4th November 2020

European Medicines Agency

Domenico Scarlattilaan 6

1083 HS Amsterdam

The Netherlands

To: Dr Martina Schussler-Lenz, CAT Chair

Dr Harald Enzmann, CHMP Chair

Subject: Withdrawal of Roctavian, valoctocogene roxaparvovec, 2 x 1013 vector genomes/ml, solution for infusion, for Marketing Authorisation Application, EMEA/H/C/0004749

Dear Dr Schussler-Lenz and Dr Enzmann,

On behalf of BioMarin, I would like to inform you that, at this point in time, BioMarin International Limited has taken the decision to withdraw the application for Marketing Authorisation for Roctavian, valoctocogene roxaparvovec, 2 x 1013 vector genomes/ml, solution for infusion, which was intended to be used for the treatment of adults with severe haemophilia A.

This withdrawal is based on the following reason: BioMarin has determined that it will not be able to provide the data requested to resolve the CAT's major objection related to the results of clinical studies within the current procedure.

BioMarin confirms that the withdrawal of the Roctavian MAA has no impact for patients in ongoing clinical trials. BioMarin remains fully committed to developing Roctavian for the treatment of severe haemophilia A.

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

I agree for this letter to be published on the EMA website.

Yours sincerely,

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