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| uw bericht van | uw kenmerk | ons kenmerk | bijlagen | datum |
|---|---|---|---|---|
| | | FAGG/DGPRE/R&D/[redacted] | | 21.06.2024 |

**Dossier GMO: B/BE/24/BVW4 (2022-503112-17-00): A Phase 3 Multinational, Open-label, Systemic Gene Delivery Study to Evaluate the Safety and Efficacy of SRP-9003 in Subjects with Limb Girdle Muscular Dystrophy 2E/R4**

Geachte [redacted]

Wij informeren u dat uw vergunningsaanvraag werd goedgekeurd.

De vergunning is conform 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. (http://www.ejustice.just.fgov.be/cgi_loi/change_lg.pl?language=nl&la=N&cn=2005022131&table_name=wet)

Uw vergunning wordt verleend op basis van het gunstig advies van de Adviesraad voor Bioveiligheid van 27 mei 2024, onder de voorwaarden die in de conclusie van bovengenoemd advies zijn vermeld, namelijk:

*"Based on the scientific assessment of the notification made by the Belgian experts, the Biosafety Advisory Council concludes that it is unlikely that SRP-9003, developed as a gene therapy approach for the treatment of limb girdle muscular dystrophy 2E/R4 disease, will have adverse effects on human health or the environment in the context of the intended clinical trial provided that all of the foreseen safety measures are followed in detail as described in the following updated documents:*

- *Pharmacy Manual (v1.0, 10 August 2023)*
- *Biohazardous Safety Data Sheet (v4.0, July 2023)*
- *Hygiene-Guide_BE_07May2024*
- *Dose Administration Manual (v1.0, 11 August 2023)*
- *Safety-Instructions-for-INV-and-Staff_BE (May 2024)*
- *Main ICF BE (v1.0, 20 March 2024)*
- *BE_GMO_Public CAF (April 2024)*
- *BE_GMO_Confidential Annex (v1.2, April 2024)*
- *BE_GMO_SNIF (May 2024)*
- *PMC_Standard Precautions Instructions Sheet*
- *CMRN Mobile Visit Training*
- *CMRN Mobile Visit Training Accidental Spills Sheet*

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

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

*- As committed by the applicant, specific recommendations must be provided to pregnant women, lactating women and sexually active subjects enrolled in this clinical trial and on the length of time these recommendations need to be applied. These recommendations must be included in the next version of the IB.*

*- As confirmed by the applicant, recommendations for donating blood, organs, tissues and cells for transplantation and the length of time they must applied, will be included in the next version of the IB.*

*- Regarding the study specific instructions for caregivers that visit patients at home, the notifier is requested to also include instructions for collecting and disposing of waste generated during the visit.*

*- Any protocol amendment must be previously approved by the Competent Authority.*

*- It is the responsibility of the notifier to verify that the study centre has qualified personnel experienced in handling infectious material and that the principal investigator has the required authorizations to perform these clinical trial activities within the hospital (including the laboratory, pharmacy, hospital room, consultation room, etc.) 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 must provide, along with the delivery of a control sample, the detailed methodology protocol for conservation and analysis of the control sample.*

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

*- At the latest six months after the final visit of the last patient included in the trial, the notifier must send the competent authority (attention to the Biosafety Advisory Council) a report detailing the biosafety aspects of the project. This report minimally includes:*

- *o The total number of patients included in the trial and the number of these patients that were 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-9003."*

Hoogachtend,

Frank Vandenbroucke
Vice-eersteminister en
minister van Volksgezondheid
en Sociale Zaken

Zakia Khattabi
Minister van Klimaat,
Leefmilieu, Duurzame
Ontwikkeling en Green Deal