



WORKSHOP ON CLINICAL TRIALS

Shaping the future of European Clinical Trial Legislation

**22 April 2009
Diamant Conference Centre
Brussels, Belgium**



Report written by Pete Wrobel

SHAPING THE FUTURE OF EUROPEAN CLINICAL TRIAL LEGISLATION

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This event was kindly sponsored by: Celgene, CSL Behring, ERYtech Pharma, European Federation of Pharmaceutical Industries and Associations (EFPIA), GlaxoSmithKline, Merck-Serono, Novartis Pharma

Supporting Organisations: European AIDS Treatment Group (EATG), European Biopharmaceutical Enterprises (EBE), European Forum for Good Clinical Practice (EFGCP), European Organisation for Research and Treatment of Cancer (EORTC), Irish Platform for Patients' Organisations, Science and Industry (IPPOSI), Rett Syndrome Europe



Cover picture : David Haerry, Ingrid Klingmann and Patricia Pellier, Co-Chairs of the Workshop



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Organizing Committee co-chaired by:

David H.-U. Haerry
EATG
representing Patients

Ingrid Klingmann
EFGCP
representing Science

Patricia Pellier
Merck-Serono
representing Industry

A workshop on clinical trials held in Brussels, 22 April 2009

When the Clinical Trials Directive came into force in 2004, it arrived with dire warnings. Many said it would bring about the end of clinical research, especially for academia. Five years on, the Directive is still here, and clinical trials are still here. True, there are also problems, and in a meeting in Brussels on 22 April, stakeholders from patients' organisations, academia, industry, regulators and policy makers came together under the auspices of EPPOSI to look at some hard data about the clinical research landscape in Europe, and to consider what changes might need to be made to the Directive.



N. Dedes

It was a day of two halves. In the first part, delegates heard presentations from the European Commission, from the Clinical Trials Facilitation Group, from industry and from patients. The centrepiece was the much-awaited first public – albeit preliminary – report of results from the ICREL project, which is attempting to measure the impact of European legislation on clinical research.

In the second half, delegates split up into a range of workshops to come up with specific proposals. They then came back together to agree on a small number of key suggestions for policy action.

This report outlines the discussion and the points made in the first half of the day, and concentrates then on the recommendations to emerge from the plenary and break-out discussions.

The results are put forward as a tool for the Commission and stakeholders to use in the continuing discussion of how to advance Europe's conduct of clinical trials.

The position now

The focus of the day was naturally on the Clinical Trials Directive. But as Stefan Führung from the European Commission's Directorate Enterprise and Industry pointed out, the implementation of a Directive is not just about one set of text. It links in with other legislation, such as the Advanced Therapies Regulation¹, and it depends also on guidelines that give a detailed steer to stakeholders on how to implement it and the transposition in 27 Member States.

Führung presented delegates with an overview of where things currently stand. The Commission, he said, is evaluating comments with EMEA, the European Medicines Agency, on Good Clinical Practice for advanced therapies². The guidelines for legislation on paediatric research need updating, he said, in particular relating to the Paediatric Investigation Plans³. The EudraCT Application Form⁴ for clinical trials has been getting longer and longer – but it will have to grow longer still, he said. The idea now is to restrict the information required in the form to that which will support its aim: to be a means whereby national authorities can see where similar trials are happening.

Certainly, transparency has been improved, or will be, with the decision to make accessible parts of EudraCT, the clinical trials database. The Commission has now instructed EMEA to change the underlying programme to make some fields public. It sounds simple, said Führung but he warned that it is a complicated matter to achieve in practice.

The Commission is working on revision of the guideline for clinical trial applications, substantial amendments, and declaration of the end of a trial, with an impact assessment planned to start before the end of the summer. That introduces the opportunity for greater harmonisation – which legislators are calling for – but “it hasn't happened yet”, cautioned Führung.

