Frequently Asked Questions

(Questions received via our EA Mailbox through 02-Dec-2019)

Expanded Access Resource Group

Extension of the Global Clinical Supplies Group

FAQ Tracker

Date Question Received DD-MMM-YY	Question	Response	Date response made DD-MMM-YY	Request from sponsor or vendor
29-Apr-2019	Is off-label use considered as Expanded Access? (Question received at GCSG conference)	At this time off-label use in not considered as Expanded Access. Expanded Access is providing an investigational drug, in general free of charge, prior to the drug's market approval and having reimbursement established. Expanded Access requests (e.g., Single Patient IND) require regulatory approval. The regulatory agency is not required to approve off-label requests. However, the FDA is evaluating if, in rare conditions, if off-label use can follow the Expanded Access paradigm and establishing a process. The FDA is expected to have a decision/process communicated by end of 1Q2020.	29-Apr-2019	sponsor
29-Apr-2019	Is QP release required or not? (Question received at GCSG conference)	QP release is required at the batch level upon importation of the material into the EU. It is not required at a NPU request/shipment level.	29-Apr-2019	sponsor
29-Apr-2019	Why can we not do Expanded Access (EA) in a country we do not plan to register ? (Question received at GCSG conference)	You can do EA in any country that allows EA. However, if you support individual expanded requests or initiate an expanded access program in a country you do not intend to register, you have no exit strategy. Therefore, you will be supporting those patients for life and/or disease progress. In general, expanded access is discontinued in a country once the drug available on the market and reimbursement is established	29-Apr-2019	sponsor

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12-May-2019	I work for a small company and we recently got a Compassionate Use request for a patient in Spain. Here is some additional information: • The product is not approved yet, nor we have clinical trials running in Europe yet. • The investigational product (capsules in a bottle) come from the US and we need to import/export to Spain. I have some follow up questions: • Are there any specific labeling requirements for the investigational product? (I.e. translation, Annex 13 guidelines, etc.) • What are the import license requirements, if any? Who should apply for it: the treating physician? Any other conditions I should be aware of? I have been checking several websites (EMA, GCSG, etc.) to get some additional information, but it is still not clear to me how to proceed. If you can provide me some guidance as soon as possible, I would truly appreciate it!	Spain is one of the easier countries to execute an Expanded Access (compassionate use) request. In summary: For expanded access purposes you are allowed to divert material from an ongoing trial and associate it the expanded access request. Material can only be shipped to a pharmacy and not a private practice. Therefore, your English-labeled bottes are acceptable. However, please be aware that if the material is going home with the patient, you must provide a translated leaflet. The physician/institution must submit the request to Spanish Agency of Medicines and Health Products – (AEMPS). Included in this submission must the name of the drug, packaging presentation, dosage form and number of units provided. Authorization/approval of the AEMPS is for "single use" only. Each approval authorizes a number of medicine packs for a particular patient. When this patient has consumed the packs approved by the Spanish Agency, the hospital will request another authorization to the Spanish Agency. It is recommended that no more than a 3-month supply be provided per shipment. Once the authorization is granted, AEMPS will work with the physician/institution on Importation of the material. Below please find a link to the EAMPs website, section Access to Medicines in Special Situation. Here you will find more specific details/requirement regarding how to support your expanded access request. Access to Medicines in Special Situations Key points from the website: For example, article 5 covers importations and states (via a translator application): **Chapter 1, Article 5. Imports.** When the medicines destined to the uses regulated in this royal decree, require to be imported, such circumstance must be included in the applications provided for in Chapters II and IV.	14-May-2019	sponsor

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		From Chapter Two:
		
		Article 7 outlines the two methods of making drug available:
		• Authorization of an individualized access
		  • Requires 'conformity of the promoter of the clinical trials or of the applicant for the marketing authorization in the cases that require it' (this would be your method)
		• Temporary authorizations for use
		  • Geared toward IMP in advanced stage of clinical research (Group Expanded Access Programs)
		
		Article 12. Obligations of the promoter of the clinical trials or applicant for the marketing authorization.
		The promoter of the clinical trials or applicant for the marketing authorization of the investigational medicinal product, in the access to investigational medicines by patients not included in a clinical trial, will be responsible for:
		• Collaborate with the Agency in defining the conditions of temporary authorizations for use, based on the available results of efficacy and safety.
		• Notify the Agency immediately any information regarding the safety of the medication that could have an impact for the purposes of compassionate use authorizations.
		• c) Notify the Agency of the cases in which express consent prior to the supply of the medication is required.
		• d) Confirm to the Agency the availability of the medicine for patients who meet the conditions of the temporary authorization of use until the moment of commercialization of the medicine or the end of the temporary authorization of use, and guarantee the supply.
			14-May-2019	Sponsor

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16-Oct-2019	Are expanded access trials ***always*** roll-over patients from a previous trial that has shown positive results?	The answer is no. Expanded access trials can enroll new patients that meet the criteria set out in the expanded access protocol. (Note: Companies are not required to set up an EA protocol (new study) at the conclusion of a clinical study. Another strategy to continue to supply patients from a clinical study is to amend the existing clinical protocol to allow patients to continue.)	24-Oct-2019	Vendor