European Federation of Pharmaceutical Industries and Associations

10 March 2014

EFPIA policy statement on Clinical Trials Regulation

The European Federation of Pharmaceutical Industries and Associations (EFPIA) welcomes the political agreement reached by the European co-legislators, with the aim of a rapid adoption of the new Regulation on Clinical Trials. However, the original objectives of this legislation should be remembered: These were to enhance efficiency in the Clinical Trials Regulation process and, in turn, to boost the EU's competitiveness as a place to conduct research and make for more efficient patients access to new innovative treatments. EFPIA believes that some of the initial objectives of the legislation have been only partially achieved; therefore the success of this legislation strongly depends on how it will apply in practice.

When establishing further legislation and procedures at European and at national level, it will be essential that relevant stakeholders will have the opportunity to provide input for remaining questions and concerns. This is necessary to ensure that the legal revision achieves its objectives of establishing a streamlined and a more harmonised system in the European Union.

The new Regulation includes a number of provisions that will allow for a better coordinated and more efficient approach, which will be directly binding to Member States and sponsors. Key advances include a single submission of a clinical trial application to the future EU portal and database, and a defined and coordinated assessment procedure integrating review by competent authorities and ethics committees. The outcome of this review leads to a single administrative decision per Member State. Additionally, the provisions to ensure safety and integrity of clinical trials participants have been further clarified

While the legal framework provides for the possibility to apply extended assessment timelines, it is now up to each Member State to establish processes and practices with timelines, which can still be considered competitive compared to other regions. EFPIA calls on Member States to consider the impact of timelines on the overall attractiveness to conduct clinical research both in each Member State and in Europe as a region.

In detail, EFPIA calls on the European Commission, the European Medicines Agency and the Member States to address the following points during the implementation phase:

- **The significant extension of timelines for the assessment procedures:** This includes a maximum timeline of 156 days in the case of clinical trials for Advanced Therapies Medicinal Products (ATMPs) and biologic medicinal products, and an additional 52-83 days for the inclusion of a new Member State in the trial. If these timelines are applied on a routine basis, the European Union (EU) could have some of the longest timelines worldwide. Clinical trials sponsors' experiences using the Voluntary Harmonised Procedure (VHP) demonstrate that, on average, 56 days are sufficient to assess a trial[1]. Future guidance should stipulate that although maximum timelines are specified in the Regulation, in order to maintain European competitiveness in clinical research, Member States should endeavour to complete the assessment process within a shorter timeframe. This is particularly relevant for single-country trials, addition of new Member States.
- **Cooperation of ethics committees:** The lack of a greater cooperation of ethics committees: Provisions on greater collaboration between ethics committees would improve the pace of approval of a trial as well as provide the potential for networking, collaboration and convergence of standards

[1] Dr. Hartmut Krafft, Chair CTFG and VHP-Coordinator, *The Voluntary Harmonisation Procedure: Where are we now?* Presentation, BIA, London, October 2013. Available at: http://www.bioindustry.org/document-library/dr-hartmut-krafft/

in the field; collaboration between ethics committees, such as through the creation of ethics committee networks, should be encouraged during the implementation phase.

- **Opt-out mechanisms:** The significant broadening of the reasons for opt-out from coordinated Part I assessment: The use of the opt-out reason referring to "safety and data reliability and robustness considerations" will have to be carefully monitored to avoid disharmonised approaches at the detriment of patients, investigators and sponsors.
- **Substantial modifications:** The Regulation provides for a complex process for substantial modifications and introduces additional restrictions on the timing of submission of amendments. As with the initial submission, EFPIA calls on Member States to apply flexible timelines for the assessment.
- **Transparency provisions:** Clarification is needed in terms of which elements of the EU database should be considered confidential at which point in time.
- **Notifications:** The Regulation contains new provisions for declaration of start and end of trial, e.g. of start of trial in the Member State (MS), First Patient Visit in the MS, end of recruitment in the MS and end of trial in the MS, in all EU MSs and all countries worldwide. A clear and simple notification process via the European portal will have to be put in place, leading to minimal administrative burden and avoiding duplication of work for sponsors and thus avoid this provision becoming an administrative burden.
- **Safety reporting provisions**: Clarity to ensure a harmonised approach, in particular what information goes to investigators and ethics committees, annual reporting and presentation of data. Furthermore, safety reporting must be fully aligned with existing pharmacovigilance rules.
- **Functionality of EU Portal and Database:** Clear timelines and milestones need to be fixed by the European Commission, the EMA and the Member States in consultation with relevant stakeholders, in order to avoid any delay in application of the new legal framework. It is important that all parties affected have sufficient time to interpret all available guidelines to up-date their processes and systems. Early stakeholder consultation will facilitate implementation.
- **Review of experience with legislation:** To be able to monitor the application of the legislation, the Database need to deliver the metrics needed for the assessment in a "real time" modus as to ensure discussion and practical adaptation where possible, but also in preparation of the 5 years review. The future Clinical Trials Advisory Group CTAG should take a key role in monitoring the functionality of the Regulation of such metrics and this should be vested in the rules of procedures.

EFPIA and its member companies, as the largest contributors to clinical research in Europe, stand ready to provide the necessary input in order to assist in facilitating a Regulation that meets the original objectives.

About EFPIA

The **European Federation of Pharmaceutical Industries and Associations (EFPIA)** represents the pharmaceutical industry operating in Europe. Through its direct membership of 33 national associations and 37 leading pharmaceutical companies, EFPIA is the voice on the EU scene of 1,900 companies committed to researching, developing and bringing to patients new medicines that will improve health and the quality of life around the world. EFPIA supports a vision of modern and sustainable healthcare systems in Europe, where patients have equal and early access to the best and safest medicines, which supports innovation, empowers citizens to make informed decisions about their health and ensures the highest security of the medicines supply chain. **For more information on EFPIA and Clinical Trials, please visit our website www.efpia.eu**