Question for written answer E-000088/2014
to the Commission
Rule 117
Olga Sehnalová (S&D)

Subject: Rules for conditional approval of the drug Ataluren

The new drug Ataluren (PTC124), which is currently being trialled, is intended for the treatment of patients with Duchenne muscular dystrophy (DMD). DMD is the most severe form of muscular dystrophy. According to data communicated by the civic association Parent Project, it affects around one in 3,600 boys. DMD results in progressive and inevitable weakening of the muscles, leading to a loss in the ability to walk and eventually to the failure of respiratory and cardiac functions. Most patients with DMD die at around 20 years old. According to Parent Project, there are no treatment options currently in existence for boys with DMD in Europe. According to information from Parent Project, Ataluren presents an alternative for 13% of boys and young men with DMD caused by a rare genetic mutation known as a point nonsense mutation. In clinical trials, including large randomised and placebo-controlled studies, Ataluren was well tolerated and results show that it clearly slows down the process of losing the ability to walk. The European Commission has granted the drug Ataluren the status of “orphan drug”. The commercial sponsor PTC Therapeutics has applied to the European Medicines Agency (EMA) for a conditional approval of the drug. Unless this substance is now granted conditional approval by the EMA, it will take another three years for boys and young men with DMD to get access to it.

Can the Commission advise what rules currently apply for granting the status of conditional approval for new medicines as compared with the status of orphan drug?

What is the Commission’s attitude toward the benefits of treating patients with Duchenne muscular dystrophy with the drug Ataluren (PTC124)?