

DG PRE/Afdeling R&D/CTR

IQVIA RDS & Integrated Services Belgium NV/SA

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België

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GMO Dossier B/BE/25/BVW9 (2025-522697-37-00): Phase 1/2 Investigation Of Novel Experimental Regimen in Amyotrophic Lateral Sclerosis (PIONEER-ALS): An Open-Label, Uncontrolled, Multicenter Study to Assess the Safety and Tolerability of Two Doses of VTx-002

[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 12 mei 2026, 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 VTx-002 developed to treat patients with amyotrophic lateral sclerosis, by means of endogenous production of a humanised anti-TDP-43 single chain variable fragment (scFv) in the central nervous system, 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 :

- o Latest version of the ICF
- o Latest version of the Protocol
- o Latest version of the SNIF
- o CAF_non confidential_v5
- o CAF_Confidential_Annex v3
- o Participant Instructions_Participant Hygiene Leaflet V2.0Global_BEL
- o Study Staff Instructions_Pharmacy Manual_V6.0 Belgium
- o Study Staff Instructions_Handling Instruction V3.0Global

- To ensure participants know which effective solutions to use for decontaminating potentially contaminated areas, examples of suitable wipes or disinfectant for cleaning any visible soiling on surfaces should be included in the Participant Hygiene Leaflet.

- As committed by the applicant, the Protocol and the ICF will be updated at the next amendment by adding the recommendation to refrain from donation of blood, organs, tissues, and cells for the rest of their life.

Furthermore, women treated with VTx-002 in Belgium must not donate eggs and men must not donate sperm for the rest of their lives.

- As committed by the applicant, the protocol, ICF and any other relevant study documents will be updated by clearly indicating that participants who are women of childbearing potential (WOCBP), as well as male participants with female partners who are WOCBP, must use a highly effective method of for a fixed period of one year following administration of study drug, applicable to all treated subjects in Belgium irrespective of continued study participation or early discontinuation.

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

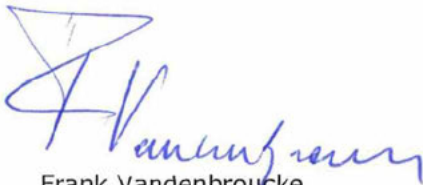
- The notifier is responsible to verify that each study centre has qualified personnel experienced in handling infectious material and that the investigator has the required authorizations to perform the clinical trial activities inside the hospital (laboratory, pharmacy, hospital room, consultation 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.

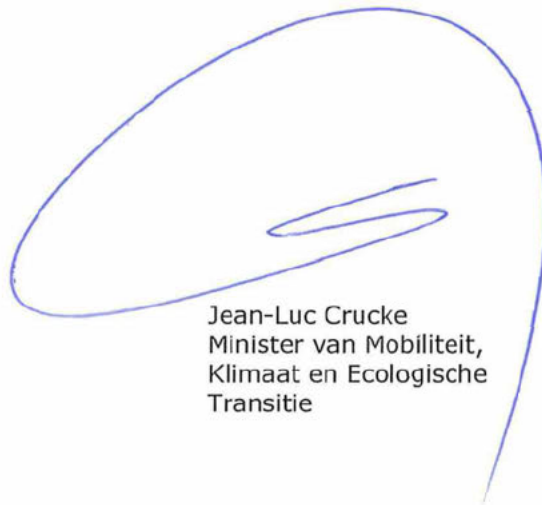
- At the latest six months after the last visit of the last patient included in the trial, the notifier must send to the competent authority at the attention of the Biosafety Advisory Council a report with details concerning the biosafety aspects of the project. This report will at least contain:

- 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 VTx-002.

Hoogachtend,



Frank Vandenbroucke
Vice-eersteminister en
minister van Sociale Zaken en
Volksgezondheid, belast met
Armoedebestrijding



Jean-Luc Crucke
Minister van Mobiliteit,
Klimaat en Ecologische
Transitie