Congress of the United States
Washington, DC 20510

February 13, 2017

Jeffrey S. Aronin
Chairman and Chief Executive Officer
Marathon Pharmaceuticals, LLC
1033 Skokie Boulevard
Northbrook, IL 60062

Dear Mr. Aronin:

We are writing to request information about Marathon Pharmaceuticals' outrageous plan to begin charging $89,000 per year for the drug Emflaza (deflazacort) to treat a deadly genetic muscle deterioration disorder that affects about 15,000 Americans, mostly boys.

Deflazacort has been available to patients in the European Union and Canada for many years, and patients in the United States have been importing it for decades.[1] This drug is currently available in Canada and the United Kingdom for approximately $1,000 per year.[2]

Marathon did not develop deflazacort. Rather, Marathon acquired the rights to historical clinical trial data from the 1990s and completed some additional analyses to gain approval from the Food and Drug Administration (FDA) to sell the drug in the United States.[3]

We believe Marathon is abusing our nation's "orphan drug" program, which grants companies seven years of market exclusivity to encourage research into new treatments for rare diseases – not to provide companies like Marathon with lucrative market exclusivity rights for drugs that have been available for decades.

Marathon will have a monopoly on deflazacort for years to come, preventing less expensive generic competitors from entering the market, despite the fact that this drug is already available in generic form in other countries. Your price to patients, insurers, and taxpayers reportedly will be 50 to 70 times higher than the price currently charged overseas.[4]

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[1] John Carroll, *Marathon's Cheap, Old Steroid Breezes Through the FDA for Duchenne MD, and Gets Priced at $89K*, Endpoints (Feb. 9, 2017) (online at https://endpts.com/an-old-steroid-breezes-through-the-fda-for-duchenne-md-a-disease-that-has-confounded-developers).
[2] Ed Silverman, *Marathon CEO, Who Was Praised by Martin Shkreli, Causes a New Stink Over Drug Pricing,* STAT (Feb. 10, 2017) (online at https://www.statnews.com/pharmalot/2017/02/10/marathon-dmd-martin-shkreli-drug-pricing/).
[3] Joseph Walker, *Marathon Pharmaceuticals to Charge $89,000 for Muscular Dystrophy Drug After 70-Fold Increase,* Wall Street Journal (Feb. 10, 2017) (online at www.wsj.com/articles/marathon-pharmaceuticals-to-charge-89-000-for-muscular-dystrophy-drug-1486738267).
[4] *Id.*

Deflazacort is used to treat a variety of conditions. For this reason, experts have raised concerns that its orphan drug status may be an example of "salami slicing" – the practice of obtaining an orphan drug designation for a narrow disease indication, with the expectation that it will be also used widely for additional off-label treatments.[5]

Marathon's Chief Financial Officer has defended the outrageous price tag for this drug by claiming that it is "modestly priced for an orphan drug."[6] That argument is not a defense of Marathon's actions, but rather an indictment of a system that allows drug companies to engage in such opportunistic pricing behavior.

Marathon also received another lucrative award from the government for the approval of this drug – a rare pediatric disease Priority Review Voucher (PRV). This PRV can be used to gain accelerated approval of another of Marathon's drugs, or can be sold to another company for hundreds of millions of dollars.[7]

We are investigating how Marathon set its price for this drug and how much the company stands to make as a result. We request that you provide the following documents and information by February 27, 2017:

(1) the identity of the company from which Marathon licensed the clinical trial data that was originally generated by Nordic Merrell Dow;[8]

(2) Marathon's total expenses relating to the development and approval of this drug, including the acquisition and review of historical clinical trial data, new clinical trials and analyses, the FDA approval process, and any other expenses;

(3) Marathon's projections and estimates for total revenues and profits for future sales of this drug, including alternative projections and estimates based on various pricing structures;

(4) all communications, including e-mail communications, between Marathon executives or employees and any insurance companies, pharmacy benefit

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[5] Sarah Karlin-Smith, *Double Whammy? New Drug Approval Raises Concerns About Pricing, Abuse of Rare Disease Incentives*, Politico (Feb. 9, 2017) (online at www.politicopro.com/health-care/story/2017/02/double-whammy-new-drug-approval-raises-concerns-about-pricing-abuse-of-rare-disease-incentives-147936). See also Carolyn Johnson, *An Old Drug Gets a New Price to Fight a Rare Disease: $89,000 a Year,* Washington Post (Feb. 10, 2017) (online at 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=.31eac01e7766).

[6] Joseph Walker, *Marathon Pharmaceuticals to Charge $89,000 for Muscular Dystrophy Drug After 70-Fold Increase,* Wall Street Journal (Feb. 10, 2017) (online at www.wsj.com/articles/marathon-pharmaceuticals-to-charge-89-000-for-muscular-dystrophy-drug-1486738267).

[7] *Regulatory Explainer: Everything You Need to Know About FDA's Priority Review Vouchers,* RAPS (Nov. 3, 2016) (online at www.raps.org/Regulatory-Focus/News/2015/07/02/21722/Regulatory-Explainer-Everything-You-Need-to-Know-About-FDA%E2%80%99s-Priority-Review-Vouchers/#sthash.Rd4M1uLu.dpuf).

[8] Ed Silverman, *Marathon CEO, Who Was Praised by Martin Shkreli, Causes a New Stink Over Drug Pricing,* STAT (Feb. 10, 2017) (online at https://www.statnews.com/pharmalot/2017/02/10/marathon-dmd-martin-shkreli-drug-pricing).

managers, or government payers – including Medicare, state Medicaid programs, and the Veterans Administration – regarding coverage of this drug; and

(5) a description of any patient assistance or coupon programs Marathon plans to offer for this drug, and information on the qualifications, anticipated number of patients served, anticipated tax deductions, and the value of the drugs provided under these programs.

We first wrote to you in October 2014 regarding Marathon's staggering price increases for two older heart medications you acquired, Isuprel and Nitropress, which you then sold to Valeant Pharmaceuticals.[9] We remain gravely concerned about these recurring abuses in the pharmaceutical industry. Exorbitantly pricing potentially life-saving medications that should be widely available for a fraction of the price hinders patient access and drives up costs for the entire health care sector.

We urge you to significantly lower your price for this drug before it goes on the market next month. Marathon's apparent abuse of government-granted exclusivity periods and incentives to sell what should be a widely available drug for $89,000 a year is unconscionable.

Sincerely,

Bernard Sanders
United States Senator

Elijah E. Cummings
Ranking Member
House Committee on Oversight and Government Reform
House of Representatives

[9] Letter from Senator Bernard Sanders, Chairman, Subcommittee on Primary Health and Aging, Senate Health, Education, Labor, and Pensions Committee, and Ranking Member Elijah E. Cummings, House Committee on Oversight and Government Reform Committee, to Jeffrey Aronin, Chairman and Chief Executive Officer, Marathon Pharmaceuticals, LLC (Oct. 2, 2014) (online at www.sanders.senate.gov/download/letter-to-mr-aronin-chairman-and-ceo-marathon-pharmaceuticals-llc?inline=file).