1. http://ec.europa.eu/enterprise/pharmaceuticals/advtherapies/advanced_en.htm
2. http://ec.europa.eu/enterprise/pharmaceuticals/advtherapies/advanced_keydoc.htm
3. <http://www.emea.europa.eu/htms/human/paediatrics/pips.htm>
4. <https://eudract.emea.europa.eu/eudract/index.do>

The Commission is aiming to conclude its evaluation and impact assessment of the Directive in 2010 – but Führung warned that the process might not automatically lead to new legislation. In any case, a decision on whether to review the Directive will not be taken by this Commission. For that, we will have to wait for the new Commission, perhaps until 1 January 2010.

Führung concluded with two points. First, the importance of a harmonised approach across Europe, given that 60 per cent of clinical trials are multinational. Second, the need to see whether the rules are adapted to risk. “Not all clinical trials pose the same risks to the patient, and it is important to give some flexibility to take account of



S. Fuehring

this,” he said. “Regulatory requirements should be proportionate to the risk involved, regardless of whether the trial is ‘commercial’ or ‘non-commercial’.”

With most trials involving more than one country, great things are expected of the Voluntary Harmonisation Procedure⁵ (VHP). Hartmut Krafft, Chair of the Clinical Trials Facilitation Group (CTFG), which devised the VHP, took delegates through its three phases.

First, the sponsors ask for a trial to be part of the procedure. If the CTFG agrees, it identifies the relevant National Competent Authorities, the organisations responsible for the regulation of medicinal products in Europe. In the second phase, the authorities review and assess the application within 60 days, with administrative coordination from the VHP coordinator. Finally – if all has gone well – a formal Clinical Trial Application is sent to the National Competent Authorities, for a quick decision: within 10 days.

5. http://www.hma.eu/uploads/media/VHP_public_CBB_22_Dec_08_hk_jan12.pdf

That’s the theory, but so far, with only two months’ experience of the VHP by the time of the workshop, it’s too early to say how it is working. One critical question is whether everyone sticks to the principle that, if no problems are raised in the scientific assessment (the second phase), they shouldn’t come up during the final phase.

Is such speed always a good thing? Michael Bone from the UK Association of Research Ethics Committees referred to what he called “too hurried” assessment of the disastrous TeGenero trial at Northwick Park Hospital in 2006. He was concerned that “in the drive to be efficient, we may be throwing the baby out with the bath water and not protecting the patients”. Krafft disagreed: “Through the VHP we are increasing the critical mass of scientific assessment by bringing countries together.” And a lot of time was taken with the TeGenero trial, he said.

One thing that did stand out was the absence of patient organisation involvement in the VHP. Would it help or hinder the efficiency of the process? Krafft said he had never experienced patient organisations being involved in the approvals process, and it is not foreseen at present – but he is “open for proposals”. Nikos Dedes from the European AIDS Treatment Group, who had opened the workshop, said it is important to identify opportunities for involving patient organisations. With a nod in the direction of Führung’s earlier comments about risk, he said it is crucial to any risk assessment to get the opinion of patients – “and ditto when decisions go to ethics committees”.



H. Krafft

Optimistically, Katarina Edfjäll from Celgene, Switzerland, asked whether the CTFG is trying to launch a harmonised procedure for ethics committees (many see the current totally unharmonised system as a major stumbling block). No, said Krafft: “Ethics committees will not be happy to be told by Competent Authorities how they should work.” The initia-

tive, he said, must come from the ethics committees. “So we’re not trying to stimulate this,” he said, though he added, “But we are open to it.”

Patricia Pellier from Merck-Serono, Switzerland, in a summary of industry’s assessment of the clinical trials procedures, pointed to highly varied experiences with Competent Authorities and ethics committees. There are, for example, different interpretations of what constitutes an innovative medicinal product, or a substantial rather than a non-substantial amendment, and the regulatory frameworks for drugs, devices and diagnostics differ in many respects.



P. Pellier

Add to this factors such as different modes of submission for Competent Authorities and ethics committees, different timescales and different reporting requirements, and it’s no wonder that industry welcomes the VHP initiative even though the procedure does not apply to all trials. Pellier recognised that “it is always difficult when you have a new challenge”, and added that although the VHP is not binding, “we must ensure that both Competent Authorities and industry play the game”.



