

2 recent workshops on patient registries for rare diseases

March 13, 2008

Rare Diseases Task Force

**PATIENT REGISTRIES
AND DATABASES IN THE
FIELD OF RARE
DISEASES**

**TECHNICAL, LEGAL AND
ETHICAL ISSUES**

April 2009

March 18, 2009



EPPOSI Workshop on Patients Registries for Rare Disorders
*Need for data collection to increase knowledge on rare disorders and
optimize disease management and care*

Wednesday 18 – Thursday 19 March 2009
Scientific Institute of Public Health
Brussels, Belgium

EPPOSI

European Platform for Patients' Organisations,
Science and Industry

Industry

Patients

Academe

Partners or protagonists?

The following slides are verbatim from the draft report

From the executive summary:

Without no established forum for sharing experience on the operation of registries, too many people are re-inventing the wheel. The workshop identified a string of areas where the establishment of best practice, advice and templates could save time and money. In particular, the workshop concluded that a working group needs to be established

“Recommendations and conclusions”

More fairly described as *“Points made during workshop”*

Funding

1.The independence of registries depends more on its governance and funding conditions than on the origin of the funding.

2.Ideally, funding from an interest group will be provided on an unrestricted basis.

3.Industry should consider joining academic-originated registries.

4.Whatever the ideal, it is important that EU funding continues, and increases, both with regard to overall funding and to the proportion of costs that can be funded under Framework and the Health Programme.

5.Registries should be seen as infrastructures rather than projects, except in the initial phase of establishment, and EU funding of a registry should allow Member States to co-fund (and they should be encouraged to do so).

6.While it is right that funding be time-limited and renewable (ie, no automatic right for funding to continue) a mechanism should be found whereby support can be assured to a registry that can show it is properly run, achieving its objectives and has a good reason to continue.

Ownership

1. Whether or not legal ownership of registry data resides with operator of the registry, the governance of the registry should reflect the concepts of responsibility and custodianship rather than ownership.
2. That responsibility and custodianship will be augmented if the conditions under which patients and doctors give data to a registry incorporate the principle of a conditional gift.
3. Oversight Committees and their members must be bound by rules requiring them to operate according to ethical principles.

Data collection and data quality

1. Health authorities have a duty to fund not just the setting-up of databases but ongoing data collection as well.
- 2. Data entry can be greatly assisted by having dedicated documenters visiting the clinical centres and inputting data.**
- 3. Data quality should be ensured by focused data management and defined quality standards.**
- ~~4. Consideration should be given to financial incentives to encourage physicians to add information to registries.~~
5. Time and effort invested by physicians in contributing to registries should also be recognised by professional credits.
6. Web interfaces for data collection should be promoted online with patient communities and via Search Engine Optimisation.

Duplication of effort

- 1. When planning a registry, see what is out there already and plan for data integration if possible.**
- 2. Also when planning a registry, consider data harmonisation and sharing from the first.**

Economies of scale

1. The aim should be a common *resource* for registries, rather than common registries.

2. EPPOSI should consider convening a working group to investigate the feasibility of producing downloadable, customisable registry software that would save on time and money and increase the interoperability of different registries.

Governance

- 1.All registries should establish the principle of independence in their governance.**
- 2.Registry rules must be written, public and expressed in simple language that can be understood by all the stakeholders.**
- 3.Representatives of all the stakeholders should be actively involved in the governance of a registry.**
- 4.The registry must be clearly accountable to its stakeholders.**
- 5.An Oversight Committee should be established with a specific brief to protect the interests of patients; it should be independent of industry.**

Legal and ethical issues

1. Whether required by local law or not, **informed consent should be sought from patients.**
2. It is not possible to anonymize data completely and still control quality.
3. Patient identity should be kept as confidential and secure as possible.
4. EPPOSI should consider further the idea of a central ethics body ~~in each country~~ to oversee submissions regarding registries.
5. **The training of research ethics committees should include training about registries and databases.**

Charging industry for access

1. Those setting up registries need to think about the charging structure, even if they decide they don't want to charge at all.

2. Registries should maintain a set of publicly accessible rules about fee structure or benefit sharing if a therapy is successfully developed using information derived from the registry.

Technical issues

- 1 EPPOSI should consider providing a list of reference centres experienced in data management.
- 2 **EPPOSI should consider elaborating a checklist for evaluating IT companies**
- 3 **The data model is critical: everything will rely on it. Spend time thinking about what kind of data should be in your system. Use experience from your partners.**
- 4 Depending on the granularity required, use ontology based on your own data model and on your own goals.
- 5 **Security needs to be addressed through the highest standards of back-up, prevention, build and audit.**
- 6 Think about scalability and modularity from the very beginning. Allow for change.
- 7 Experience on data sharing needs to be accumulated and itself shared.
- 8 **Invest in user testing to ensure a user-friendly interface**
- 9 **Registries and those planning them would benefit from a list of quality control checks**

Appendix: Setting up a rare disease registry: an outline checklist for beginners

Questions for EURORDIS

1. Accept conclusions & recommendations of EPPOSI report?

1. Prepare an independent report from this Workshop?

2. Agree that a working group should be set up?

3. If so, under what auspices?

4. Rôle of the expert patient?

5. Database of EU databases?

continued

Questions for EURORDIS 2

6.<All RD> vs disease specific?

7.National vs European?

8.Commonalities of - expertise? –software?

9.Funding?

10.ENSURING THE PATIENT VOICE IS HEARD