10 November 2016

Dr. Tomas Salmonson
European Medicines Agency
30 Churchill Place
Canary Wharf
London E14 5EU
United Kingdom

RE: Withdrawal of Kepnetic, aceneuramic acid, 500mg, prolonged release tablet
Procedure Number: EMEA/H/C/004176

Dear Dr. Salmonson,

I would like to inform you that, at this point of time, Ultragenyx UK Limited has taken the decision to withdraw the application for Marketing Authorisation of Kepnetic, aceneuramic acid, 500 mg prolonged release tablets, which was intended to be used for the "Treatment of Adult Patients with GNE Myopathy".

This withdrawal is based on the following reason:

In order to allow early access of Kepnetic to patients, the Applicant filed a conditional Marketing Authorisation Application based on data from a single preliminary Phase 2 clinical study.
The CHMP considers that the data were encouraging, but did not provide a sufficient amount of evidence to support an approval at this time. The Applicant intends to obtain additional efficacy data from an ongoing Phase 3 study to further confirm the effects of Kepnetic for Marketing Authorisation.

There are no consequences for patients currently included in clinical trials with Kepnetic.
Kepnetic currently is being studied in several ongoing clinical trials worldwide.

We reserve the right to make further submissions at a future date in this or other therapeutic indication(s).

Ultragenyx agrees for this letter to be published on the EMEA website.

Yours sincerely,

[redacted]