Good practices For the Collaboration between Centres of Expertise and Patients' Organisations

Centres of Expertise for Rare Diseases (CoEs) and Patient Organisations (POs) share common objectives: delivering high-quality care, including effective and safe treatments. Centres of Expertise and Patients' Organisations both recognise that collaboration in the development of working relations - based on sharing complementary skills and competences - is effective. The interest of such collaboration is reinforced by the participation of patients' representatives in the shaping of National Plans/Strategies for rare diseases and in the European Union Committee of Experts on Rare Diseases (EU-CERD).

Following extensive multi-stakeholders discussions at National and European level from 2006 to 2008 in the frame of the RAPSODY[1] project, recommendations for the definition and objectives of Centres of Expertise for rare diseases were published and provided to the European Commission. These recommendations contributed to the Commission Communication on rare diseases (2008), and to the Council Recommendations in the field of rare diseases (2009).

All stakeholders are convinced that patients' organisations, with their extensive knowledge on their rare disease, can fruitfully collaborate with Centres of Expertise in different aspects (see 1. Basic Collaborative Support and following).

Those involved in Centres of Expertise for rare diseases face highly variable situations; therefore a universal regulation for all situations would be unrealistic and ineffective. However, certain rules are required to maintain a relationship of trust between the various stakeholders (health care professionals, POs, patients, health insurance, policy makers, industry…) and are essential for a fruitful collaboration. Good Practices for the Collaboration between Centres of Expertise and patients' organisations are necessary for high quality care to patients followed at the Centres.

The aim of these Good Practices, firstly developed at EURORDIS General Assembly 2008, and then updated within the frame of the POLKA Project[2], is to improve the collaboration between patients, their representatives and Centres of Expertise and to encourage transparent and effective dialogue between interested parties.

[1] Rare Disease Patients' Solidarity, a project by EURORDIS and its partners: Rare Disorders Denmark, Federacion Espanola de Enfermedades Raras, Association Française contre les Myopathies, Barretstown, Orphanet-Inserm, State Institute for Drug Control Czech Republic, Frambu, National Centre For Information on Metabolic Diseases UK, Fundacio Doctor Robert and supported by the European Commission DG SANCO. 2006-2008

[2] Patients ' Consensus on Preferred Policy Scenarii for Rare Diseases, a project project by EURORDIS and its partners: Rare Disorders Denmark, National Commissioning Group NCG-NHS, Fundacio Doctor Robert, and supported by the European Commission DG SANCO. 2008-2011.

Concretely

The Good Practices are proposed as a set of general principles to be adhered to. In contrast to a Regulation, implementation of these Good Practices is solely based on the goodwill of both Centres of Expertise and patients' organisations, who voluntarily agree to share these principles.

The Good Practices will be reviewed and modified, as appropriate.

- Any Centre of Expertise who agrees with the underlying principles of the Good Practices is invited to adopt them.

- EURORDIS commits to facilitating the implementation of the Good Practices by proposing to continue the evaluation of relations between patients' organisations and their Centres and by making proposals both for the management and the policy on Centres of Expertise

General principles

1. Basic Collaborative Support

To facilitate partnership with Centres of Expertise, patients' organisations:

a. Participate in the governance of the Centre:
   - Be on the governing board of Centre;

b. Contribute to the work of the Centre:
   - Offer counselling, especially for new patients, patients with special needs, Patient / family support before / post-diagnosis
   - Train patients on how to have an active role in the management of the disease and how to participate in pharmacovigilance, risk management programmes, cost effectiveness studies, patient-reported outcomes studies
   - Manage discussion groups inside and/or outside the Centre (teachers, fellow students, and employers), self-help groups, How to improve the functioning of the Centre(discussions between Health care professionals and patients.

c. Contribute to the dissemination of Centre' work;

d. Contribute to the evaluation of the Centre:
   - Conduct surveys on patient needs and satisfaction;
   - Work on monitoring and quality assurance of the Centre
   - List missing competences and infrastructures, when needed, with up-to-date contact details on where to find them outside the centre

e. Offer space and fulfil expectations of patients:
   - Patients' organisation visibility and availability in centre;
   - Patients' organisations can advise on day-to-day organisation and medical practices adapted to patient special needs within the centre: for example waiting times , consultation days and hours compatible with attendance at school and work, blood tests and other examinations, physical infrastructure of the centre, and adequate care pathways…

f. Participate in creating information on the disease and its treatments:

- To produce Information sheets for patients / families / enablers and to facilitate the care pathway:
    1. On available services and how to access them
    2. On medical treatment options
    3. On social care / social work
    4. On useful contacts (i.e., helplines)
    5. Good practice advice on pain alleviation, nutrition, cleaning medical devices, emergency situations, learning disabilities etc.
    6. On how to travel with the disease, including cross-border care
- Train patients on how to have an active role in the management of the disease
    1. To understand treatments
    2. To be involved in the definition and conduct of Therapeutic Education Programmes
    3. To help find contacts on an international level of other health care professionals in other centres

2. <u>Additional Collaborative Support both for patients, their organisations and health care professionals</u>

a. Participate in trainings to health care and non-health care professionals
    - Communication, within the Centre, but also with affiliated health care professionals (general practitioners, allied specialists, etc.)

b. Be involved in working groups, and disseminate information about research, patient registries and Biobanks
    - To understand studies and trials, to be a partner in designing, executing, translating (international) trials
    - To understand research on basic science, and/or to participate in clinical trials, drug development, translational research…
    - To participate in registries: databases, especially on an international level, but even in some countries where registries are regional and not nationalised

c. Consult patients in , be involved in working groups, and disseminate information on Standards of Diagnosis and Care

d. Participate in the working group, collect existing examples, disseminate survey of needs, and disseminate conclusions on Social Care and Work Guidelines

3. <u>Operational Support</u>: for the day-to-day work of Patients' Organisations

Centres and patients' organisations are encouraged to define together the collaboration domains on an ad-hoc basis; this can be done via a written agreement[3] between the two parties.

What are the major drawbacks to the development of such collaboration?

1. Funding: it may come from the National Plan for Rare Diseases, the Centre itself, patients' organisation membership fees or fundraising, private grants, etc.

[3] Describing what is included or excluded from the collaboration, what are the POs' commitments, which points are potentially considered confidential by the Centres of Expertise and how to ensure confidentiality, clauses in case of restrictions to data access (registries, surveys, databases, scientific communication…), in adequate language and formats according to age, language, and culture.

2. The PO should be legally constituted, which the National Alliances can help with.

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[4] Rare Disorders Denmark (Denmark), National Commissioning Group NCG-NHS (England), Fundacio Doctor Robert, Spain