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): February 21, 2024

CATALYST PHARMACEUTICALS, INC.
(Exact Name Of Registrant As Specified In Its Charter)

Delaware	**001-33057**	**76-0837053**
(State or other jurisdiction of incorporation)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

355 Alhambra Circle **Suite 801** **Coral Gables, Florida**	**33134**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (305) 420-3200

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 CFR240.14d-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	Name of Exchange on Which Registered	Ticker Symbol
Common Stock, par value $0.001 per share	NASDAQ Capital Market	CPRX

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 February 21, 2024, the Company issued a press release announcing that the peer-reviewed journal Neurology has published Santhera Pharmaceutical's ("Santhera") study titled "Efficacy and Safety of Vamorolone Over 48 Weeks in Boys With Duchenne Muscular Dystrophy" presenting the findings of Santhera's VISION-DMD study conducted by Santhera. The Company holds the exclusive rights to commercialize AGAMREE® (vamorolone) in North America.

A copy of the press release is attached hereto as Exhibit 99.1.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

99.1	Press release issued by the Company on February 21, 2024.
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 caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Catalyst Pharmaceuticals, Inc.

By: /s/ Michael W. Kalb
Michael W. Kalb
Executive Vice President and Chief Financial Officer

Dated: February 21, 2024

Catalyst Pharmaceuticals Announces Publication of Santhera Pharmaceutical's VISION-DMD Vamorolone (AGAMREE®) Study Results in the Peer-Reviewed Journal *Neurology*

Santhera's Study Reports the Results of the 48-Week Treatment with Vamorolone in Patients with Duchenne Muscular Dystrophy (DMD) in the VISION-DMD Study, Supporting the Long-Term Efficacy and Safety Profile of Vamorolone

CORAL GABLES, Fla., February 21, 2024 — Catalyst Pharmaceuticals, Inc. ("Catalyst" or "Company") (Nasdaq: CPRX), a commercial-stage, patient-centric biopharmaceutical company focused on in-licensing, developing, and commercializing novel high-quality medicines for patients living with rare and difficult to treat diseases, today announced that the peer-reviewed journal *Neurology* has published Santhera Pharmaceutical's ("Santhera") study titled "*Efficacy and Safety of Vamorolone Over 48 Weeks in Boys With Duchenne Muscular Dystrophy*" presenting the findings from Santhera's VISION-DMD study conducted by Santhera [1]. Catalyst holds the exclusive rights to commercialize AGAMREE® (vamorolone) in North America.

Santhera reports that the VISION-DMD study was a randomized, double-blind, placebo-controlled, and prednisone-controlled clinical trial of 2 doses of vamorolone in participants with Duchenne muscular dystrophy ("DMD") in the ages four years to younger than seven years at baseline that was conducted at 33 sites in 11 countries from 2018 to 2021. Based on the published study, Santhera has reported that the results of the VISION-DMD study support the long-term efficacy and safety profile of vamorolone and conclude that vamorolone was generally well tolerated, consistent with the 24-week study findings, as published previously in *JAMA Neurology* [2] As cited in Santhera's recent press release, the *Neurology* publication of their study states:

> "Vamorolone is a dissociative corticosteroid that selectively binds to the glucocorticoid receptor and has shown similar efficacy and reduced safety concerns in comparison with prednisone in Duchenne muscular dystrophy (DMD)[2]. This study [VISION-DMD] was conducted to determine the efficacy and safety of vamorolone over 48 weeks and to study crossover participants (prednisone to vamorolone; placebo to vamorolone).
>
> A total of 121 participants with DMD were randomized. Vamorolone at a dose of 6 mg/kg/d showed maintenance of improvement for all motor outcomes to week 48 (e.g., for primary outcome, time to stand from supine [TTSTAND] velocity, week 24 least squares mean [LSM] [SE] 0.052 [0.0130] rises/s vs week 48 LSM [SE] 0.0446 [0.0138]). After 48 weeks, vamorolone at a dose of 2 mg/kg/d showed similar improvements as 6 mg/kg/d for North Star Ambulatory Assessment (NSAA) (vamorolone 6 mg/kg/d– vamorolone 2 mg/kg/d LSM [SE] 0.49 [1.14]; 95% CI -1.80 to 2.78, p = 0.67), but less improvement for other motor outcomes. The placebo to vamorolone 6 mg/kg/d group showed rapid improvements after 20 weeks of treatment approaching benefit seen with 48-week 6 mg/kg/d of vamorolone treatment for TTSTAND, time to run/walk 10 m, and NSAA. There was significant improvement in linear growth after crossover in the prednisone to vamorolone 6 mg/kg/d group, and rapid reversal of prednisone-induced decline in bone turnover biomarkers in both crossover groups. There was an increase in BMI after 24 weeks of treatment that then stabilized for both vamorolone groups.

Improvements of motor outcomes seen with 6 mg/kg/d of vamorolone at 24 weeks of treatment were maintained to 48 weeks of treatment. Vamorolone at a dose of 6 mg/kg/d showed better maintenance of effect compared with vamorolone at a dose of 2 mg/kg/d for most (3/5) motor outcomes. Bone morbidities of prednisone (stunting of growth and declines in serum bone biomarkers) were reversed when treatment transitioned to vamorolone."

