EGAN
European Genetic Alliances' Network

Consultation Response

Revision of the 'Clinical Trials Directive' 2001/20/EC
Concept paper submitted for public consultation

Response by European Genetic Alliances' Network

The European Genetic Alliances' Network is an alliance of national genetic alliances and European disease specific patient groups with a special interest in genetics, genomics and biotechnology. We work for a voice in research and health policy and seek a world in which genetic diseases are understood, effectively treated, prevented and the affected people supported.

We welcome this review and the opportunity to respond.

1.1 Single submission with separate assessment

Consultation item no. 1: (Single EU Portal)

EGAN supports single submission through an EU portal. This will relieve applicants of burdensome and costly collation of documents for each competent authority, and will provide a route towards standardisation of required documentation.

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Consultation item no. 2: (Separate assessment)

EGAN agrees with this appraisal: that subsequent separate assessment would not solve any of the issues that have triggered this review.

1.2 Single submission with subsequent central assessment

Consultation item no. 3: (Central assessment)

EGAN disagrees with this appraisal.

A streamlined central assessment of the risk-benefit component of multinational clinical trials will produce consistent assessment results across the EU.

The concept paper states that a central assessment would insufficiently take account of ethical, national and local perspectives. EGAN agrees with this point, but does not believe this is a barrier to central assessment. Ethical decision making should remain a competence of Member States as public opinion on ethical issues varies widely across the EU.

In the majority of Member States, ethical decisions are made by a separate body from that which carries out technical appraisals. Therefore change to the ethical decision making structures currently employed by Member States would not be necessary.

Similarly, national and regional issues can only be addressed at an appropriate level. The advantage of a central scientific assessment of the risks and benefits of a trial would be that all ethics committees and all regional and national decisions are made on the basis of consistent, agreed science. Scientific analyses would not be carried out multiple times by multiple competent authorities. Applicants would not have to satisfy the questions of multiple competent authorities. The quality of this information would be improved by central assessment, as the personnel carrying out the scientific analysis can be pooled.

The concept paper states that the volume of clinical trials would make centralised assessment difficult. EGAN believes this methodology should only be applied to multinational clinical trials. Alternatives to this system (separate assessment or co-ordinated assessment procedure) require competent authorities in Member States

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hosting a trial to either carry out a full appraisal themselves, or to examine another Member State's appraisal to decide whether to disagree with the assessment report; both are significant duplication of work. Central assessment is by definition more efficient than other methodologies.

The concept paper states that the involvement of all Member States is not needed, as few multinational clinical trials are rolled out in more than five or six states. EGAN agrees with this point. EGAN does not believe a body resembling the Committee for Medicinal Products for Human Use (CHMP) will be necessary to oversee every decision by a central assessment body. It may be decided that such a committee structure may be necessary to oversee the work of such a body, but EGAN believes the main body of work can be carried out by a combination of a secretariat body, similar to the European Medicines Agency (EMA) if not the EMA itself, and appointed expert scientists, most probably seconded from national competent authorities. The bulk of decisions would be made by groups that are more like the working parties of the scientific committees of the EMA than the scientific committees themselves.

Member States would have input to the composition of the decision making bodies, but not participate in each and every decision.

A means to further streamline this body could be to operate on a virtual basis, whereby the majority of scientists would remain at their Member State competent authority and work remotely.

A structure such as this would provide a framework (alongside existing pharmacovigilance structures) for the central portal philosophy to continue for safety reporting.

The concept paper states that the costs and fees involved would make a central assessment unattractive for academic researchers. EGAN believes there should be a reduction of fees for academics if this does prove to be the case.

1.3 Single submission with a subsequent 'co-ordinated assessment proceduce'

Consultation item no. 4: (Catalogue of the three components of a clinical trials application)

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EGAN believes this catalogue is complete.

Consultation item no. 5: (Restriction of the scope of the CAP to risk-benefit assessment)

EGAN agrees that any assessment at a level higher than Member States should only be concerned with risk-benefit analysis. Ethical issues and national and regional issues should remain Member State competences.

Consultation item no. 6: (Disagreements amongst Member States about the assessment done under CAP)

EGAN believes this question illustrates a central problem with the concept of the Co-ordinated Assessment Procedure (CAP). Inevitably Member States will have different analytical methodologies and differences of opinion will arise. Any of three resolution solutions presented in the concept paper will slow down the process of approval and may lead to the applicant having to apply separately in the disagreeing Member State(s).

EGAN is grateful for the opportunity to comment on this concept paper.

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