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| Votre lettre du | Vos références | Nos références | Annexe(s) | Date |
|---|---|---|---|---|
| | | AFMPS/DGPRE/R&D/[redacted] | | 24/06/2022 |

**Dossier OGM : B/BE/21/BVW5 (2019-003374-91): A Phase 3 Multinational, Randomized, Double-Blind, Placebo-Controlled Systemic Gene Delivery Study to Evaluate the Safety and Efficacy of SRP-9001 in Subjects With Duchenne Muscular Dystrophy (EMBARK)**

[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 30 mai 2022, 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 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 following updated documents:*

- *Addendum to Pharmacy Manual_Belgium_update_12May2022*
- *BEL Hygiene Guidance for participants - to be updated in accordance with condition 1 here below*
- *Confidential EU Common Application GMO_20220516*

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

*- Given that Zaidy et al (2019) recommend caregivers to practice good hand hygiene for approximately 60 days after the injection with adeno-associated viral (AAV) vector containing the human SMN gene, the BAC recommends, as a precautionary measure, to increase the period of time to practice appropriate hand hygiene for at least 60 days. As a consequence, the notifier is requested to adapt the document "BEL_Hygiene_Guidance" by specifying for each instruction the period during which the recommendation must be followed.*

*- The notifier and the investigators must strictly apply the clinical trial protocol version 3, and all the safety instructions as described in the dossier and the updated and new documents listed here above.*

*- 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 SRP-9001;*
*o The BAC would genuinely appreciate to receive an update of the shedding results upon completion of the SRP-9001-103 study."*

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