Duchenne muscular dystrophy is a very rare genetic disease that appears in early infancy and leads to death at around the age of 25. The severest symptoms are already occurring by the age of ten, and include an inability to walk and then loss of use of the arms, followed by lung complications and then heart complications.

The disease is incurable, but a drug called Translarna (active substance Ataluren), which is currently on the market, slows the advance of the disease and can help ensure those afflicted remain mobile for up to three and a half more years.

On 26 January 2024, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) issued a negative opinion on renewal of the marketing authorisation for Translarna in Europe.

According to clinical evidence, this medicine does not present a risk to patients’ health.

Besides this, the trial shows taking this medicine to have extended patients’ ability to walk by as much as three and a half years.

The medicine and those three and a half years of better quality of life offer real hope not just to sufferers, but also to their families.

Can the Commission state whether it will grant an extension for the marketing of Translarna and support further and prolonged clinical trials on Translarna?

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