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Update to the Duchenne Community,

The European Commission (EC) has adopted the opinion of the Committee for Medicinal Products for Human Use (CHMP) to not renew the marketing authorization for Translarna™ (ataluren) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). This means that Translarna no longer has conditional marketing authorization in the European Economic Area. However, the Commission indicated that individual countries within the European Union have a mechanism to potentially allow access to Translarna. This potential for continued treatment speaks to its safety, benefit and lack of alternative treatments for boys and young men with nmDMD.

We are very disappointed that after this prolonged period of review the EC has decided to adopt the CHMP negative opinion for Translarna. We understand the devastating impact of this decision for the Duchenne muscular dystrophy community and continue to believe in the strength of data supporting Translarna. PTC will work with the local health authorities in individual markets to provide Translarna where possible.

This decision does not impact countries that have standalone regulatory approvals, such as the United Kingdom (England, Scotland, Wales, and Northern Ireland) and Brazil.

Patients should work with their treating physician to assess their current treatment plan and understand potential pathways to access treatment.

We are grateful for the passionate work of the community and courageous efforts to demonstrate the need for and the effectiveness and safety of Translarna. We thank the community for their unwavering support.