D. Haerry

David Haerry from the European Aids Treatment Group summed up patients’ experience with the Directive. What works? There is, he said, more transparency, more harmonisation, and more rights to protect patients in commercial as well as non-commercial trials. Even though academia has to cope with a greater administrative burden, he said, the Directive has brought about more consistency in independent investigator-driven trials.

What doesn’t work? Haerry highlighted a lack of transparency for patients and the general public once trials get going – and also when they end. He also noted that the Directive does not cover non-interventional clinical trials. Above all, he was unhappy at the lack of what he called “the European ethical dimension”: there are no harmonised standards, and ethics committees lack coordination and training. “What are ethics committees made for?” he asked. “Are they really there to protect the patients? I have doubts sometimes when I see the overall position across Europe.”

Haerry made a particular point about informed consent. It is intended for the protection of clinical trial subjects, yet there are wide differences across Europe in terms of the quality and quantity of information provided – in one case, 27 pages of university-level language. “I cannot imagine how informed consent is established with a patient joining such a trial,” he said.

The highlight of the morning, though, was Ingrid Klingmann’s presentation of provisional results from ICREL, a Commission-funded project to look at the impact of European legislation on clinical research.

Measuring the impact is a mammoth task, said Klingmann, and not one that can be done directly. What the project had done was to look at a number of indicators over the period from 2003 to 2007 – numbers of studies, amendments, numbers of patients, sites, clinical trials applications and so on, as well as costs, timelines and the number of non-approvals.

The task was not helped, either, by the difficulty the ICREL team had in getting the data it needed – not only from Competent Authorities, companies, investigators and ethics committees but also from the EudraCT database. Nonetheless, a picture has emerged.

Full details of the study will be published elsewhere, and certainly the figures need careful analysis – for example, there is a spike in applications to some national Competent Authorities in 2004 that is almost certainly due to sponsors rushing in applications so as to avoid having them come under the new (and at the time unpredictable) framework.

The predicted collapse had not occurred – apart, that is, from a big fall in non-commercial trials in the UK. But neither, said Klingmann, had there been a perceptible increase in the number of trials. Within the figures, there has been a rise in multinational trials (a long-term trend), and a fall in trials conducted in one country alone.



I. Klingmann

Among the mass of data, three points stand out very strongly.

- The first is that there has been a sharp increase in the number of substantial protocol amendments submitted for approval – up from 40,000 in 2003 to 100,000 in 2007. This, as Klingmann pointed out, is a major overhead for trial sponsors.
- The second, based on information from National Competent Authorities, is that the mean time to authorisation per Competent Authority has fallen in Europe from an average of around 70 days in 2002 (and 60 days in 2003) to around 49 days in 2007.
- Third, and shockingly, the mean time from protocol finalisation to the inclusion of the first patient - reported by the respondents - rose by around 30 per cent between 2003 and 2007 – and by an even more worrying 89 per cent when the figures were adjusted for the size of the commercial and academic sponsors. And despite a clear increase in the number of centres and countries per trial, there has been little increase in the number of participants.

“Something is fundamentally wrong,” said Klingmann. “We are not getting better recruitment and we are not getting faster trials.” Why the increase in the time taken to recruit patients to trials, asked Nikos Dedes? The answer is not yet clear, although Klingmann said that the most likely explanation is that the national differences in requested changes to a submitted CTA resulting in an increase in the number of

substantial amendments make it difficult to coordinate national decision-making – “one country asks for one, then you have to get the others to agree again,” she said. But then again, with the big increase in the number of multinational trials, what we don’t know is whether without the Directive things would have been even worse.

Rosalind Smyth from the Medicines for Children Network, UK, was concerned that ICREL had to struggle so hard to get the information in the first place. “This is information that the whole community – patients and academics – need on an ongoing basis. It is important that this information is collected prospectively,” she said.

The recommendations

With the full spectrum of the Directive to address, the workshop had to limit itself to a small number of key recommendations, and it did so against a background of perceived urgency. As David Haerry warned, “If Europe doesn’t get its act together and simplify procedures research will be gone to China in five to seven years.”

