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The Pharma View of the EU Clinical Trial Regulation

6TH CONFERENCE ON CLINICAL TRIALS IN THE NORDIC COUNTRIES

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Pfizer

25th October 2017

Objectives

- Overview of Industry's Key Priorities
- What keeps us up at night
  - Implementation – when?
  - Portal/Database – Fit for Purpose?
  - Transitioning of trials
  - GMO and Medical Device legislation
  - Advances in trial designs
  - Brexit

About EFPIA and CTR 536/2014

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 40 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 sees the implementation of the Clinical Trials Regulation as an opportunity to demonstrate Europe's commitment to clinical innovation, scientific collaboration and transparency of clinical trials information. Successful implementation of EU CTR is one of EFPIA's priorities."

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Industry's Key Priorities

- **Consistency in approach / predictability**
- **Maintain EU's competitiveness**
- **Efficient & user-friendly IT systems**
- **A future-ready system**
- **Disclosure of clinical data from EU Portal & Database**
- **Everything in place prior to implementation**

Implementation Date

Update on the EU Clinical Trial Regulation and development of the clinical trial EU Portal and Database

T he Board heard an update on the EU Portal and Database project. The development is progressing, though still requires close monitoring. More precision of the delivery timeframe will be possible after a planned cycle of extensive testing by Member States and sponsor representatives and when further progress with the auditable version of the system has been made. The development remains aligned to the schedule that enables the EU Clinical Trial Regulation to come into application in the second half of 2019.

Ref: EMA Management Board Press Release, 6-Oct-2017

- **Delays – disappointing!!, but:**
  - EUPD must facilitate operation/function of EU CTReg
  - Welcome sponsors' involvement in UAT

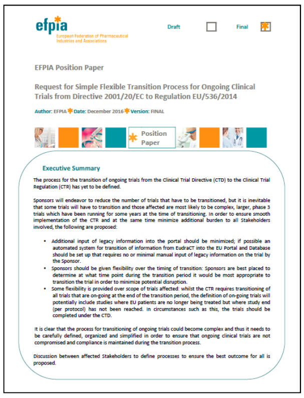
Additional EUPD Development Priorities

- **Availability of a test/dummy system**
- **Upload of externally built CT application and application form**
- **Completion of implementation of User Management approaches**
- **Search and reporting functionality**
- **Download of documents**

Transitioning Trials

Large companies: ~80 trials need transition

- **Simple, Flexible, Process to transition Ongoing Trials from Directive to Regulation**
  - Additional input of legacy information minimized and automated
  - Flexibility over the timing of transition
  - Flexibility on scope of trials affected
    - E.g. EU LPLV reached but study end (per protocol) has not been reached – Out of scope



Draft Final

EFPIA Position Paper

Request for Simple Flexible Transition Process for Ongoing Clinical Trials from Directive 2001/20/EC to Regulation EU/536/2014

Author: EFPIA Date: December 2016 Version: FINAL

Position Paper

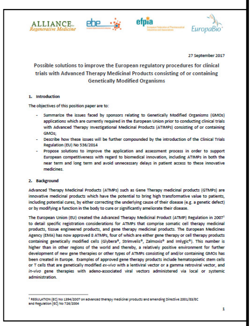
Executive Summary

The process for the transition of ongoing trials from the Clinical Trial Directive (CTD) to the Clinical Trial Regulation (CTR) has yet to be defined.

Sponsors will endeavor to reduce the number of trials that have to be transitioned, but it is inevitable that some trials will have to transition and those affected are most likely to be complex, larger, phase 3 trials which have been running for some years at the time of transitioning. In order to ensure smooth implementation of the CTR and at the same time minimize additional burden to all Stakeholders involved, the following are proposed:

Links with other Legislation

- **New Medical Device Legislation**
  - Inter-operability between MedDev clinical database and EUPD
  - 'Clinical performance' study for diagnostic-drug combination – CTReg or IVDReg?
- **Genetically-Modified Organisms**
  - Case-by-case evaluation of risks
  - Data requirements, timelines, processes vary by MS
  - Need integration of risk assessment process with CTReg processes
    - Other steps to take in short/medium-term



27 September 2017

Possible solutions to improve the European regulatory procedures for clinical trials with Advanced Therapy Medicinal Products consisting of or containing Genetically Modified Organisms

1. Introduction

The objectives of this position paper are to:

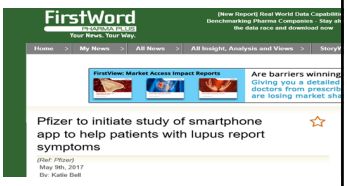
- Summarize the issues faced by sponsors relating to Genetically Modified Organisms (GMOs) applications which are currently required in the European Union prior to conducting clinical trials with Advanced Therapy Investigational Medicinal Products (ATIMPs) consisting of or containing GMOs;
- Describe how these issues will be further compounded by the introduction of the Clinical Trials Regulation (EU) No 536/2014
- Propose solutions to improve the application and assessment process in order to support European competitiveness with regard to biomedical innovation, including ATIMPs in both the near term and long term and avoid unnecessary delays in patient access to these innovative medicines.

2. Background

Advanced Therapy Medicinal Products (ATMPs) such as Gene Therapy medicinal products (GTMPs) are innovative medicinal products which have the potential to bring high transformative value to patients,

Future-Ready System?

- **Clinical trial designs are evolving**
  - Electronic data capture, and prevalence of wi-fi connectivity driving change in conduct/analysis of trials
  - Remote data capture
  - Adaptive trials, combined phases, bucket/platform trials
- **Is there flexibility in CT Regulation to facilitate these approaches?**



Brexit

- **Short-term impacts:**
  - UK-based sponsors/legal representatives
  - CT supplies released through the UK
  - Inspections
- **Longer-term considerations**
  - UK involvement in CT Regulation

CTR Implementation – Industry Summary

- **CTReg – Golden opportunity:**
  - Balanced approach to access data,
  - Protect research
  - Support global drug development objectives
- **Stakeholders to embrace and work with new processes and new thinking**
- **Ensure we get it right:**
  - User-friendly systems
  - Bureaucracy reduction:
    - Flexible approaches
    - Inter-operability
  - Adaptable to scientific/technical advances
  - Embraces changing macro environment

Thank you