BIOMARIN

31 May 2016

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

Re: Withdrawal of Kyndrisa, drisapersen, 188.7 mg/ml, solution for injection Marketing Autorisation Application, EMA/H/C/0003846

Dear Tomas Salmonson,

I would like to inform you that BioMarin has taken the decision to withdraw the Marketing Authorisation Application for Kyndrisa, drisapersen, 188.7 mg/ml, solution for injection, which was intended to be used for the treatment of Duchenne muscular dystrophy in ambulatory patients aged 5 years and older with mutations in the dystrophin gene that are amenable to treatment with exon 51 skipping as determined by genetic testing.

This withdrawal is based on the following reasons: BioMarin has determined that it will not be able to resolve the CHMP's major objection related to the results of clinical studies within the regulatory timeframe.

BioMarin intends to discontinue clinical and regulatory development of Kyndrisa.

BioMarin plans to work with physicians, patient groups, and regulatory authorities to provide currently treated patients the opportunity to access the remaining supply of Kyndrisa, as legally permitted, in a program to be designed.

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

BioMarin agrees to this letter being published on the EMA website.