# 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):		orted):	September 29, 2014	
		RMACEUTICAL PA	•	
	Delaware	001-33057	76-0837053	
(	State or other jurisdiction of incorporation)	(Commission File Number)	(I.R.S. Employer Identification No.)	
	355 Alhambra Circle Suite 1500 Coral Gables, Florida		33134	
(Address of principal executive offices)		<del>s)</del>	(Zip Code)	
	Registrant's telephone number, including ar	ea code:	(305) 529-2522	
	Former Na	<u>Not Applicable</u> ame or Former address, if changed since last	report	
	ck the appropriate box below if the Form 8-K filing is invisions:	ntended to simultaneously satisfy the filing	obligation of the registrant under any of the following	
	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))			

# **Item 8.01** Other Events

On September 29, 2014, the Company issued a press release announcing the positive top-line results from its pivotal Phase 3 clinical trial of Firdapse<sup>TM</sup> (amifampridine phosphate tablets equivalent to 10 mg amifampridine) for symptomatic treatment of Lambert-Eaton myasthenic syndrome.

A copy of the Company's press release is attached as Exhibit 99.1 to this Form 8-K and is incorporated herein by reference.

# Item 9.01 Financial Statements and Exhibits.

- (d) Exhibits
- 99.1 Press release issued by the Company on September 29, 2014.

# **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 Pharmaceutical Partners, Inc.

By: /s/ Alicia Grande

Alicia Grande

Vice President, Treasurer and CFO

Dated: September 29, 2014



# Catalyst Pharmaceuticals Announces Positive Top-Line Phase 3 Data from Pivotal Firdapse Clinical Trial in Patients with Lambert-Eaton Myasthenic Syndrome (LEMS)

Treatment with Firdapse achieved statistical significance for both co-primary clinical endpoints

Catalyst expects to initiate rolling NDA submission in early-2015

Conference call tomorrow, September 30 at 8:30am EDT

CORAL GABLES, Fla., Sept. 29, 2014 — Catalyst Pharmaceutical Partners, Inc. (Nasdaq:CPRX), (Catalyst Pharmaceuticals), a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, today announced positive top-line results from the pivotal Phase 3 clinical trial of Firdapse<sup>TM</sup> (amifampridine phosphate tablets equivalent to 10 mg amifampridine) for the symptomatic treatment of Lambert-Eaton myasthenic syndrome (LEMS). Both co-primary endpoints, quantitative myasthenia gravis score (QMG) and subject global impression (SGI), demonstrated that Firdapse was significantly superior to placebo, as did a secondary endpoint for the physician's clinical global impression of improvement (CGI-I).

This clinical trial was designed as a double blind, randomized, "withdrawal trial" in which all patients were initially treated with Firdapse during a 91 day run-in period followed by treatment with either Firdapse or placebo (randomly assigned, about 1:1) during a 2 week randomization period. A total of 38 patients completed the 3 month run-in period and subsequent 2 week randomization period. In a trial of this design, the clinically significant findings, when present, are worsening of symptoms in the placebo group.

# **Summary of Clinical Trial Results for Firdapse**

- · Primary endpoints
  - o The primary endpoint of change in quantitative myasthenia gravis score, or QMG, at day 14 reached statistical significance (p=0.0452) with a clinically significant worsening of 2.2 points observed in the placebo group.
  - o The primary endpoint of change in subject global impression, or SGI, at day 14 was highly statistically significant (p=0.0028) with a clinically significant worsening of 2.6 points observed in the placebo group.

- Secondary endpoints
  - o The secondary endpoint for the physician's clinical global impression of improvement, or CGI-I, reached statistical significance (p=0.0267) with a clinically significant observation at day 14 of 4.7 points in the placebo group.
  - o The secondary endpoint of change in walking speed at day 14 showed a worsening of 9.67 ft/min in the placebo group. As expected, this was a quantitative worsening in walking speed in the placebo group, but the magnitude of the change relative to the variance inherent in this test prevented reaching statistical significance for this endpoint with this small sample size.
- · Patient tolerance of Firdapse
  - o Firdapse was generally safe and well tolerated.
  - o All subjects who were randomized into the trial elected to continue with Firdapse in the safety follow-up phase of the study.

Patrick J. McEnany, Chief Executive Officer of Catalyst Pharmaceuticals, stated, "Today is an important day for LEMS patients and their families as the results from this Phase 3 trial were very positive." Mr. McEnany continued, "These results show that Firdapse provides an important benefit to LEMS patients and continues to demonstrate a favorable safety profile. We plan to meet with the FDA in the near future to determine the fastest way to get an NDA for Firdapse approved. We are committed to the LEMS patient community and are pleased that we will be launching our expanded access program next month. This program will provide Firdapse at no charge to patients who meet the inclusion/exclusion requirements."

Natacha T. Pires, MBBS, director of medical and public affairs at The Neuropathy Association, stated, "Having well-controlled Phase 3 clinical trials and ultimately an FDA-approved medication is an important step forward for all LEMS patients. We are pleased to see companies like Catalyst investing in the development of therapies for patients with these rare muscular degenerative diseases."

Shin J. Oh, MD, Distinguished Professor Emeritus of Neurology, University of Alabama at Birmingham, School of Medicine, stated, "I am extremely pleased to see the results from the first ever Phase 3 controlled study in this LEMS patient population, which confirmed a positive treatment effect for Firdapse. When combined with the favorable safety profile, the results indicate that the drug should become the standard of care in treating LEMS patients once approved by the FDA."

