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Your letter from	Your reference	Our reference	Annex	Date
		FAGG/DGPRE/R&D/VDC 1169555		

Dossier GMO : B/BE/19/BVW2

2017-003573-34: A Phase 3 Open-Label, Single-Arm Study To Evaluate The Efficacy and Safety of BMN 270, an Adeno-Associated Virus Vector–Mediated Gene Transfer of Human Factor VIII at a dose of 4E13 vg/kg in Hemophilia A Patients with Residual FVIII Levels ≤ 1 IU/dL Receiving Prophylactic FVIII Infusions.

2017-003215-19: A Phase 3 Open-Label, Single-Arm Study To Evaluate The Efficacy and Safety of BMN 270, an Adeno-Associated Virus Vector–Mediated Gene Transfer of Human Factor VIII in Hemophilia A Patients with Residual FVIII Levels ≤ 1 IU/dL Receiving Prophylactic FVIII Infusions.

Geachte [redacted],

Hierbij informeren wij u dat de vergunningsplicht krachtens het Koninklijk besluit van 21 februari 2005 tot reglementering van de doelbewuste introductie in het leefmilieu evenals van het in de handel brengen van genetisch gemodificeerde organismen of van producten die er bevatten u wordt toegekend op basis van het gunstig advies van de Adviesraad voor Bioveiligheid daterend van 24 mei 2019 en dit volgens de voorwaarden hernomen in de conclusie van bovenvermeld advies, dat wil zeggen:

"- The notifier and the investigators must strictly apply the clinical trial protocol, and all the safety instructions as described in the dossier.

- The document 'Safety data Sheet' should be updated as mentioned under section 4 in the BAC advice.

- Any protocol amendment has to be previously approved by the Competent Authority.

- The notifier is responsible to verify that each study centre has qualified personnel experienced in handling infectious material and that the investigator has the required authorizations to perform the clinical trial activities inside the hospital (laboratory, pharmacy, hospital room, consultation room...) according to the Regional Decrees transposing Directive 2009/41/EC on Contained use of genetically modified micro-organisms.

- The Biosafety Advisory Council should be informed within two weeks when the first patient starts the treatment and the last patient receives the last treatment.

- At the latest six months after the last visit of the last patient included in the trial, the notifier must send the competent authority for the attention of the Biosafety Advisory Council a report with details concerning the biosafety aspects of the project. This report shall contain at least:

o The total number of patients included in the trial and the number of patients included in Belgium;

o A summary of all adverse events marked by the investigators as probably or definitely related to the study medication;

o A report on the accidental releases, if any, of BMN 270."

Met hoogachting,

Maggie De Block

Minister van Sociale Zaken, Volksgezondheid, Asiel en Migratie

Marie Christine Marghem

Minister van Energie, Leefmilieu en Duurzame Ontwikkeling