

# Time for a change

Implementation of the EU clinical directive 2001/20/EC has changed the regulatory environment.

**Criticism of the EU's 2001 clinical Trial Directive is nothing new. Consensus about its adverse effects on clinical research both commercial and academic, has been voiced since its prevision came into effect in 2004.**

EU and US have frameworks in place to encourage the development of medicine for rare diseases (Incentives, scientific support during development...).

**However, in EU hurdles associated with the implementation of Clinical Trials are huge: Regulatory framework is fragmented and complex.**

## How to support the development of a better clinical trial environment for rare disease in the EU?

### Challenges

- Understanding of the disease
- Striking a balance between optimal science and doable clinical trial (*expl: powering studies for proper endpoint ability to recruit the adequate number of patients*)
- Lack of guidelines/Lack of standard therapy
- Availability of clinical experts
- Geographic localisation of patients: patient travel substantial distances for study participation.

### Trials have to be multicenter or even international to enroll enough patients

- Administrative hurdles**
  - Absence of standardisation of requirements
  - Extra paperwork
- Cost hurdles**
  - In EU, the cost of insuring CT has risen by almost one order of magnitude since 2004.

**Companies should not waste resources making multiple CTAs applications.**

**All stakeholders (member states, regulatory agencies, patients, physicians, companies) need to make big efforts to get the commission make the necessary changes.**

## URGENT

**to develop recommendation on how to improve clinical development of orphan drugs in the EU.**

**A suitable regulatory framework must be encouraged to ensure that new therapeutic options are entering the market in a reasonable timeframe:**

- ✓ A procedure for facilitating submission of CTA should be in place
- ✓ Harmonisation of ethics committees requirements
- ✓ To bring some degrees of standardisation
- ✓ Single authorisation for multinational trials
- ✓ Consideration to involving ancillary sites to manage routine visits
- ✓ Expedited sites initiation process for patient in emergency medical conditions.

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