#### **About the Clinical Endpoints**

The protocol for this study specified 2 co-primary endpoints, QMG score and SGI. The QMG score is a physician-rated evaluation consisting of 13 assessments (each rated 0 to 3, which are totaled to obtain a QMG score) including tests for arm strength, leg strength, face and neck muscle performance, swallowing, grip strength, forced respiration, and gaze impairment. The SGI score is a subjective scale on which the patient rates their satisfaction with the effects of Firdapse or placebo on their LEMS symptoms with scores from 1 ("Terrible") to 7 ("Delighted").

Catalyst's clinical trial also included two secondary clinical endpoints for the physician rated clinical global impression of improvement (CGI-I) score and an evaluation of the time needed to walk 25 feet (timed 25 foot walk test). The CGI-I score captures the investigator's overall impression of improvement or worsening of the patient's symptoms when treated with the Firdapse. This 7-point scale is subjectively scored by the investigator based on changes in symptoms, behavior, and functional abilities, with scores ranging from 1="Very much improved" to 7="Very much worse". The timed 25 foot walk test is conducted in duplicate with a short resting period between each test. The time to cover 25 feet is recorded and converted to walking speed for analysis.

All endpoints were evaluated as a change in the respective evaluation between day 1 and day 14 of the randomization phase of the trial. The change in QMG, SGI, and CGI reached statistical significance and the change observed in the placebo group was clinically significant for all three of these endpoints.

# **Upcoming Scientific Presentation**

Catalyst will be presenting more details on the results of the trial on October 12th in an oral and poster presentation (Poster # S737WIP) at the 139th Annual Meeting of the American Neurological Association, to be held at the Baltimore Waterfront Marriott.

#### **Conference Call**

Catalyst Pharmaceuticals will host a conference call tomorrow, September 30, 2014 at 8:30 am ET to discuss positive top-line results from the Firdapse clinical trial. Interested participants and investors may access the conference call at 8:30 am ET by dialing 877-407-8912 (U.S./Canada) or 201-689-8059 (international).

An audio webcast can also be accessed via the Investors section of the Catalyst Pharmaceuticals corporate web site at <a href="http://ir.catalystpharma.com/events.cfm">http://ir.catalystpharma.com/events.cfm</a>, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 11:00 a.m. ET tomorrow. Access numbers for this replay are 877-660-6853(U.S./Canada) and 201-612-7415 (international); participant code 13591688.

#### **About Firdapse**

Firdapse, amifampridine phosphate or 3,4-diaminopyridine (3,4-DAP) phosphate, is a potassium channel inhibitor. By blocking this ion channel, Firdapse: 1) increases the nerve repolarization time, 2) causing an increase in the influx of calcium, 3) increasing acetylcholine release, and 4) restoring muscle fiber contraction, thus relieving muscle weakness caused by LEMS. In addition to LEMS, other potential orphan neuromuscular indications for Firdapse include certain types of Myasthenia Gravis and Congenital Myasthenic Syndrome, among others.

Firdapse has been granted breakthrough therapy and orphan drug designations by the FDA and the Company intends to request a pre-NDA meeting with the FDA in the near future. The Company plans to initiate a rolling NDA submission in early-2015.

#### About the Phase 3 Trial of Firdapse

The Firdapse Phase 3 trial utilized a randomized, double-blind, placebo-controlled, discontinuation design. The trial was conducted at sites in the United States and Europe. Following enrollment, patients were treated with open label drug for a minimum of 91 days, and then randomized to either continue on Firdapse or be discontinued to placebo over a 2-week period. Following the randomization phase of the trial, patients were then eligible to receive open label Firdapse treatment for a two-year follow-up period to obtain additional long term safety data.

The primary endpoints of the Phase 3 trial were a comparison in patients randomized to continue Firdapse versus those who transitioned to placebo of changes that occur in both the QMG score, which measures muscle strength, and subject global impression score, on which the subject rates their global impression of the effects of a study treatment during a 14-day double-blind efficacy evaluation period. The secondary endpoints were change in the investigator's assessment (CGI-I) of worsening of disease symptoms and changes in walking speed (Timed 25-foot walking test) during the two-week, double-blind testing period. Further details regarding the Phase 3 trial and its design can be found on <a href="https://www.clinicaltrials.gov">www.clinicaltrials.gov</a> (NCT01377922).

### **About Catalyst Pharmaceuticals**

Catalyst Pharmaceuticals is a biopharmaceutical company focused on developing and commercializing innovative therapies for people with rare debilitating diseases, including Lambert-Eaton Myasthenic Syndrome (LEMS), infantile spasms, and Tourette Syndrome. Catalyst's lead candidate, Firdapse for the treatment of LEMS, recently completed testing in a global, multi-center, pivotal Phase 3 trial and has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA). Firdapse is the first and only European approved drug for symptomatic treatment in adults with LEMS.

Catalyst is also developing a potentially safer and more potent vigabatrin analog (designated CPP-115) to treat infantile spasms, and epilepsy, as well as other neurological conditions associated with reduced GABAergic signaling, like post-traumatic stress disorder and Tourette Syndrome. CPP-115 has been granted U.S. orphan drug designation for the treatment of infantile spasms by the FDA and has been granted E.U. orphan medicinal product designation for the treatment of West Syndrome by the European Commission.

#### Forward-Looking Statements

This press release contains forward-looking statements. 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 whether the receipt of breakthrough therapy designation for Firdapse will expedite the development and review of Firdapse by the FDA or the likelihood that the product will be found to be safe and effective, whether an NDA for Firdapse will ever be accepted for filing by the FDA, the timing of any such NDA filing or acceptance, whether Catalyst will be the first company to receive an approval for amifampridine (3,4-DAP), giving it 7-year marketing exclusivity for its product, whether any of Catalyst's product candidates will ever be approved for commercialization or successfully commercialized, and those other factors described in Catalyst's Annual Report on Form 10-K for the fiscal year 2013 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.

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