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February 4, 2019

Patrick J. McEnany
President and Chief Executive Officer
Catalyst Pharmaceuticals, Inc.
355 Alhambra Circle, Suite 1250
Coral Gables, FL 33134

Dear Mr. McEnany:

I am writing to your company to request information about the staggering list price you have set for Firdapse, a drug to treat a rare neuromuscular disease called Lambert-Eaton myasthenic syndrome (LEMS). On the Catalyst Pharmaceuticals December 13, 2018 call with investors, Catalyst announced its intention to set the Firdapse annual list price at $375,000.[1] Until now, patients have been able to access an unapproved version of this drug for free through a Food and Drug Administration compassionate use program.

Catalyst did not invent the active ingredient in this drug, called 3,4-diaminopyridine (3,4-DAP). In fact, 3,4-DAP has been used to treat LEMS and related conditions for more than thirty years. When Catalyst acquired the North American license for Firdapse, it knew the clinical effectiveness had been established over decades in Europe and the United States.[2]

Catalyst's decision to set the annual list price at $375,000 is not only a blatant fleecing of American taxpayers, but is also an immoral exploitation of patients who need this medication. Simply put, it is corporate greed. The cost of the formulation ranges from estimates of $1,600 to $6,000 per year.[3,4] I am profoundly concerned that Catalyst's actions will cause patients to suffer or die.

The fear that neuromuscular doctors around the country predicted in 2015 – the concern "for the possibility of a potentially harmful price increase of 3,4-DAP in the United States (US) in the near future" – has indeed come to pass.[5] You responded to that letter in November 2016 and said that "[s]peculative issues of pricing and perceived use of loopholes divert focus away from the

[1] Catalyst Pharmaceuticals, Inc., Securities and Exchange Commission, Form 8-K (Dec. 13, 2018), (online at https://www.sec.gov/Archives/edgar/data/1369568/000119312518350053/d677721dex991.htm).
[2] Donald B. Sanders et al., "A randomized trial of 3,4-diaminopyridine in Lambert-Eaton myasthenic syndrome," *Neurology* (2000).
[3] Ted M. Burns and Gordon Smith et al., "Editorial by concerned physicians: Unintended effect of the Orphan Drug Act on the potential cost of 3,4-diaminopyridine," *Muscle & Nerve* (Dec. 2015).
[4] Meghana Keshavan, "A drug that was once inexpensive gets a new price tag: $375,000," *STAT* (Dec. 13, 2018), (online at https://www.statnews.com/2018/12/13/catalyst-pharma-drug-price).
[5] *See* note 3.

main issue at hand: the problem of access."[6] This is no longer a speculative issue of pricing – this is a real issue, and it is an access issue. By setting such a high price and forcing production and distribution of the older, inexpensive version to cease, you are threatening access that patients had to a cheap version of this product, and handing a completely unwarranted bill to American taxpayers.

To evaluate the impact on our constituents of the price you are setting, we request that you provide the following documents and information for the time period covering January 1, 2012, to the present:

1. Please describe the financial and non-financial factors that contributed to your company's decision to acquire the licensing rights to this drug and set the list price at $375,000.

2. How many patients will suffer or die due to Catalyst's decision to set the annual list price of Firdapse at $375,000?

3. How much did Catalyst pay BioMarin Pharmaceuticals or other third parties for the North American licensing rights to amifampridine? Please list cash payments as well as payments or commitment of future payment of any kind, including stocks, royalties, and milestone payments to BioMarin as well as former Huxley Pharmaceuticals shareholders, and other third-party licensors.

4. Please provide the actual or estimated price paid for Firdapse and anticipated total spending listed by payer. Please include commercial payers and Medicare, Medicaid, the Department of Defense, Federal Bureau of Prisons and the Department of Veterans Affairs.

5. How much is Catalyst Pharmaceuticals paying to purchase or produce Firdapse's active pharmaceutical ingredient and all inactive ingredients?

6. Please provide information on Catalyst's expanded access program prior to the approval of Firdapse, including the number of patients served and total expenditures for medication per patient per year, as well as the number of patients Catalyst plans to serve through this program over the next seven years. Please include all income, asset, and insurance eligibility requirements.