After extensive discussion, the following recommendations were agreed overwhelmingly (if not always unanimously).

Informed consent

- In principle, the process of informed consent should be harmonised across Europe.
- Whatever the harmonisation or opinion-building process to reach agreement on informed consent, it must include input from patients.
- The structure of the patient information sheet should be harmonised as far as possible across Europe, driven by a multinational group of experts brought together for that express purpose.

Study preparation

- Research is urgently needed to understand why the mean time from the finalisation of a protocol to the recruitment of the first patient

has risen so dramatically since the introduction of the Clinical Trials Directive.

Transparency

- There needs to be activity to gain international harmonisation of what is required in terms of posting summary results from clinical trials
- Regulatory Authorities and international organisations such as ICH and the WHO should work toward further global harmonisation on requirements for clinical trials.
- There should be consideration of extending this transparency to trials not covered by the Directive, such as those relating to medical devices, surgery, psychotherapy and so on.
- There should be consideration of whether it will be in patients' best interests to publish full trial protocols (with substantial amendments) along with the results of trials.
- Ethics committees should be obliged to follow the Directive and publish their opinions.
- The registration of clinical trials in public databases should be harmonised.

Access

- The EU should address in more detail the questions of follow-on treatment and cross-border access to clinical trials.

Risk

- Quality control measures should be adapted according to a risk-based approach.
- The effort in quality assurance should be proportional to the risk to patients.
- Sponsor questionnaires to assess risk should be developed with and validated by patients organisations.
- Patients and patients' organisations must be included in the assessment of risk..

Directive, or stronger?

- The next step towards improving the clinical trial situation in Europe must be an intense exploration of the forces hindering a smooth clinical trial environment and the options for change.

- These options could include the renewal of items of the Directive, but also a stronger alternative – the establishment of a Regulation that would apply in precisely the same terms in all countries
- Top-down legislation and regulation should be combined with a continuation of the existing bottom-up approach to harmonisation, including examination of the extension of the Voluntary Harmonisation Procedure to an obligatory single Clinical Trials Authorisation for all multinational trials.

Reports from the break-out sessions

A number of detailed recommendations also emerged from the workshop's break-out sessions.



1 How to measure quality in clinical trials

The whole purpose of the Clinical Trials Directive is to raise the quality of trials – but what is quality, and how do you measure it? As Rosalind Smyth reported, the workshop discussed the different perspectives. Industry sees quality as related to patient safety and the quality of data. Patients say trials should be understandable (with proper informed consent and proper dissemination of the results), comfortable (with acceptable procedures), and satisfactory in their conduct. Academics are looking for academic rigour and quality.

Discussion concentrated on two aspects.

- The first was peer review – delegates felt that more trial proposals should be subject to proper peer review.

- The second related to what patients see as “satisfactory”, and concluded that patients should be surveyed on this – both quantitatively and qualitatively – via interview or questionnaire.

2 How to better balance the level of required documentation and supervision with the feasibility of clinical trials and the quality of collected data?

As may be imagined with such an open-ended topic, discussion focused on a few aspects of the issue. As David Haerry reported back, several clear recommendations emerged.



- Future legislation should build in the participation and involvement of patient organisations in the process of clinical trial approval and conduct.
- Linked to this, the break-out group took up the issue of informed consent, arguing that future legislation should lay down some standards on the content and delivery of the consent process, including specifying the role and responsibility of trial nurses and the training of clinical trial teams. (The proposal on informed consent did not go unchallenged: Hugh Davies from the UK National Research Ethics Service, for example, said he was “cautious” about providing yet more guidance about informed consent that would only take its place in a growing library of other guidance.)
- In a short discussion on ethics committees, the group agreed on the need to reflect on minimum standards and guiding principles with respect to the composition and training of research ethics committees in order to guarantee adequate ethical standards across Europe.

Although we will never agree on European ethical principles, it should still be possible to develop a European standard on experimental ethics, delegates thought. They also recommended establishing “double sided” appeals to ethics committee decisions, that is, appeals to both positive and negative decisions.

