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Update to the Duchenne Community,

The Committee for Medicinal Products for Human Use's (CHMP) of the European Medicines Agency (EMA) issued a negative opinion following the re-examination procedure for the conditional marketing authorization of Translarna (ataluren) in Europe. This opinion was issued despite the stated support from the patient community and expert physicians as well as the strength of the data. We are highly disappointed and understand the devasting impact this recommendation may have for European children and young men with Duchenne Muscular Dystrophy caused by a nonsense mutation (nmDMD).

The opinion will now be sent to the European Commission for ratification which should occur within 67 days. During this time, Translarna will remain on the market, and any questions about an individual treatment plan should be discussed with your healthcare professional.

The passionate work of the community and the courageous efforts to demonstrate the need for and effectiveness of Translarna have been inspiring.