

# United States Senate

WASHINGTON, DC 20510

March 3, 2017

Mr. Jeffrey S. Aronin  
Chairman and CEO  
Marathon Pharmaceuticals, LLC  
1033 Skokie Blvd.  
Northbrook, IL 60062

Dear Mr. Aronin:

We write to understand the pricing scheme for Marathon's recently approved product Emflaza, known by the non-proprietary name deflazacort. Deflazacort, like prednisone, is a steroid and has been used internationally to treat the symptoms of Duchenne Muscular Dystrophy (DMD) for decades.<sup>1</sup> DMD is a rare genetic disorder that affects approximately 1 in 3,500 male births in the United States, and most boys do not live past their teens and 20s. The circumstances surrounding the development of Emflaza, and the benefits that will accrue to Marathon as a result of its approval as a new drug, raise serious questions about whether there is any justification for such a dramatically high price.

Deflazacort, like many steroids, has numerous medical applications. However, no company has sought FDA approval of this drug until Marathon's new drug application (NDA) filing in 2016. Many DMD patients have been importing deflazacort from Canada and Europe at about \$1000 per year for personal use.<sup>2</sup> Last month, the FDA announced the approval of NDA for deflazacort. Following the FDA approval of Marathon's NDA, Marathon announced that it planned to charge \$89,000 a year for the drug – a price 50- to 70- times more expensive than the price in Europe.

Marathon executives have defended the price of the drug, arguing that other treatments for "orphan diseases" – those that affect fewer than 200,000 nationwide – cost more than \$300,000. Marathon's Chief Financial Officer (CFO) has stated that Emflaza is "modestly priced for an orphan drug."<sup>3</sup> While this is true, orphan drugs for rare diseases are generally not old compounds that have been used to treat such rare disease for decades.

Many pharmaceutical companies justify the costs of their products based on the expense and risk of developing new compounds and bringing them through FDA approval. One example for DMD is Exondys 51, a novel exon-skipping, genotype specific drug, which was recently

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<sup>1</sup> <http://www.reuters.com/article/us-fda-marathon-pharmaceuticals-idUSKBN1502N6>

<sup>2</sup> FDA exercises enforcement discretion in these circumstances of personal importation. Section 804(j) of the Federal Food, Drug, and Cosmetics Act.

<sup>3</sup> <https://www.wsj.com/articles/marathon-pharmaceuticals-to-charge-89-000-for-muscular-dystrophy-drug-1486738267>

approved for a small subpopulation of boys with DMD. In contrast, Marathon did not develop deflazacort, nor is it a new molecule. Marathon acquired the rights to clinical trial data from the 1990s that had not been fully analyzed. In order to gain approval for Emflaza, the FDA required Marathon to complete a full analysis of the old trial data and conduct minor clinical pharmacology and toxicology studies. Given that Marathon is a privately held company and does not report to the SEC, it is unknown how much it paid to acquire the old trial data or spent on research and development, but we do know that unlike novel compounds, there was no risk in developing deflazacort – as it is already used by about 22% of DMD patients.<sup>4</sup>

Emflaza was approved as a new drug, which means Marathon will now receive benefits under the Orphan Drug Act for the product.<sup>5</sup> This includes a seven-year monopoly on the DMD indication for deflazacort, even though it has long been available as a generic in other countries.<sup>6</sup> Marathon also received a rare disease priority review voucher, which allows companies that gain approval for pediatric orphan drugs to demand faster approval from the FDA for another drug. Marathon can either use the voucher itself or sell it to another company. These vouchers have been sold for up to \$350 million.<sup>7</sup>

We are concerned that Marathon’s pricing unfairly exploits the DMD patient population and the FDAs orphan drug incentives. We understand that your company has delayed the marketing of Emflaza due to concerns about the price, and ask that you respond to the following questions by March 13, 2017:

1. Please provide the methodology used by Marathon to establish Emflaza’s \$89,000 list price.
  - a. How much did Marathon pay to acquire the deflazacort clinical trial data?
  - b. How much did Marathon spend to analyze the clinical trial data and collect additional pre-clinical and clinical data before FDA approval?
  - c. How much does Marathon anticipate earning from the seven-year monopoly on the DMD indication of deflazacort?
  - d. How much does Marathon anticipate on spending on post-market studies of deflazacort?
2. Marathon’s CFO has committed that Emflaza will be a “zero to low out-of-pocket expense” for patients because of insurance and financial assistance programs.<sup>8</sup>
  - a. What agreements have you reached with pharmacy benefit managers and insurance companies to establish payment rates for deflazacort?
  - b. Please provide details of the financial assistance programs available for low-income patients.
  - c. What is your plan to ensure that patients who are not covered by insurance or who have high deductible drug plans are able to afford the drug?