The group also picked up the issue of the risk-based approach alluded to by Stefan Führung earlier. The suggestion:

- That the effort in quality assurance should be proportional to the risk to patients – which should be established through the development and validation of sponsor questionnaires.

In other suggestions, the group wanted a centralised CTA application to the EMEA for clinical trials, mimicking the mechanism of the centralised or mutual recognition procedures for marketing authorisation, though it recognised the need to think about non-interventional and other trials not currently under EMEA’s competence.

It also proposed the direct entry of suspected unexpected serious adverse reactions (SUSARs) into the EudraVigilance database and their centralised assessment, enhanced by giving trial participants the ability to report SAEs directly.

3 How to balance between intellectual property rights and patients’ needs for information on clinical trial results?

Transparency, transparency, transparency – that was the mood that Christian Ohmann, from the European Clinical Research Infrastructures



A. Freeman, N. Dedes; C. Ohmann

Network (ECRIN) brought back from this breakout group. The group wanted transparency not only with respect to the results of clinical trials but also:

- *Trial registries* – there should be registration from Phase I through to Phase IV, using WHO fields as a minimum
- *Trial protocols* – in principle, the whole protocol should be publicly available, but it was recognised that the timing and implications of this need to be evaluated carefully.
- *Trial results* – the results from all registered trials should be published, in addition to journal publishing, not instead. Publication should include a summary, the standards for which need to be explored and harmonised internationally. Publication should be timely after the end of the trial, although it is very hard to achieve publication within 6 months for pediatric trials and 12 months for non-pediatric trials. And although efforts have to be made to ensure that there is no negative impact on intellectual property, there must be provision for exceptions to this.

There was, however, no consensus on whether the raw database should be made accessible.

4 What are the requirements to ensure proper access to follow-on treatment?

Kathy Pritchard-Jones, from the International Society of Paediatric Oncology Europe and the Institute of Cancer Research, UK, reported that follow-on treatment is not well addressed in the Directive, apart from a sentence that says trials should make clear what will happen. The workshop felt that widening early access to promising drugs has benefits for patients, clinicians (who have something to offer patients) and sponsors (provides increased information for efficacy calculations in wider population, plus ongoing safety data). But as the extended discussion on the report-back showed, this is not simple matter.

Which types of patients should be considered for wider early access? If for all patients in all clinical trials, said Pritchard-Jones, it would be both difficult and expensive. Better, then, to consider defined patient populations, such as those with rare diseases and people in life-threatening conditions where there is no established treatment.

At what stage of drug development should follow-on treatment be established? Here the group recommended that at Phase 1 and Phase II it should be only for enrolled patients who have been responding well. At Phase IIb/III there is a need to establish wider access. The group also raised the issue of patient mobility for the purpose of accessing clinical trials. The EU's cross-border health initiative deals only with established treatments: it should also cover clinical trials.



Final Recommendations.

- First, that any revision of the Directive should require full consideration of access to follow-on treatment (for defined patient populations) and a harmonised process at European level.
- Second, that greater involvement of patients' organisations in initial trial design, and greater dialogue with industry and regulators, will assist informed and accelerated access to promising new drugs.

The group didn't agree with those who have concluded it is best to leave the request for follow-on treatment to ethics committees and investigators to decide on a case-by-case basis. As things stand now, patients, said Pritchard-Jones, find it very difficult to access new drugs, and often cannot afford to wait two years to find out whether a drug will be available.

There have also been concerns that extending access to follow-on treatment might become

simply market penetration by the manufacturer. To this David Haerry responded that the HIV / AIDS Community has a long experience of expanded access programmes:—not only they have saved many lives – they also yielded much valuable data. One model is provisional authorisation, as with the French Autorisation Temporaire d'Utilisation⁶ (ATU), although Pritchard-Jones warned that such approval is also a complex procedure.

Ingrid Klingmann was one of those who think we need to be very careful with concerns about market penetration – “careful about which patients under which conditions should have access to such programmes”. But, she added: “We were talking primarily about rare diseases where there is very little market and often no competition”. It's a question of finding the right balance between feasibility, affordability and the possibility of patient access to potentially life-saving drugs.

