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Update to the EU Duchenne Community:

Today we shared the disappointing news that the Committee for Medicinal Products for Human Use (CHMP) has issued a negative opinion on the renewal of the conditional marketing authorization of Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in Europe. This opinion follows the return of the previously issued negative opinion by the European Commission for re-review.

As part of this review procedure, the CHMP convened a Scientific Advisory Group which concluded that the evidence of significant efficacy provided by STRIDE "should not be ignored." This view of the expert committee is consistent with the view of Duchenne key opinion leaders and makes the CHMP opinion even more disappointing. We continue to believe the strength of the totality of evidence of Translarna safety and benefit supports its continued authorization.

Our work to maintain and broaden access to Translarna will continue. We plan to request re-examination of the CHMP opinion. During this time the marketing authorization for Translarna remains in effect. Based on the timeline of the reexamination procedure and the subsequent review of the opinion by the European Commission, we expect Translarna to remain on the market through the end of 2024 even if the negative opinion is maintained and ratified.

We will continue our efforts to maintain availability of Translarna on the market for as long as possible. We thank you for your continued support.