Myeloma Patients Europe
Helping patients reclaim a life of possibility

29.01.2026

Myeloma Patients Europe response to 'EMA Reflection paper on patient experience data'

A. General comments

Myeloma Patients Europe (MPE) is a pan-European umbrella organisation representing 52 myeloma and AL amyloidosis patient organisations in 33 countries. As a member of the European Medicines Agency (EMA) Patient and Consumers Working Party (PCWP), and as an organisation regularly involved in EMA activities and assessments, we welcome the opportunity to comment on the 'Reflection paper on patient experience data.'

We commend the EMA for this work and bringing patient experience data (PED) in focus as part of a wider effort to have patient involvement in medicine regulation as an active, meaningful, and holistic process. Patients are the end users of medicines and only by involving them will we understand their impact and value. Given our experience in regulation and PED, we would like to offer the following general and specific comments on the consultation:

1. More clarity is needed on how PED can and should be used in EMA decision making. Whilst the EMA reflection paper has done an excellent job in highlighting the types and ways of gathering patient experience data, there is little consideration of how this is assessed and valued by the EMA in decision-making. There are also no clear examples or EMA case studies set out in the reflection paper of best practice in PED – what do the EMA want to see from different stakeholders to be able to use the data in their decision-making?

2. Whilst we understand that mandating PED might be difficult in every circumstance, we'd like to see a stronger emphasis on the importance of PED to EMA decision-making and stronger language incentivising industry to generate and utilise this data. It should not be optional for companies bringing drugs to market to have PED to support their decision-making and the decision-making of regulatory and HTA bodies.

3. The reflection paper does not acknowledge or address the challenges brought by the new EMA and EUHTA approaches to conflicts of interest (CoI). Whilst we really commend the message that patients should be involved throughout drug development, and understand the difficulties presented by CoI, this is watered down by the sentiment that if patient organisations or patients have any interaction with a pharmaceutical company then they cannot take part in EMA or EUHTA decision-making.

4. Whilst the consultation acknowledges that patient groups have the capability of generating patient evidence and sets out the current ways of patient involvement in the EMA, it overstates the mechanisms available to patient organisations to submit evidence on new medicines (both in scientific advice and in committee decision-making). In the reflection paper, and in the EMA current processes, there is no clear way that patient organisations can seek advice or submit PED as part of an ongoing medicines assessment – unless it is in the general comments sought at the start of an assessment, which are neither product specific nor clear how they are considered. We would like to see clearer avenues set out on how patient groups (1) seek advice from EMA on PED (2) how we submit it (3) what the EMA want to see from patient organisations and (4) how it would be taken into account.

5. There is a lack of specific guidance on how carers are and should be included in PED. Except for paediatric diseases, where the EMA acknowledges the essential role carers have in PED, carers are systematically excluded from mentioning in the reflection paper and from PED generally. They can provide a wealth of information for patients that are not well enough to participate in the collection of PED, as well as unique and intimate perspectives of a patient's Quality of Life that are not captured either by patients or physicians. We'd appreciate guidance on carer data and the value it holds to EMA decision-making.

6. The reflection paper describes the different patient reported outcome measures (PROMs) that can be used to capture quality of life and health related quality of life in clinical trials and real world. What would make these descriptions more useful wouldbe situation specific guidance and case studies on the types of tools that are useful – for example, does the EMA prefer certain tools over others? In CHMP medicines assessments, should industry use both generic and disease specific PROMs? How should industry engage with the EMA and EU HTA on PROM selection. A lot of clear examples and best practice on PROs are hidden in the references and should be mentioned directly, such as SISAQOL-IMI.

B. Specific (line) comments

Line 104: it is unclear what "PED are generated by patients" means here. PROMs and other types of surveys, are generated by patient organisations or industry by involving patients ("from patients" rather than "by patients"). Please clarify the wording in the definition to ensure that it isn't implemented literally.

Line 105: we appreciate that there are avenues to provide individual patients to the decision-making process and to submit general evidence to the EMA. However, there currently is no clear process for advocacy group members (patient organisations) to submit PED in support of a regulatory decision-making process (either as part of scientific advice or a part of an assessment) on a medicine and to have this considered. The reflection paper should make the process clear, if it exists, or we'd appreciate more detail on how the EMA sees the value of PED from patient organisations and how this should be generated.

Line 108: it is unclear what "third-party interpretation" would mean here – this should be clarified. Technically all raw data is analysed by a third party – unless the EMA require raw data?

Line 114: please define the term "applicant" here. Can patient organisations submit and have the data considered?

Lines 125-134: we welcome the inclusion of patient groups in the list of stakeholders that can request support from the EMA. MPE historically has worked with the EMA to pilot specific patient preference methodologies. However, despite mentioning patient groups here, there is no clear way of patient organisations engaging with the EMA, seeking advice or submitting any PED in a systematic way. Most of the listed mechanisms are formal ways industry and larger non-profits can seek advice. How, for example, would MPE formally seek advice from / submit data generated to an ongoing assessment? Clarification on the process would be very welcome.

Lines 157-161: it is our understanding that this process can take a long time, require complex hearings and be costly to undertake. Clear examples of how these processes can be used (case studies) and timelines would be beneficial to set out in the reflection paper to ensure the process is transparent and understandable. In line with the above comment, who can access and use these processes?

