submit a new drug application (NDA) around mid-year 2022.

A copy of ACADIA's press release issued December 6, 2021 is furnished herewith as Exhibit 99.1.

Forward-Looking Statements

Statements in this Current Report that are not strictly historical in nature are forward-looking statements. These statements include, but are not limited to, statements related to: intended activities with respect to the Lavender study and the Company's planned engagement with the FDA. These statements are only predictions based on current information and expectations and involve a number of risks and uncertainties. Actual events or results may differ materially from those projected in any of such statements due to various factors, including the risks and uncertainties inherent in drug development, approval and commercialization, and the fact that past results of clinical trials may not be indicative of future trial results. For a discussion of these and other factors, please refer to the Company's annual report on Form 10-K for the year ended December 31, 2020 as well as its subsequent filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All forward-looking statements are qualified in their entirety by this cautionary statement and the Company undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit Number	Description
99.1	Press Release dated December 6, 2021
104	Cover Page Interactive Data File (embedded within the Inline XBRL document).

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.

Acadia Pharmaceuticals Inc.

Dated: December 6, 2021

By: /s/ Austin D. Kim
Austin D. Kim
Executive Vice President, General Counsel & Secretary

Acadia Pharmaceuticals Announces Positive Top-line Results from the Pivotal Phase 3 Lavender Trial of Trofinetide in Rett Syndrome

- Trofinetide met co-primary efficacy endpoints demonstrating statistically significant improvement over placebo in the Rett Syndrome Behaviour Questionnaire (RSBQ) (p=0.0175) and the Clinical Global Impression of Improvement (CGI-I) (p=0.0030)

- Trofinetide met key secondary endpoint demonstrating statistically significant improvement over placebo in CSBS-DP-IT–Social (p=0.0064), caregiver scale of ability to communicate

- Pre-New Drug Application meeting with the U.S. FDA planned for the first quarter 2022

- Conference call and webcast to be held today at 4:30 p.m. Eastern Time

SAN DIEGO — December 6, 2021 – Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) today announced positive top-line results from the pivotal, Phase 3 Lavender™ study evaluating the efficacy and safety of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome. The 12-week placebo-controlled study demonstrated a statistically significant improvement over placebo for both co-primary endpoints. On the Rett Syndrome Behaviour Questionnaire (RSBQ), change from baseline to week 12 was -5.1 vs. -1.7 (p=0.0175; effect size=0.37). The Clinical Global Impression–Improvement (CGI-I) score at week 12 was 3.5 vs. 3.8 (p=0.0030; effect size=0.47). The RSBQ is a caregiver assessment of the core symptoms of Rett syndrome and the CGI-I is a global physician assessment of worsening or improving of Rett syndrome.

Additionally, trofinetide demonstrated a statistically significant separation over placebo on the key secondary endpoint, the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist–Social composite score (CSBS-DP-IT–Social) change from baseline to week 12 was -0.1 vs. -1.1 (p=0.0064; effect size=0.43).

“These are encouraging results for patients and families affected by Rett syndrome. Patients reported improvements in core symptoms, like being able to respond to a choice when asked by their parents, or experiencing more freedom from the repetitive hand movements that create obstacles in other areas of their lives,” said Jeffrey L. Neul, M.D., Ph.D., Annette Schaffer Eskind Chair and Director, Vanderbilt Kennedy Center; Professor of Pediatrics, Division of Neurology, Pharmacology, and Special Education, Vanderbilt University Medical Center and Lavender study investigator. “The positive Lavender study results support a potential treatment for Rett syndrome and represent an important step forward in addressing this rare and serious neurological disease.”

Study treatment discontinuation rates related to treatment emergent adverse events (TEAEs) were 17.2% in the trofinetide group as compared to 2.1% in the placebo group. The most common adverse events were diarrhea (80.6% with trofinetide vs. 19.1% with placebo), of which 97.3% in the trofinetide arm were characterized as mild-to-moderate, and vomiting (26.9% with trofinetide vs. 9.6% with placebo), of which 96% in the trofinetide arm were characterized as mild-to-moderate.

Serious adverse events were observed in 3.2% of study participants in both the trofinetide and placebo groups. Patients completing the Lavender study had the opportunity to continue to receive trofinetide in the open-label Lilac and Lilac-2 extension studies. More than 95% of participants who completed the Lavender study elected to roll-over to the Lilac open-label extension study.

The results from this study will be submitted for presentation at upcoming medical meetings.

“The consistent efficacy across primary and key secondary endpoints in the Lavender study demonstrates the potential of trofinetide to treat Rett syndrome,” said Kathie Bishop, Ph.D., Acadia’s Senior Vice President, Chief Scientific Officer and Head of Rare Disease. “We want to thank the patients, their caregivers, study site personnel, physicians and everyone who participated in the Lavender study for their contribution to making this milestone a reality. We look forward to continuing this important work and potentially delivering an FDA-approved treatment for this rare and devastating disease.”

