European Federation of Allergy and Airways Diseases Patients' Associations

EFA response to the recommendations of the expert group on clinical trials on the summary of results for laypersons: the perspective of patients with allergy, asthma, and chronic obstructive pulmonary disease

Who are we?

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is an independent non-profit organisation with its central office located in Brussels, Belgium. EFA is a European alliance of allergy, asthma and chronic obstructive pulmonary disease (COPD) patients' associations representing 30% of European citizens currently living with these diseases. Founded in 1991 in Stockholm, Sweden, EFA currently has 41 members in 25 European countries.

As a European patient-led advocacy and action organisation, we strive to involve patients with asthma, allergy and COPD in all European decisions that influence their health. Through our members, we advocate to ensure that the right and access of people with asthma, allergy and COPD to best quality care, to participate in their care, have a safe environment and to live uncompromised lives is guaranteed by EU decision-makers.

What do we do?

- EFA fosters dialogue, exchange and collaboration among its network of national patients' associations to help people with allergy, asthma and COPD understand, be informed about and continuously take part in the EU legislative process through news, briefings, educational tools, position papers, responses to consultations or advocacy materials.

- EFA represents patients with allergy, asthma and COPD at high-level fora, such as the European Medicines Agency Patients' and Consumers' Working Party.

- EFA regularly organises high level events providing opportunities for stakeholders to come together with a patient-centred agenda on issues under discussion, raising awareness and giving visibility to topics that are crucial for people with allergy, asthma and COPD.

- EFA develops and promotes long term partnership with health professionals and scientists, industry and third sector organisations active in health, consumption and environment matters to ensure a holistic approach to policy changes affecting patients' health.

- EFA coordinates the active involvement of patients in research activities, ensuring that patient needs and expectations are met by the project outcomes, and disseminate the project findings to a broader audience and in patient-friendly language.

- EFA co-runs the secretariat of an Interest Group in the European Parliament composed of Members of the European Parliament committed to tackle asthma and allergy in Europe and to help patients relieve their symptoms.

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Our role in the adoption of the clinical trials regulation

EFA, in conjunction with our partner organisation, the European Patients' Forum (EPF), has advocated for an improved clinical trials regulation that results in patients being safer and more involved, as well as in a more transparent process and legislation that supports running 'good trials for good care'. All our actions and previous documents are available online: http://www.efanet.org/medecines-and-clinical-trials/clinical-trials.

Our general views on the recommendations on the summary for laypersons

The regulation makes it compulsory for sponsors of clinical trials to publish within one year after the end of the trial, irrespective of its outcomes, in an EU database the summary of clinical trials' results for laypersons. Annex V of the legislative document specifies the aspects that need to be mentioned in these summaries, but it does not say how much in detail they should be described. For this reason, we are pleased to see these recommendations developed by the expert group on clinical trials for the implementation of the new regulation.

Patients have the unique expertise of living with their disease and therefore their views should be sought at any stage of the clinical trial, from the design to the dissemination of results. Involving people with allergy, asthma and COPD would lead to have a clear and understandable summary of the results by a layperson. This is crucial as, on this basis, patients may also decide on future involvement in other clinical trials and they will get up-to-date information on the latest treatment progress and scientific breakthrough on their disease areas. Indeed, patients who participate in a trial want to know the results of it, the lack of continuous information on trials and their results may even push patients not to participate in trials in future anymore. We therefore welcome the recommendation that suggests clinical trials' sponsors to directly inform patients who have taken the trials, and not only through the layperson summary.

