12th June 2023

2023 EU GMO-CTR Survey of GMO-IMPs

The objective of the survey is to understand industry experiences when submitting a clinical trial application (CTA) for a GMO Investigational Medicinal Products (GMO-IMPs) since the Clinical Trial Regulation (CTR, 536/2014) has been in application (that is, since 31st January 2022).

All answers will be aggregated and processed **anonymously**.

Please email **one completed survey per company** to nathalie.lambot@pharma.be and stuart.beattie@biogen.com before 31st August 2023.

Please contact us if assistance is required.

Section 1: Submitting CTAs under CTR (for GMO-IMPs)

Question 1: **Have you submitted a clinical trial application (CTA) or multiple CTAs with a GMO-IMP (or GMO-IMPs) within the EU since the application of the CTR, January 31st 2022 (regardless of whether submitted through the CTR or through the Clinical Trials Directive)?**

Yes/No:

Question 2: If not, have you submitted a CTA with a GMO-IMP in another region of the world (outside the EU) since 31st January 2022?

Yes/No:

Please list countries submitted to outside of the EU (and number of times a CTA submitted to each country):

Answer(s):

Question 3: If you answered yes (to question 2), did any of the following factors influence your decision not to submit a CTA within the EU?

Please select all that apply and please elaborate where possible.

- Complexity / lack of clarity of CTR procedures: □
- Complexity of national/local application procedures (under the CTR): □
- Complexity of local GMO application procedures □
- Complexity of GMO interplay/harmonisation with CTR □
- Other (please specify):

Question 4: If you submitted an initial CTA for a GMO-IMP within the EU, did you submit according to the Clinical Trial Regulation (CTR, 536/2014) via CTIS, or under the Clinical Trials Directive via EudraCT?

Select which applies and how many CTAs were submitted for each:

- CTR: □
- CTD: □
- Both CTR and CTD (for separate CTAs): □

Question 5: If you have submitted a CTA with a GMO-IMP under the Clinical Trials Directive (rather the CTR) was this due to any of the below reasons?

Please select all that appliable and elaborate where possible.

- Lack of harmonization of EU Member States in the field of GMO CTAs: □
- Lack of alignment between the local application process/timelines for the GMO and the procedure for the CTR: □
- Other reason not related to GMO requirements: □
- Other (please specify):

Question 6: If you submitted an initial CTA for a GMO-IMP under CTR, which countries did you select for your study, and why?

List EU countries

Answer:

Section 2: GMO Procedures at National Level

Question 7: If you submitted a GMO package of documents (since 31st January 2022) was the submission and evaluation procedure clear and well-defined at a national level?

Yes/No:

Question 8: If you submitted a GMO package of documents, was the review timeline for the GMO package aligned with the CTR timelines?
Yes/No/Not submitted under CTR:

*Answer (Please specify for **each** national GMO competent authority / liaison office):*

Question 9: If you answered No (to question 8) did the GMO application delay initiation to the clinical trial?
Yes/No:

*(Please elaborate for **each** national GMO competent authority / liaison office)*

If initiation of the clinical trial was delayed, please indicate whether the delay was:
< 1 month
1-3 months
3-6 months
6-12 months
Longer than a 12months

Answer (per national GMO competent authority / liaison office)

Question 10: Please indicate the number of GMO submissions per modality that your organisation has submitted since January 2022.
(Please only enter once per investigational product):

- Gene Therapy IMPs consisting of a Viral Vector intended for direct administration (e.g., adeno-associated viral vector):

- GMO Virus-based Vaccine:

- IMPs containing genetically modified human cells - modified *ex vivo* using a viral vector (e.g., CAR-T cells modified using a lentiviral, or retroviral vector):

- IMPs containing other types of genetically modified cells (e.g., genetically modified bacteria):

- GMO submissions for other IMPs other than listed above, e.g., human cells genetically modified with non-viral vectors:

Question 11: Please provide other comments to your experiences submitting a CTA and GMO package for an GMO-IMP since 31st January 2022?

*Answer (Please elaborate for **each** national GMO competent authority / liaison office):*