Platform of Plasma Protein Users

REVISION OF THE CLINICAL TRIALS DIRECTIVE 2001/20/EC

EUROPEAN COMMISSION CONCEPT PAPER CONSULTATION

PLUS is the platform of plasma protein users representing patient organisations including the Alfa-1 Federation Europe, the European Haemophilia Consortium, the GBS/CIDP Foundation International, the International Patient Organisation for C1 Inhibitor Deficiencies (HAEI), the International Patient Organisation for Primary Immunodeficiencies (IPOPI), the ITP Support Association, and the World Federation of Haemophilia (WFH).

Collectively these seven organisations represent the views of 80,000 known patients in Europe and they represent persons with conditions with a probable European prevalence of over 650,000. These patients are affected by *rare plasma related disorders*. Clinical trials for these rares conditions are very often difficult to perform due to the small patient populations and the thinly spread medical expertise.

PLUS has followed with great interest the developments around the revision of the Clinical Trials Directive and welcomes the European Commission's concept paper consultation.

PLUS member organisations actively collaborate in the development of clinical trials on rare plasma related disorder in order to contribute towards the production of high-quality knowledge on diseases and development of effective and safe treatments. The involvement of patients in EMEA committees such as COMP or CPWP is a testimony to the importance of taking the patients' viewpoints and expertise into account when discussing such issues. PLUS firmly believes that legislative and regulatory decisions that will affect the lives of patients should always be taken having included patients from the start in the decision-making process.

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PLUS welcomes the preliminary appraisals and preferred options outlined by the Commission in the concept paper and does not wish to comment on all of them. PLUS however would like to stress specific points on the following consultation topics:

- Consultation items 1-3 – Cooperation in assessing and following up applications for clinical trials

  PLUS welcomes the proposal from the Commission to establish a single submission approach followed by a coordinated assessment procedure which would allow for a joint assessment with a practicable committee structure. Currently, the lack of harmonization in dealing with ethical committees, different national requirements for assessments, and even local differences makes the entire process of conducting a clinical trial a very expensive, lengthy procedure. As a result the numbers of clinical trials are decreasing and are being conducted outside the European Union.

- Consultation item 10 – 2.1.2 *Excluding clinical trials by 'academic/non-commercial sponsors' from the scope of the Clinical Trials Directive*

  PLUS fully agrees with the Commission's preliminary appraisal that rather than limiting the scope of the Directive, it would indeed be more appropriate to have harmonized and proportionate requirements for clinical trials, independently of the nature of the sponsor. In the field of rare diseases, the academic/non commercial sector plays an important part in stimulating research, which the commercial sector may not always necessary support. PLUS believes that the nature of the sponsor should therefore not be a criteria for exclusion of the Directive

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- Consultation item 17 – 3. Ensuring compliance with good clinical practices in clinical trials performed in third countries

  PLUS fully supports that any disregard of the rules that protect patients participating in clinical trials calls for determined action. PLUS agrees that particular attention should be paid to third country clinical trials where data is submitted in the EU in the framework of the authorisation process of clinical trials and medicinal products. In the field of rare disease where expertise may be geographically spread the application of the proposed European Commission appraisal is particularly relevant.

PLUS would welcome a dialogue with European Union policy makers to further discuss these aspects in more detail.

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