Priority question for written answer P-000643/2024
to the Commission
Rule 138
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Subject: Research for the treatment and cure of Duchenne muscular dystrophy

Duchenne muscular dystrophy (DMD) is a rare genetic disorder that becomes apparent during early childhood. By early adolescence, it causes severe symptoms such as rapidly progressive muscle weakness and muscular atrophy.

DMD affects around one in 3 500 newborn boys. It is therefore classed as a ‘rare’ disease – despite the fact that the European Medicines Agency (EMA) estimates that approximately 26 000 people suffer from the condition in Europe. At present, DMD has no cure. Medicines to alleviate symptoms are rare or scarcely available to those affected.

1. What research projects is the Commission currently supporting to advance research into the condition, improve its treatment and to find a cure?

2. Are there currently any specific research projects or calls under the current or future Horizon Europe programme focusing on this condition?

3. Are there currently any promising medicines for the treatment of DMD which have been given the ‘orphan designation’ and receive scientific and regulatory support from the EMA so that they can be sufficiently developed for an application to be submitted for their approval?

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