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Update to the Duchenne Community,

We'd like to share an update on the current regulatory processes for Translarna (ataluren) in Europe and the United States.

In the U.S., PTC recently participated in a [Type C] regulatory meeting with the U.S. Food and Drug Administration (FDA) to discuss a potential path to a resubmission of a New Drug Application (NDA) for Translarna. The discussion focused on the totality of evidence collected to date from the Translarna clinical trials and the STRIDE registry. Based on the discussion, FDA suggested that we request a follow-up meeting to align on the specific contents that could support an NDA filing. We look forward to continuing to collaborate with the FDA and expect this next discussion to occur in the first quarter of 2024.

In Europe, we continue to pursue a re-examination of the initial opinion from the Committee for Medicinal Products (CHMP) on the renewal of the conditional marketing authorization for Translarna. The submission of the briefing document, a central part of this process, is now complete. The re-examination is expected to take place through late January 2024, when an opinion will be provided by CHMP. This opinion will then be sent for adoption by the European Commission within 67 days. As a reminder, Translarna remains available for current patients in Europe, and physicians can continue to prescribe Translarna for new patients at this time.

With approximately 3,000 patients in over 50 countries treated with Translarna, we continue to firmly believe in the strength of the totality of evidence gathered which demonstrates short-and long-term benefits on key functional aspects of the disease, including ambulatory and neuromuscular function. Many of you have shared that you have witnessed these outcomes firsthand.

We're pleased with these recent steps forward and know that there is still much work ahead of us. We thank you for your continued support, strength and partnership and wish everyone happy holidays.