Acadia is preparing for a pre-NDA meeting with the U.S. Food and Drug Administration (FDA) in the first quarter of 2022 and plans to submit a New Drug Application (NDA) around mid-year 2022. Trofinetide has been granted Fast Track Status and Orphan Drug Designation for Rett syndrome. Trofinetide has also been granted Rare Pediatric Disease (RPD) designation by the FDA. An NDA with Orphan Drug Designation is eligible for priority review. With an RPD NDA we would expect to be awarded a Priority Review Voucher if approved, subject to final determination by the FDA.

In 2018, Acadia entered into an exclusive license agreement with Neuren Pharmaceuticals Limited (ASX: NEU) for the development and commercialization of trofinetide for Rett syndrome and other indications in North America.

Conference Call and Webcast Information

Acadia will discuss top-line results from its Lavender study of trofinetide for the treatment of Rett syndrome via conference call and webcast today at 4:30 p.m. Eastern Time. The conference call can be accessed by dialing 855-638-4820 for participants in the U.S. or Canada and 443-877-4067 for international callers (reference passcode 7989366). A telephone replay of the conference call may be accessed through December 20, 2021 by dialing 855-859-2056 for callers in the U.S. or Canada and 404-537-3406 for international callers (reference passcode 7989366). The conference call will also be webcast live on Acadia’s website, www.acadia-pharm.com, in the investors section and archived until January 3, 2022.

About Lavender™

The Lavender study was a Phase 3, 12-week, double-blind, randomized, placebo-controlled study of trofinetide in 187 girls and young women aged 5-20 years with Rett syndrome, designed to evaluate its efficacy and safety. The co-primary endpoints of Lavender included both a caregiver (Rett Syndrome Behaviour Questionnaire [RSBQ]) and physician (Clinical Global Impression–Improvement [CGI-I]) assessment. The key secondary endpoint was also a caregiver assessment designed to evaluate communication skills, the Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist – Social Composite Score (CSBS-DP- IT–Social).

About Rett Syndrome

Rett syndrome is a rare, debilitating neurological disorder that occurs primarily in females following apparently normal development for the first six months of life. Rett syndrome is often misdiagnosed as autism, cerebral palsy, or non-specific developmental delay. Rett syndrome is caused by mutations on the X chromosome on a gene called *MECP2*. There are more than 200 different mutations found on the *MECP2* gene that interfere with its ability to generate a normal gene product.

Rett syndrome occurs worldwide in approximately one of every 10,000 to 15,000 female births and in the United States impacts 6,000 to 9,000 patients. Rett syndrome causes problems in brain function that are responsible for cognitive, sensory, emotional, motor and autonomic function. Typically, with symptoms presenting between six to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use (fine motor skills), development of hand stereotypies, absent or impaired mobility (gross motor skills), loss of communication skills (including eye contact) and inability to independently conduct activities of daily living. Symptoms also include seizures, disorganized breathing patterns, an abnormal side-to-side curvature of the spine (scoliosis), and sleep disturbances. Currently, there are no FDA-approved medicines for the treatment of Rett syndrome.

About Trofinetide

Trofinetide is an investigational drug. It is a novel synthetic analog of the amino-terminal tripeptide of IGF-1 designed to treat the core symptoms of Rett syndrome by potentially reducing neuroinflammation and supporting synaptic function. Trofinetide is thought to stimulate synaptic maturation and overcome the synaptic and neuronal immaturities that are characteristic of Rett syndrome pathophysiology. In the central nervous system, IGF-1 is produced by both of the major types of brain cells – neurons and glia. IGF-1 in the brain is critical for both normal development and for response to injury and disease. Trofinetide has been shown to inhibit the production of inflammatory cytokines, inhibit the overactivation of microglia and astrocytes, and increase the amount of available IGF-1 that can bind to IGF-1 receptors.

Trofinetide has been granted Fast Track Status and Orphan Drug Designation for Rett syndrome. Trofinetide has also been granted Rare Pediatric Disease (RPD) designation by the FDA. Upon FDA approval of a product with RPD designation, the sponsor can receive a Priority Review Voucher, which can be used to obtain FDA review of a New Drug Application for another product in an expedited period of six months.

About Acadia Pharmaceuticals

Acadia is trailblazing breakthroughs in neuroscience to elevate life. For more than 25 years we have been working at the forefront of healthcare to bring vital solutions to people who need them most. We developed and commercialized the first and only approved therapy for hallucinations and delusions associated with Parkinson’s disease psychosis. Our late-stage development efforts are focused on dementia-related psychosis, negative symptoms of schizophrenia and Rett syndrome, and in early-stage clinical research we are exploring novel approaches to pain management, and cognition and neuropsychiatric symptoms in central nervous system disorders. For more information, visit us at www.acadia-pharm.com and follow us on LinkedIn and Twitter.

Forward-Looking Statements

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