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We are providing this update to the Duchenne muscular dystrophy (DMD) community following the decision in February to withdraw the ataluren (Translarna™) approval application in the United States based on FDA feedback.

PTC resubmitted the approval application for ataluren to FDA in the summer of 2024. The FDA accepted the NDA for review in October 2024. Following discussions with FDA in late January 2026, it was clear that the overall ataluren data package would not meet FDA's threshold for approval. Therefore, PTC made the decision to withdraw the application given the seeming certain negative outcome and the need to provide clarity to nonsense mutation Duchenne muscular dystrophy (nmDMD) families who were waiting for a decision from FDA for over 16 months.

We understand this outcome is disappointing for individuals and families with nmDMD who have long desired a therapy specifically targeting the nonsense mutation. We have worked tirelessly over 20 years to gain approval for ataluren and regret that we will not be able to achieve this goal. Given that we have existing drug supply in the US, we will continue to provide ataluren for individuals currently on treatment while existing supply lasts. We expect this will be for approximately six months and will provide an update on timing in the early summer. In order to continue receiving drug, access will be provided through individual physician INDs. Therefore, treating physicians must submit a request to FDA to provide drug, a process familiar to most DMD physicians. If your physician has questions regarding this process, they can contact PTCActs@ptcbio.com. Due to limited drug supply, we are only able to provide ataluren to those currently on treatment.

We know how meaningful the ataluren program has been to the Duchenne community. We are disappointed that, despite our best efforts, we have not been able to achieve FDA approval. We are grateful for the community's courage and dedication, and we sincerely thank you for the many years of unwavering support. We especially want to thank the individuals and families who took part in ataluren clinical trials. Your pioneering participation—among the earliest Duchenne clinical trials—helped advance the field and contributed lasting knowledge that continues to inform research and progress for the community.