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<sup>4</sup> <https://www.cdc.gov/ncbddd/muscular dystrophy/data.html>

<sup>5</sup> <http://www.forbes.com/sites/matthewherper/2017/02/10/a-6000-price-hike-should-give-drug-companies-a-disgusting-sense-of-deja-vu/#1fb77b8f7327>

<sup>6</sup> [https://www.washingtonpost.com/news/wonk/wp/2017/02/10/an-old-drug-gets-a-new-price-to-fight-a-rare-disease-89000-a-year/?utm\\_term=.642eb7f20eb3](https://www.washingtonpost.com/news/wonk/wp/2017/02/10/an-old-drug-gets-a-new-price-to-fight-a-rare-disease-89000-a-year/?utm_term=.642eb7f20eb3)

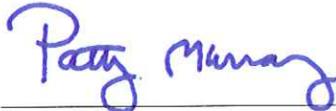
<sup>7</sup> <http://www.businessinsider.com/r-update-1-fda-approves-marathon-pharmaceuticals-dmd-drug-2017-2>

<sup>8</sup> [https://www.washingtonpost.com/news/wonk/wp/2017/02/10/an-old-drug-gets-a-new-price-to-fight-a-rare-disease-89000-a-year/?utm\\_term=.642eb7f20eb3](https://www.washingtonpost.com/news/wonk/wp/2017/02/10/an-old-drug-gets-a-new-price-to-fight-a-rare-disease-89000-a-year/?utm_term=.642eb7f20eb3)

- d. How much does Marathon anticipate earning from public and private insurers for patients who receive out-of-pocket assistance?
3. What are Marathon's plans for the rare disease priority review voucher it received for the approval of Emflaza? Does Marathon plan to apply the voucher for its own future drug applications or does it plan to sell the voucher?
4. How much yearly gross revenue does Marathon anticipate from deflazacort for each year of the 7-year exclusivity period?
5. Marathon cites that the number of DMD patients treated with deflazacort in the United States before approval is 7-9%, while the CDC reports that it is 22%.<sup>9</sup> Please provide the data supporting your statistic.
6. Does Marathon have any other products with orphan drug designation? If so, have these products been approved in other countries or the United States? Are they new molecules? Were they developed by Marathon Pharmaceuticals or acquired from other companies?

We appreciate your prompt response to our requests. If you have any questions, please contact Remy Brim (202-224-7675) or Elizabeth Letter (202-224-6403) with Senator Murray's HELP Committee staff.

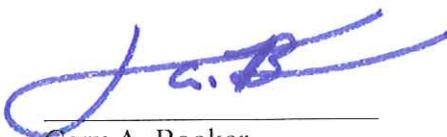
Sincerely,



Patty Murray  
U.S. Senator



Tammy Baldwin  
U.S. Senator



Cory A. Booker  
U.S. Senator



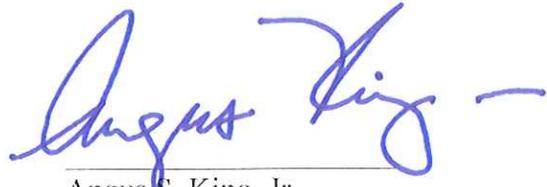
Al Franken  
U.S. Senator

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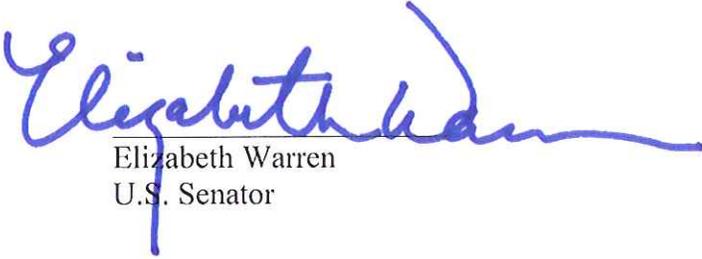
<sup>9</sup> <https://www.cdc.gov/ncbddd/muscular dystrophy/data.html>



Margaret Wood Hassan  
U.S. Senator



Angus S. King, Jr.  
U.S. Senator



Elizabeth Warren  
U.S. Senator



Sheldon Whitehouse  
U.S. Senator