EFA's specific comments

- *Involvement of patients* (point number 4): the document only recommends the involvement of patients and patient organisations in the drafting and review process of the summary of clinical trials' results. We strongly believe this participation should be made compulsory as by doing that, the summary will meet patients', and more in general end-users', needs. Medical writers can of course be considered, but their expertise and knowledge cannot and should not substitute the patients'/carers perspective. Moreover, we encourage the involvement of the patients not only in the summary of clinical trials' results but also in the development of other documents and material that will be used by the patients enrolled in the clinical trials. This was successfully tested in the IMI-funded U-BIOPRED project, where a group of volunteer patients (the Patient Input Platform) reviewed informed consent forms and study protocol for lay language and suggested the inclusion of details which were important to them, such as patient's privacy, possibility to file complaints, potential risks and measures to address them. This helped significantly with recruitment and participant withdrawal. In U-BIOPRED, EFA was also involved in developing lay-updates on the trial for the study participants, as well as exist questionnaire to check satisfaction in the trial. EMA has set up a tried system for patient review of PILs, EPARs as well as safety communication on medicines evaluation. This involves

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confidentiality undertaking, which means that when a document comes under review, the confidentiality is already in place. We do evaluate the system successfully, and it could be taken into consideration as best practice.

- *Readability and comprehensibility* (points number 6 and 8): we welcome all recommendations regarding minimum font size, use of simple language, avoidance of jargon and of any promotional language. In addition, we recommend including headings to present any new element of the clinical trial results. The results' summary should be objective and neutral. Visuals should be used as long as they are easy to understand and not too technical or complicated; in this case, a simple sentence summarising the visual's message could be more helpful. When the clinical trial studied a drug for children, we encourage researchers to include visuals adapted for under 18 readers. The terms of the agreement between the sponsor and the patient have be clear and understood by the layperson, and the patients should receive the signed consensus document to participate in the clinical trial, with parents' consensus in case of children's involvement.

- *Communication of results to participants:* we think feedback to all clinical trial' participants, independently of the results, should be compulsory and timely if there is informed consent to be re-contacted.

- *List of compulsory elements* (annex I): some points are missing here. In particular, nothing is said on which end points were used and why, as this is really very relevant information for example in respiratory disease to understand the trial, and frame expectations. Perhaps this should be included under point 3 (general information on the trial). Under the same point, we would like to bring additional comments. First, we could include substantial modifications and protocol changes made along the way, as well as basic explanations for these adjustments. Moreover, we believe it could be also useful to provide information about future protocol amendments, and the respective explanation for these modifications. Finally, limitations of the study, how potential sources of bias and imprecision were addressed, and caveats are requested for the summary of the results (annex IV of the regulation), but not for the layperson summary. Yet, those elements are equally important for lay readers and could be included under point 8 (comments on the outcome of the trial). In point 4 about population of the subjects, we believe other patients could be motivated to participate in clinical trials if there is more information about the recruitment process, how were individuals contacted and enrolled. We also do believe insurance protection should be considered when clinical trial can provoke direct or indirect damage to patient. Point 5 should provide more information in the type of intake or delivery by medical device of the drug or treatment; as this is a crucial element for patients with allergy, asthma and COPD were many routes are used and that is currently missing in the template. Under point 7 (overall results), we are convinced that aims and results of the study, not only primary endpoints but also secondary endpoints, should be clearly reported. Point 8 should clearly state if results have been positive or negative, to then explain results. We think this differentiation should appear next to the trial title at the beginning of the summary and should become a third possibility of advanced research in the database (clinical trials without results, clinical trials with positive results, clinical trials with negative results). Under point 10 (additional information), it is important to mention links to clinical trials registries, but also to other information available in the European Medicines Agency (EMA) and/or national medicines agencies' websites, possible scientific articles, etc. without being biased. To ease research and understanding, a list of keywords could be

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presented at the beginning of the summary, the list could be linked to the database search facility to allow AND or OR searches. We also recommend sponsors include general contact details at the end of the summary under point 10 for all those readers that would like to go deeper in the results of the clinical trial.

- *Database*: the EU portal in which summaries of clinical trials' results are stored should have an integral glossary that is simple and easy-to-use on the electronic interface and that will help patients understanding some words otherwise difficult to comprehend. Existing patient-friendly glossaries can be used as guidance.

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