February 20, 2018

Dear Duchenne community,

As you may have seen from our recent press release, the FDA’s Office of New Drugs has denied our appeal. While this is not the outcome we were all hoping for we are encouraged that the FDA has recommended a possible path forward for ataluren review in the US. This would involve PTC resubmitting an NDA for ataluren under the accelerated approval framework utilizing the current efficacy and safety data in conjunction with new data to be generated on dystrophin production in nonsense mutation Duchenne patients. We are in discussions with the FDA on the methods to collect the dystrophin data and expedite this potential path forward.

Patients are always at the heart of everything we do at PTC and we understand the importance of access to treatments as soon as possible. We are working as quickly as we can so that patients in the US can have access to ataluren. All patients that have participated in ataluren clinical trials have remained on ataluren, some for more than 10 years. Currently there are over 150 patients in the US receiving treatment, PTC intends to keep supplying ataluren to these patients at least until the re-submission of the NDA in the US.

In addition, we will continue to generate data from our current ongoing trial (Study 041) that is being conducted in the US and around the world. FDA has indicated that data from this trial could serve as a confirmatory post approval trial in connection with the accelerated approval framework. It is our goal that all patients who may benefit have access to Translarna.

We thank you all for your support and partnership.

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