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Votre lettre du	Vos références	Nos références	Annexe(s)	Date
		AFMPS/DGPRE/R&D/[redacted]		2310812023

Dossier OGM : B/BE/22/BVW5 (2022-000691-19): A two-part, open-label systemic gene delivery study to evaluate the safety and expression of RO7494222 (SRP-9001) in subjects under the age of four with Duchenne muscular dystrophy

Chère [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 17 juillet 2023, aux conditions reprises dans la conclusion de cet avis, à savoir:

"Based on the scientific assessment of the notification made by the Belgian expert, the Biosafety Advisory Council concludes that it is unlikely that RO7494222 (SRP-9001) developed as a gene therapy approach for the treatment of Duchenne Muscular Dystrophy disease will have 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 as described in the documents making part of the notification and the following updated documents:

- *o 4.1 2022-000691-19 20221010_BN43881 EU Common application form - Belgium Public – Clean (sent 6 July 2023)*
- *o 4.1.1 2022-000691-19 20221010_BN43881 EU Common application form - Belgium Confidential Annex – Version 2 , June 2023*
- *o 4.2 2022-000691-19 BN43881 – SNIF – Belgium Clean (sent 6 July 2023)*
- *o 4.3 2022-000691-19 SRP-9001 BELGIUM Summary of information for the public_FINAL_– and version NL_clean and FR_clean*
- *o 5.1 2022-000691-19 BN43881_Belgium Hygiene Guidance v3.0 – Final_18April2023*
- *o 5.3 2022-000691-19 BN43881_Final_Belgium_Addendum to pharmacy & dose administration manuals v2.0_FINAL*

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 (BN43881 Protocol version 2), and all the safety instructions as described in the dossier and the updated and new documents listed here above. Regarding the instruction for patients with respect to donation of blood, organs, tissues, and cells for transplantation, and referring to the notifier's commitment, relevant text in the protocol needs to be adapted and protocol clarification letter shall be distributed to all sites immediately following the approval clinical trial.

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

- The notifier is responsible to verify that the 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.

- At the latest 15 days after the start of the trial, the notifier should provide, along with the delivery of the control sample, a detailed protocol for the method of conservation and analysis of the control sample.

- 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 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 shall at least contain:

- *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 RO7494222 (SRP-9001)"*

Sincères salutations,

Frank Vandenbroucke
Vice-Premier Ministre et
Ministre de la Santé publique et
des Affaires sociales

Zakia Khattabi
Ministre du Climat, de
l'Environnement, du
Développement durable et du
Green Deal