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## **An Open Letter to the Duchenne Community**

Dear Members of the Duchenne Community:

I want to thank you all for your support and encouragement over the last several years as Marathon worked to bring EMFLAZA™ (deflazacort) through the rigorous FDA approval process so patients in the United States would finally have access to this medicine and an understanding of this product. We know this has been a decades-long goal of the Duchenne community and we are proud to have been your partners in this effort.

As we all know, deflazacort was being used without FDA approval -- and without ever having been approved anywhere in the world for Duchenne -- by a very small group of patients (roughly 7 to 9 percent) in the Duchenne community who imported it from overseas. Our goal in commercializing EMFLAZA all along has been to make it available to a much broader set of patients who, prior to FDA approval, have not had access to this therapy. There was also much we did not know about using the drug in the Duchenne patient population including proper dosing, potential side effects and drug-to-drug interactions. Please see important safety information below.

As a result of Marathon's research and the subsequent FDA approval, that information is now available to physicians in the United States so they can prescribe with confidence. I am deeply proud of this and I hope that thousands more children who would never have had the opportunity to access this drug may now benefit from it.

Since last week's approval, we have heard both support from the community and concerns about how the pricing and reimbursement details will affect individual patients and caregivers, such as how it affects coverage of other components of Duchenne treatment. Based on these questions, today we are announcing:

1. We are pausing our commercialization efforts in order to meet with Duchenne community leaders and explain our commercialization plans, review their concerns, discuss all options, and move forward with commercialization based on the resulting plan of action
2. We will continue to maintain our Expanded Access Program for patients receiving EMFLAZA
3. Patients currently receiving deflazacort from other sources may continue to have that option

We know the outcome of those discussions will be of keen interest to many of you, and we will keep the community updated.

In the meantime, I want to assure you that Marathon is committed to further research and development to effectively treat Duchenne. When we set the price of EMFLAZA, we considered the following: the resources we invested to get to this point, the resources

needed to complete the phase 4 clinical studies and FDA post-marketing study commitments, the ability to fund future research and development, and the ability to ensure broad patient access through insurer reimbursement and our own patient assistance programs. There is confusion that this is a generic drug. In the United States, FDA considers deflazacort a new drug and we had to get it approved. Our tablets are manufactured in the United States.

The resources we invested were substantial and we don't expect to recoup our investment for several years and we have only 7 years of market exclusivity.

If we are profitable, we are committed to re-investing our earnings from EMFLAZA into additional research into Duchenne. We are in regular contact with leaders of the Duchenne community about our plans and look forward to continuing that engagement.

I want to assure caregivers and others that our preliminary meetings with the payer/insurer community have gone well and many have acknowledged the price was appropriate given the very small patient population. We believe that access to deflazacort in the United States will be dramatically expanded once EMFLAZA is commercially available.

I also want you to know that we hear and understand your concerns around the price and recognize it is tied to fears you have about reimbursement for other components of Duchenne treatment. We know all parents want the very best care possible for their child and being able to access quality care is paramount. We will continue the discussion on pricing and access with you until there is a full understanding of how pricing decisions directly impact the revenues needed to fund past, current and future research as well as opportunities to purchase new therapeutic treatments.

***Most importantly of all, I want to reinforce that every patient who needs this drug will have access to it, and that price should not be a barrier. Put simply, we expect patients will pay a standard co-pay of typically \$20 or less per prescription. We have developed the most robust patient access programs allowed by law. If you have questions about access, please call 1-844-EMFLAZA and a trained nurse case manager will walk you through the programs and options. Our goal is that this will cost patients significantly less than they pay today if they were among the few who were importing it.***

In summary, please take away three important facts about Marathon's profound commitment to the Duchenne community.

1. We are committed to the Duchenne community for the long term and we will re-invest the earnings into developing and funding additional therapies.
2. Anyone who needs this medicine will get this medicine.
3. Thousands of children in this country will finally receive this medicine that they were previously denied, and every patient and physician will now have the benefit of additional science as a result of the FDA approval.

Please know we sought FDA approval of EMFLAZA to improve access to this treatment. We have and always will support you in that endeavor.

We hope this brings clarity and comfort to you and reassures you that your child's good health, access to EMFLAZA and an understanding of the clinical profile are our highest goals.

Sincerely,



Jeff Aronin  
Chairman and CEO  
Marathon Pharmaceuticals, LLC

#### **ABOUT EMFLAZA™ (deflazacort)**

EMFLAZA is indicated for the treatment of Duchenne muscular dystrophy in patients 5 years of age and older.

**EMFLAZA is not for patients who are allergic to deflazacort or any of the inactive ingredients in EMFLAZA.**

**Patients should not stop taking EMFLAZA, or change the amount they are taking, without first checking with their healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of serious side effects.**

Corticosteroids, such as EMFLAZA can cause:

- Hyperglycemia, altered glucose metabolism
- Increased risk of infection
- Changes in cardiovascular/kidney function that could lead to increases in blood pressure, salt, and water retention, and decreases in blood levels of potassium and calcium
- Behavioral and mood changes that could lead to potentially severe psychiatric adverse reactions
- Osteoporosis, decrease in bone mineral density
- Serious skin rashes
- May slow growth and development
- Cataracts or glaucoma

**Vaccinations:** The administration of live or live attenuated vaccines is not recommended while taking EMFLAZA. Killed or inactivated vaccines may be administered, but the responses cannot be predicted. Patients should discuss their vaccine history with their healthcare provider before starting EMFLAZA, and while taking

EMFLAZA, patients should check with their healthcare provider before receiving any new vaccines or booster shots.

**Common side effects that could occur with EMFLAZA include:** Facial puffiness or Cushingoid appearance, weight increased, increased appetite, upper respiratory tract infection, cough, frequent daytime urination, unwanted hair growth, central obesity, and colds.

Patients should tell their healthcare provider if they have had recent or ongoing infections, develop a fever, or experience any other side effects.

**Please see [www.EMFLAZA.com](http://www.EMFLAZA.com) for full Prescribing Information.**

You may report side effects to ProPharma Group at 1-866-562-4620 or [drugsafety@propharmagroup.com](mailto:drugsafety@propharmagroup.com).

You may report side effects to FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch).

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