



RaDiOrg.be
Rare Diseases Organisation

The Changing Role of the Patient. Research Partners for Innovative Medicinal Products?

Lut De Baere

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FIN

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- How will developments in clinical research bear upon the patients and vice versa ?
- How will the requirements of the payers to grant early access to new products affect the patients involvement ?
- What can be done to stimulate clinical research in orphan diseases ?
- What is the impact of clinical research in Belgium and how can patients benefit from this research ?
- How can increasing drug development costs be reconciled with the increasing pressure on health care budgets ?
- What are the implications for patients' access to innovative agents ?

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PAST !

Patient's & Patient Organisation's role : restricted to nonexistent



Augusto & Michaela Odone - Lorenzo's oil ...
Paul & Elizabeth Kontoyannis – Nikolas Symposium ...



Scientists are human. Embrace them.
Their lives are hectic.
Help try to make them feel that your disease is important.
Be human with them ...

&
vice versa

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Don't we all share the same objectives ?

- Obtaining high quality knowledge on diseases
- Obtaining a better quality of life
- Developing effective and safe treatments
- If possible a cure

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**Don't we all have a specific knowledge,
brought together = more complete ?**

- Sharing complementary knowledge
- Sharing complementary experiences
- Sharing complementary skills
- Sharing complementary competences

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Interaction with patients involve ...

- Activities with a legal basis e.g. Participation in the management board
Activities & involvement in EMA workshops and conferences
- Activities where no legal basis exists it occurs sporadically, despite positive feedback, in particular in benefit/risk considerations

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Added value when involving patients ?

Patients are expected to bring real-life experience of the disease and its Current therapeutic environment.

This has very positive consequences such as:

- Enriching regulatory outcome by complementing it with the views of those directly affected by regulatory decisions
- Increasing confidence and trust in the regulatory process
- Incurring a higher level of transparency
- Assist in the integration of patients as members

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Thalidomide ...

Helped in case of sleeplessness or in case of morning sickness.

Side-effect : Defects of unborn children
Missing or underdeveloped limbs

Taken off the market.

Was also treatment for Myeloma ... patients were deprived of the one thing that helped them ...

Patient organisations lobbied to put product back on the market and they succeeded !

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Eurordis Charter

All face variable situations in the field of the disease:

- Clinical aspects of the disease
- Varying skills and competence
- Size
- Experience
- Scientific commitment
- Logistical means of both sponsor and patient organisation



Eurordis Charter

Role of patient organisations in different aspect and all phases of clinical trials ?

- They can adapt the design of the study to the expectations of the patient, which will encourage their adhesion to the trial.
- By providing early information to potential participants ensures and accelerates the inclusion within the trial.
- Patients who receive support in the best possible way during the study, are more likely to be compliant resulting in less drop-outs and incomplete files.
- Quality of life should be taken into consideration as well as discussions between sponsors and patient organisations. This contributes to the assessment of clinical trials and day-to-day benefits of the treatment.

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Eurordis Charter

General Principles

1. Patient organisations (POs) should be informed on all aspects of the clinical study protocol prior to committing to collaborate. This would provide legitimacy to the inclusion of patients in the study. For the same reasons, any substantial amendment to the protocol should be communicated to the POs in real time.
2. POs should actively contribute to the documents aimed at patients – information leaflet and consent form - to ensure their content and format can be understood by lay people, thus allowing truly informed consent by patients.

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Eurordis Charter

General Principles

3. Domains and extent of collaboration should be declared in a document called “**Agreement of Understanding**” available for all stakeholders: patients, investigators, ethics committees and national competent authorities. The agreement, established on a voluntary basis by Sponsors and POs for a given clinical study, describes the areas of and potential limitations for the collaboration, without detailing its content.

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Eurordis Charter

General Principles

4. Financial relationships between Sponsors and POs should be transparent.
5. Study results should be available, even in the case of negative outcomes, inconclusive or abandoned clinical trials.
6. Patients participate in clinical studies to improve knowledge of the disease and help develop adaptable treatment. To fully respect patients' collective commitment, the data acquired during clinical trials should be made available to the scientific community, with a view to fostering scientific progress and avoiding unethical duplication of clinical trials.

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Eurordis Charter

General Principles

7. The commitment of a PO, in the design and/or development of a trial, does not change the role and responsibilities of the Sponsor, even if the study is financially supported by the PO.

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The Role of Patient Organisations

- ✓ Strong motivation
- ✓ Wide variety of people represented
- ✓ Representation beyond emotions
- ✓ Knowledge → strong & valid arguments
- ✓ Contacts with range of stakeholders
- ✓ Broad understanding of relevant issues
- ✓ Compelling and credible position

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The Role of Patient Organisations

- ✓ Need to work on strong alliances
- ✓ Need to have contacts and meetings with various stakeholders
- ✓ Can work as glue or catalyst
- ✓ We have to stay independent !!!
- ✓ establish continuity
- ✓ Maintain dialogue
- ✓ Monitor quality of our work

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The Role of Patient Organisations

- ✓ advertise for clinical trials
- ✓ add information
- ✓ make information accessible
- ✓ ...
- ✓ exchange of information / data between PO en pharma

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The motivation of the Patient

- It gives them an opportunity for improved medical care.
 - They get early access to new and innovative drugs.
 - They get extra attention: closer follow up and stronger supportive relationship.
- They are doing something to benefit science.
- It gives them a financial benefit.
 - Travel costs are reimbursed.
 - Get free medical care.
- There are some fears:
 - to be used as some sort of guinea pig
 - worry about risks

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The motivation of the Patient

- faster way to treatment and better chance to a (normal) quality of life
- beneficiary position → obligation of compliance
- right to check protocol, relevance and meaningfulness of trial
- right to be well informed in full awareness
- right to entry or withdraw at any time for any reason

- well protected by several regulations – laws – guidelines - ...

- well informed on patient organisations !!!

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Increased drug development VS increasing pressure on health care budgets

- ❖ Better development
 - ❖ Less early aged handicaps
 - ❖ Being able to study / work /
 - ❖ stay healthier for a longer time
 - ❖ Being invalid for a lesser time
- ❖ More time to contribute to society ← → less time to “*profit*” from society

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Not just economical benefit ...

Emotional impact on the patient:

- ✓ Self-esteem of the patient
- ✓ Being looked upon as a full person rather than a patient or invalid

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It's all about the money ... ???

Case of compassionate use

Cumulative cost for pharma = > 5 million euro's

Cumulative cost for health insurance = 230.000 €

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Stimulate Clinical Research in OD

What can be done ? Who can do it ?

- Making public and stakeholders aware of Rare Disease and it's impact.
- A change in attitude ?
 - Economically not viable to invest in OD
 - For those involved
 - Difference between life/death
 - Difference in quality of life
 - Being taken serious
 - Being important

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Stimulate Clinical Research in OD

Keep knocking on doors

Keep asking questions

Keep shouting as loud as we can

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Stimulate Clinical Research in OD

**The Changing Role of the ministry of
Health and the Health Insurance
In using Health Care Budgets**



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**Thank you very much
for your attention !**

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