7. Please identify the name of the lead official at the "highly regarded and recognized consulting firm" that Catalyst hired to conduct a "rigorous pricing and access study."[7]

8. What is the price of this drug in all foreign countries or markets?

9. Your website states that Catalyst plans to expand the uses of Firdapse to treat additional diseases. Please provide the status of all clinical trials, clinicaltrials.gov registration

[6] Patrick J. McEnany, "A response to a recent editorial by concerned physicians on 3,4-Diaminopyridine," *Muscle & Nerve* (Nov. 2016).
[7] *See* note 1.

numbers, and the name of the lead clinical investigator of each of the trials. How many additional years of exclusivity does Catalyst expect to be awarded if Firdapse is approved for each of these uses? How much revenue do you project to earn during these additional years of exclusivity?

Patients are currently attempting to ascertain what price they will pay in the short and long-term for Firdapse, and what financial assistance they may qualify for, if any. To that end, we also request information in response to the following questions:

1. You have reportedly said that many patients will pay about $10 per month for Firdapse.[8] What is the average and median amount that patients will pay for Firdapse? When calculating a patient's out-of-pocket expenses, are you including the cost of those patients paying their insurance deductible before coverage begins?

2. Dan Brennan, Catalyst's chief commercial officer, said that "[a]ll of the payers we talked to indicated they would provide coverage for patients."[9] Please provide us with the list of each insurance company with which you have been in communication. Please provide us with information on details of the expected insurance coverage for Firdapse, including information you have received from government payers, including information on prior authorization requirements and formulary tier placement.

3. You have been quoted as saying that Catalyst will provide a "free bridge medication supply" to patients while their insurance coverage is determined.[10] Please describe this bridge program in detail. Will patients' insurers be backcharged for drugs provided through this program? Are there any limits placed on the maximum dose a patient can access through the bridge program?

4. What daily dose of Firdapse was used to estimate the annual list price of $375,000? How much will patients pay per 10mg pill if they need more than this daily dose? Is there any dose above which your contracted pharmacies will not fill a valid prescription?

5. You told investors that Catalyst Pathways is a "comprehensive patient insurance navigation and financial assistance program designed so a patient is paying out of pocket a minimal amount of money."[11] Please provide us with a detailed description of this program. Patients have shared with us that Catalyst requires enrollment in the Catalyst Pathways program (which requires a release of Personal Health Information to Catalyst Pharmaceuticals, Inc. and its representatives, agents, contractors, and affiliates) before Catalyst will provide patients with information about what help may be available.[12]

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[8] *See* note 4.
[9] *Id.*
[10] *Id.*
[11] *See* note 1.
[12] Catalyst Pathways Enrollment Form (Dec. 2018), (online at https://www.needymeds.org/papforms/catefe3203.pdf).

6. Patients have reported to us that Catalyst will not directly administer financial assistance, and is, instead, directing patients to NeedyMeds.org. If this is true, how much money is Catalyst providing to NeedyMeds.org or other charities, and what is the expected total value of the tax breaks for these donations? What are the financial eligibility criteria used in making an eligibility determination for patients with LEMS? What is the cutoff income or asset level above which patients will not be eligible for any financial assistance? What is the income or asset level below which Catalyst will provide Firdapse for $10 or less?

7. Patients have reported to us that Catalyst is only providing Firdapse through one specialty pharmacy, Anovo, in Memphis, Tennessee. Is this true? If not, please provide information on the distribution plan for Firdapse, including a list of pharmacies from which Firdapse may be dispensed. If a patient has insurance coverage for Firdapse but Anovo or any other pharmacy with which Catalyst has contracted is out-of-network for that patient, what will that patient be charged?

Please provide the requested documents and information by February 18, 2019 to 332 Dirksen Senate Office Building, Washington, DC 20510. If you have any questions, please contact Sophie Kasimow on my staff at (202) 224-5141.

Sincerely,

Bernard Sanders
United States Senator