

Congress of the United States
Washington, DC 20510

March 22, 2017

Stuart W. Peltz
Chief Executive Officer
PTC Therapeutics Inc.
100 Corporate Court
South Plainfield, NJ 07080

Dear Dr. Peltz:

We are writing to request information about the price your company intends to charge for deflazacort, a drug that is used in the treatment of Duchenne Muscular Dystrophy.

Last week, PTC announced to investors that you purchased the rights to the brand name medicine Emflaza from Marathon Pharmaceuticals and that you plan to launch it in the United States in the near future.

Deflazacort, which has been available in other countries for decades, currently costs about \$1,000 to \$1,200 a year if imported from the United Kingdom. That is why Marathon's outrageous proposed price of \$89,000 caused a backlash from patients, their families, and the medical research community. It also generated inquiries from multiple lawmakers, many of whom are increasingly skeptical of the current orphan drug regulatory approval process.

Although PTC has not yet announced its own pricing plan, we understand that your company spoke with members of the Parent Project for Muscular Dystrophy and are setting up a more detailed conversation with them and your health economist. We urge you to keep the price of this relatively common steroid at its current importation cost. Doing so will allow patients to use deflazacort in combination therapies without going into bankruptcy.

We would also like to know if you intend to pursue an additional approval of deflazacort as an orphan drug to treat juvenile arthritis. We are carefully monitoring deflazacort's accessibility to families supporting members with Duchenne Muscular Dystrophy and look forward to hearing from you in response to our questions.

We would appreciate a reply to this inquiry by April 3, 2017. Please direct any queries pertaining to this request to Alicia Mundy of Senator Sanders' Budget Committee staff at (202) 224-0642 or Will Cunningham of Ranking Member Cummings' staff at (202) 225-5051.

Stuart W. Peltz
Chief Executive Officer
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Thank you for your attention to this matter.

Sincerely,



Bernard Sanders
United States Senator



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