6. <http://www.afssaps.fr/Activites/Autorisations-temporaires-d-utilisation/Autorisations-temporaires-d-utilisation/>

Participants during a break-out session



Programme

EPPOSI Workshop on Clinical Trials

Shaping the future of European Clinical Research Legislation

Wednesday 22 April 2009

Diamant Conference Centre – Brussels

Welcome Remarks

– Nikos Dedes, EATG

Priorities for DG ENTR in Clinical Trials regulation in 2009

– Stefan Fuehring, European Commission, DG Enterprise and Industry

The Voluntary Harmonisation Procedure (VHP); one step in the direction of harmonisation of clinical trial applications assessment by national competent authorities?

– Hartmut Krafft, Heads of Medicines Agencies Clinical Trial Facilitation Group

Facts on the table: discussion of current trends and results

– David H.-U. Haerry, European AIDS Treatment Group
– Ingrid Klingmann, European Forum for Good Clinical Practice
– Patricia Pellier, Merck-Serono

BREAK-OUT SESSIONS

1. How to measure quality in clinical trials?

Chairs: – Gérard Nguyen, Rett Syndrome Europe
– Rosalind Smyth, Medicines for Children Research Network
– Patricia Pellier, Merck-Serono

2. How to better balance the level of required documentation and supervision - with the feasibility of clinical trials and the quality of collected data?

Chairs: – David H.-U. Haerry, European AIDS Treatment Group
– Jocelyne Flament, European Organisation for Research and Treatment of Cancer
– Fabien Peuvrelle, Celgene

3. How to balance between intellectual property rights and patients' needs for information on clinical trials results?

Chairs: – Nikos Dedes, European AIDS Treatment Group
– Christian Ohmann, European Clinical Research Infrastructures Network
– Andrew Freeman, GlaxoSmithKline

4. What are the requirements to ensure proper access to follow-on treatment?

Chairs: – Jan Geissler, European Cancer Patients Coalition
– Kathy Pritchard-Jones, Institute of Cancer Research, SIOP Europe
– Dagmar Theis, Roche

REPORT OF BREAK-OUT SESSIONS

Discussion and preparation of final recommendations

Moderator: – Ingrid Klingmann, European Forum for Good Clinical Practice

Conclusions – David H.-U. Haerry, European AIDS Treatment Group
– Patricia Pellier, Merck-Serono

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**A patient-led EU partnership of patients, academic science and industry,
working together to advance healthcare policies for the prevention and treatment of serious diseases**

EPPOSI was founded in 1994 on the initiative of patients' organisations, for the exchange of information and the discussion of human healthcare policies in the EU.

EPPOSI puts patients first in this dialogue, providing a forum for patients, academia, authorities and industry to discuss innovation and policies for healthcare, health technology, and the health outcomes for patients, especially those affected by chronic, life-threatening diseases – including rare diseases.

EPPOSI's ambition is to develop strategies that benefit present and future generations.

Objectives

- To encourage timely and regular exchange of information between stakeholders on the latest developments in human healthcare related to (bio-)medical research, policy and regulations; on the ethical, social, legal and political aspects of this type of research, and on biotechnology, notably for its application to human healthcare
- To promote a mutual understanding between patients' organisations, science, industry, and EU institutions
- To contribute to equal access for all to human healthcare products and services in the EU
- To support patients' organisations in presenting timely and effective contributions to the European political debate on all matters that concern them
- To raise public awareness in Europe on the opinion of patients and their organisations
- To help sustain a dialogue within society on progress in medical science through new technologies
- To advocate the development of therapies for unmet medical needs and to facilitate partnerships within society
- To function as an information coordination centre that encourages discussion, opinion-forming, and public debate in the area of human healthcare

Achievements

EPPOSI focuses on building dialogue, consensus positions and policy recommendations for the benefit of EU patients and consumers.

These consensus positions have provided building blocks for:

- the establishment of the European Orphan Medicinal Products Regulation
- the advancement of biomedical research and the value of innovation
- the timely access to innovative medicines
- several rare-disease therapy developments and partnerships
- East-West European collaboration among patient groups
- bio-banking

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