

## *Conclusions*

As of today, the European Commission has no plans to revise the legislation in order to propose a harmonised approach in the implementation of Compassionate Use schemes. However, the current situation could be improved by adopting guidelines on Compassionate Use. In addition, the European Commission could prepare an inventory of Member States practices to describe how Compassionate Use is applied in the different Member States.

There is a need to set up a working group led by interested representatives of national competent authorities, to propose guidelines on CU programmes. This would be an informal step to assess what can be done at a higher level. We can cite as an example, the experience of the Clinical Trials Facilitation Group (CTFG) who has worked on the harmonisation of procedures and has now moved on to the evaluation.

Transparency is an issue that has been raised several times. This is a challenge that we need to take up. It is difficult to gather comprehensive information about the named-patient basis CU programmes, however, for cohorts, it is possible to ensure greater transparency in the process and to have the involvement of patients. At the end, Member States should notify the EMA in a more systematic fashion when they authorise a compassionate use programme (for a group of patients).

Greater involvement of patients is necessary. We have seen that the legislation gradually takes patients more into account. The next step will be for the pharmacovigilance committee to include patient organisation representatives. The last step will probably be the presence of patient organisation representatives at the CHMP, when the legislation will be changed. It is important that patient representatives can give their opinion on the relevance of a compassionate use programme when the CHMP is asked for an opinion.