AGAMREE® (vamorolone) oral suspension 40 mg/ml has been approved for commercialization in the United States for the treatment of DMD.

About AGAMREE® (vamorolone)

AGAMREE's unique mode of action is based on differential effects on glucocorticoid and mineralocorticoid receptors and modifying further downstream activity. As such, it is considered a novel corticosteroid with dissociative properties in maintaining efficacy that we hope has the potential to demonstrate comparable efficacy to steroid, with the potential for a better-tolerated side effect profile. This mechanism of action may allow vamorolone to emerge as an effective alternative to the current standard of care corticosteroids in children, adolescents, and adult patients with DMD. In the pivotal VISION-DMD study, vamorolone met the primary endpoint Time to Stand (TTSTAND) velocity versus placebo (p=0.002) at 24 weeks of treatment and showed a good safety and tolerability profile. The most commonly reported adverse events versus placebo from the VISION-DMD study were cushingoid features, vomiting, and vitamin D deficiency. Adverse events were generally of mild to moderate severity.

AGAMREE was granted U.S. FDA approval on October 26, 2023, and was granted Orphan Drug and Rare Pediatric Disease designation status for DMD in the U.S., making it eligible for seven years of orphan drug exclusivity upon approval. AGAMREE also has issued and pending patents that could provide protection until 2040. In Europe, it has received Promising Innovative Medicine (PIM) status from the UK MHRA for DMD.

References:

[1] Dang UJ et al. (2024) Neurology 2024;102:e208112. doi.org/10.1212/WNL.0000000000208112. Link.
[2] Guglieri M et al (2022). JAMA Neurol. 2022;79(10):1005-1014. doi:10.1001/jamaneurol.2022.2480. Link.
[3] Liu X et al. (2020). Proc Natl Acad Sci USA 117:24285-24293
[4] Heier CR et al (2019). Life Science Alliance DOI: 10.26508
[5] Ward et al., WMS 2022, FP.27 - Poster 71. Link.
[6] Hasham et al., MDA 2022 Poster presentation. Link.
[7] Applicable drug labeling: Summary of Product Characteristics (SmPC). English. German.

About Duchenne muscular dystrophy (DMD)

DMD, the most common form of muscular dystrophy, is a rare and life-threatening neuromuscular disorder characterized by progressive muscle dysfunction, ultimately leading to loss of ambulation, respiratory failure, and fatality. Current standard treatment for DMD involves corticosteroids, which often come with significant side effects. It is estimated that between 11,000 and 13,000 patients in the U.S. are affected by DMD, with approximately 70% of patients currently receiving concomitant corticosteroid treatment.

About Catalyst Pharmaceuticals

With exceptional patient focus, Catalyst is committed to developing and commercializing innovative first-in-class medicines that address rare and difficult to treat diseases. Catalyst's flagship U.S. commercial product is FIRDAPSE® (amifampridine) Tablets 10 mg, approved for the treatment of Lambert-Eaton myasthenic syndrome ("LEMS") for adults and for children ages six to seventeen. In January 2023, Catalyst acquired the U.S. commercial rights to FYCOMPA® (perampanel) CIII, a prescription medicine approved in people with epilepsy aged four and older alone or with other medicines to treat partial-onset seizures with or without secondarily generalized seizures and with other medicines to treat primary generalized tonic-clonic seizures for people with epilepsy aged 12 and older. Further, Canada's national healthcare regulatory agency, Health Canada, has approved the use of FIRDAPSE for the treatment of adult patients in Canada with LEMS. Finally, on July 18, 2023, Catalyst acquired an exclusive license for North America for AGAMREE® (vamorolone) oral suspension 40 mg/mL, a novel corticosteroid treatment for Duchenne Muscular Dystrophy. AGAMREE® previously received FDA Orphan Drug and Fast Track designations and was approved for commercialization in the U.S. on October 26, 2023.

For more information about Catalyst Pharmaceuticals, Inc., please visit the Company's website at www.catalystpharma.com. For Full Prescribing and Safety Information for FIRDAPSE®, please visit www.firdapse.com. For Full Prescribing Information, including Boxed WARNING for FYCOMPA®, please visit www.fycompa.com. For Full Prescribing Information for AGAMREE®, please visit www.agamree.com.

Forward-Looking Statements

This press release contains forward-looking statements, as that term is defined in the Private Securities Litigation Reform Act of 1995. These include statements regarding Catalyst's expectations, beliefs, plans, or objectives regarding the intended use of net proceeds therefrom. Forward-looking statements involve known and unknown risks and uncertainties, which may cause Catalyst's actual results in future periods to differ materially from forecasted results. A number of factors, including (i) whether AGAMREE will be found to have a better safety profile than other corticosteroids, (ii) whether Catalyst's commercial launch of AGAMREE in the United States will be successful, and (iii) those factors described in Catalyst's Annual Report on Form 10-K for the fiscal year 2022 and its other filings with the U.S. Securities and Exchange Commission ("SEC"), could adversely affect Catalyst. Copies of Catalyst's filings with the SEC are available from the SEC, may be found on Catalyst's website, or may be obtained upon request from Catalyst. Catalyst does not undertake any obligation to update the information contained herein, which speaks only as of this date.
Source: Catalyst Pharmaceuticals, Inc.

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