Line 158: pleased define what is meant by “innovative methods” in this context. Is this just a methodology different from what might usually be used? In what circumstances should a qualification opinion be sought – again, perhaps setting out case studies and examples could support this point.

Lines 170-172: the text stipulates that “EMA acknowledges the value of PED across all stages of the medicine lifecycle, as the patient’s voice is critical to better informing all stages of a medicine's development, from early development through regulatory assessment to post-marketing activities.” We welcome the recognition of the inclusion of patient’s voice throughout the entire lifecycle of a medicine, but it is critical to point out that EMA and EUHTA current approaches to conflicts of interest contradict this position. The most knowledgeable patients to be involved in regulatory assessment are the ones that are often involved in all stages of a medicine’s development (attending community advisory boards, reviewing protocols, reviewing and completing surveys etc). Yet they are “punished” for this involvement and excluded from regulatory assessments. Involving patients with less experience and knowledge may not support the EMA decision-making in the correct way.

Lines 176-177, Table 2: “Non-clinical research” section should also include impact on carers and established preferred location in addition to preferred administration route; “Clinical trials design” section should also include “Administration” (ensuring the route, length, location of treatment administration are accessible to patient), treatment schedule (i.e. how many hospital visits etc) and accessibility of testing schedules (e.g. number of bone marrow biopsies etc); “Regulatory benefit-risk assessment and decision making” section, in defining most relevant clinical outcomes, should also include patient preference data; “Access and use in clinical practice” section, we believe that would be important to include a point about the importance of PED to help frame decision-making on medicines. Quantitative and qualitive insights can help provide a "human" context and experience to a decision-making problem and the data being considered, defining how a medicine will impact on people in the real world.

Lines 199-200: the concept of “proxy-reported outcomes” is introduced here without much detail or context. Clarity and detail should be provided (or examples) of whether this is something the EMA would accept or has accepted and, if so, how is its importance assessed.

Lines 209-214: it would be useful here to include details on what is the best practice regarding the use of general vs specific disease tools vs symptom specific tools. What helps the EMA decision-making in this regard?

Lines 249-253: we suggest including here examples of where PPS have actually been used in submissions and decision-making. Perhaps they could be highlighted as "lessons learned". For example, we understand there were strong case studies involving patient preference evidence involving alopecia medicines which would be beneficial to highlight.

29.01.2026

Line 261: we feel that the description of qualitative data as “explorative” is diminishing the value of this type of data. It is not a fair reflection of the different methods available to generate and analyse this type of data and the fact they are a rich source of information from patients. Whilst we understand that the EMA find qualitative insights difficult to incorporate systematically and robustly in their decision-making, they can help frame and provide context to decision-making and provide clear insights from the end-users of medicines. We think this section, and the PED reflection paper generally, would benefit from an acknowledgement of this.

Lines 293-299: while these engagement activities and methodologies are to be commended, and MPE regularly gets involved, these are not systematic (for example scientific advisory group meetings are done on an ad hoc basis), are often not specific to a treatment being assessed (the form sent to patient groups at the start of an assessment is not product specific, require general disease information) and are also being hampered by an overly strict conflict of interest policy which prevents many patients from participating. We understand the challenges of the EMA in this regard, but we’d like more detail in the reflection paper on the value of PED from patient organisations, how it can be submitted and the impact it might have.

Lines 320-322: to our knowledge these methodologies are not regularly used in medicines assessment. The EMA should specify under which conditions are these methodologies used, perhaps using examples of the impact this has had.

Lines 334-342: it would be useful to include examples for how does CHMP-arguably one of the most important committees- (or the future CHMP under the new pharmaceutical legislative package) use input from individual patients and organisations through surveys.

Lines 412-413: the content here is short-sighted. The consultation is only very explicit when it comes to PROMs in Phase III trials, which we understand but there is value in generating this data earlier on in trials (if not for regulatory purposes). Collection of PROs should be encouraged at least from phase II trials, especially if they are suitably large and / or form part of a larger clinical development programme. If the EMA set this out explicitly, there is the chance that industry will not generate QoL data outside of Phase III clinical trials.

Lines 469-479: the use of social media as a source of data can be problematic. From an ethical point of view, patients’ data is used without their consent and out of the context from which it was intended. From the point of view of accuracy, the media used to express the opinions might influence the opinions themselves. Perhaps it would be prudent for the EMA to explore the role of this data further and the ethical issues associated with it, before setting it out its use.

Lines 575-577: Firstly, in the discussion around CoI, this section confuses PED with standard ways of involving patients in EMA decision-making. This would benefit from more detail and strengthening. We understand the CoI policy set out for patient involvement in the processes, but does this extend to the PED generated by patient groups? By it’s nature PED is generated from patients, and the methods / analysis can overlap with CoI issues.

We also know that there are many differing opinions on the value of PED, the robustness and the impact it can have on decision-making. The perceived lack of value is outlined in this section, but the comments are too general and do not help strengthen the reflection paper. In our experience, most HTA bodies in Europe and stakeholders agree on the value of PROMs as a robust way of generating QoL data and to input into decision-making. The perceived lack of value of PED in most cases does not come from a lack of validation from HCPs, it is more nuanced than this and this should be reflected – including a lack of understanding of the value and how it is generated, misperceptions on CoI in patient advocacy and industry, variation across actors / disease areas to get this data and fears about having to amend decision-making processes to take it into account systematically. We believe this section should be rewritten to better reflect the differences in stakeholder opinion and how they can be addressed.