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Votre lettre du	Vos références	Nos références	Annexe(s)	Date
		[redacted]		

Dossier OGM B/BE/26/BVW3 (2023-505187-11-00): Microdystrophin (GNT0004) Gene Therapy Clinical Trial in Duchenne Muscular Dystrophy: A phase I/II/III study with a dose determination part followed by an efficacy and safety evaluation, quadruple blind placebo-controlled part and then by a long-term safety follow up part, in ambulant boys.

[redacted]

Par la présente, nous vous informons que votre demande d'autorisation a été approuvée.

L'autorisation est conforme à l'arrêté royal du 21 février 2005 réglementant la dissémination volontaire dans l'environnement ainsi que la mise sur le marché d'organismes génétiquement modifiés ou de produits en contenant.
(http://www.ejustice.just.fgov.be/cgi_loi/change_lg.pl?language=fr&la=F&cn=2005022131&table_name=loi)

Votre autorisation est accordée sur base de l'avis favorable du Conseil de Biosécurité daté du 21 mai 2026, aux conditions reprises dans la conclusion de cet avis, à savoir:

Based on the scientific assessment of the notification made by the Belgian experts, the Biosafety Advisory Council concludes that it is unlikely that GNT0004 developed to treat patients with Duchenne muscular dystrophy (DMD), by means of an optimized microdystrophin protein, will have any adverse effects on human health or on the environment in the context of the intended clinical trial provided that all the foreseen safety measures are followed.

Therefore, the Biosafety Advisory Council issues a ***positive advice with the following conditions****:*

- The notifier and the investigators must strictly apply the clinical trial protocol, and all the safety instructions as described in the following documents:

- *Latest version of the ICF*
- *Latest version of the Protocol*
- *SNIF_06May2026*
- *CAF_ NC_BEL_06May 2026*
- *CAF_ C_BEL_06May 2026*
- *DR_Study Staff Instructions_06May 2026*

- As committed by the applicant in his response of 14 April 2026 to our first list of questions, it will be clearly indicated in the ICF that patients who receive treatment must not donate blood, organs, tissues, or cells for transplantation in order to be consistent with recommendation provided in the EPAR of EU registered medicinal products containing recombinant AAV.

- 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 enrolled in the trial, the notifier must send to the competent authority at the attention of the Biosafety Advisory Council a report with details concerning the biosafety aspects of the project. This report will contain as a minimum:

- *The total number of patients enrolled in the trial and the number of patients from Belgium;*
- *A summary of all adverse events documented by the investigators as likely or definitely related to the study medication;*
- *A report on accidental releases, if any, of GNT0004.*

Veuillez agréer mes salutations distinguées,

Frank Vandenbroucke
Vice-Premier ministre et
ministre des Affaires sociales et
de la Santé publique, chargé de
la Lutte contre la pauvreté

Jean-Luc Crucke
Ministre de la Mobilité, du
Climat et de la Transition
environnementale