

IMPLEMENTATION AND EVALUATION OF A TRAINING PROGRAMME ON ORPHAN DRUGS TARGETING PATIENTS' ASSOCIATIONS AND FAMILIES OF PATIENTS WITH RARE DISEASES



D Pierannunzio¹, A Trama¹, M De Santis¹, C Giannelli¹, I Luzi¹
¹ National Institute of Health, National Center for Rare Diseases - Rome, Italy



Background

Although the concept of patient engagement is not necessarily new, it is only in very recent years that different countries such as Australia, United States, Canada, UK, Norway have begun to embrace the concept as a viable remedy for improving quality of care on a broad scale. There is a growing belief among policy-makers that patients/citizens can contribute to quality improvement at both an individual and a collective level. As individuals or family members patients can play a distinct role in their own care, it is believed that patient and public engagement in determining priorities for service development could help to ensure that policy-making keeps in tune with population requirements.

A growing body of evidence shows that patients engagement in treatment decisions and in managing their own health care can improve patients' experience and often results in better health outcomes and more appropriate and cost effective utilisation of health services. Particularly in the field of rare diseases, patients' associations play even a more critical and essential role ensuring a communication network for families. Initiatives aiming at building the capacity of patients are scarce.

The National Centre for Rare Diseases (NCRD) and patients' Associations are collaborating in several projects, in order to increase and to define interventions in public health. Focusing on patients' associations with rare diseases, the project aims at increasing patient's knowledge and understanding on orphan drugs.

Methodology

The project using a training of trainees approach aims at reaching all members of the Associations amplifying with a cascade mechanism the target of the intervention. Within each Associations one person will be identified and trained. This person will be responsible to train additional members of his/her Associations multiplying the number of trainers available and therefore multiplying the number of person properly informed and skilled on orphan drugs. The intervention will work with Associations of patients with rare diseases, registered in the database dedicated to rare diseases patient's organisation of the NCRD.

The list of project activities follow:

1. Analysis of the status of the art of Associations knowledge, attitude and activities about orphan drugs.
2. Assessment of information/training needs of Associations. The needs assessment will be done through a questionnaire. Questionnaires results will be integrated with information on patient's needs deriving from the toll free number service and the Narrative Medicine project.
3. Development of the training curriculum on the basis of the needs assessment.
4. Implementation of the training programme through residential meetings.
5. Implementation of trainers activities within each Associations. Each trainer will have to identify and implement before the end of the second year of the project at least one activities related to orphan drugs within his/her organisation.
6. Final workshop to present the activities performed by each trainer. This will allow the exchange of information providing a common moment to learn from each other experience.

Objectives

The project aim is to increase Associations knowledge on orphan drugs.

Specific objectives are:

- Measure patients' Association knowledge on orphan drugs (drugs development, regulatory process, clinical trials and ethical issues).
- List and describe patients' Association activities on orphan drugs.
- Identify specific information/educational needs of patients' Associations on orphan drugs.
- Develop a training curriculum on orphan drugs on the basis of the information/educational needs assessment.

Further objective is to create, through a participatory training programme, a transparent and collaborative partnership between patients' Associations.

Current achievements

Needs assessment

Targeted to all the Associations (220) officially registered in the database dedicated to rare diseases patient's organisation of the national centre for rare diseases.

An on line questionnaire was developed to assess Associations information needs; 107 Associations filled in the questionnaires. Accordingly to our preliminary analysis, the areas where information is more needed follow:

- Drug development process
- Regulatory process for orphan drugs including reimbursement procedures
- Development and availability of guidelines for rare diseases
- Innovative medicines.

The development of the training curriculum is on going.

The questionnaire was completed directly on line accessing the area of the National Centre web site dedicated to patients' Associations.



Expected outcomes

The project will increase the capacity of each Associations and members/patients to collaborate and be actively involved in regulatory processes, ethical committees and clinical research.

Moreover, it will build the capacity of patients take responsible decision on their health and specifically on their treatment options.

References

1. Brigioni P, Bianco E., Sampaolo L., Carra L. (2005), PNLG - Il coinvolgimento dei cittadini nelle scelte in Sanità - Una rassegna di studi, esperienze, teorie; Revisione: dicembre 2005
2. Mosconi P, Apolone G. (2005), "Approvazione di nuovi farmaci: la parola ai cittadini" in Gli Ospedali della Vita: 3-4, Ottobre 2005
3. Mosconi P, Colombo C. (2005), "Come, dove, quando vengono coinvolti i cittadini in Sanità", Clinical Governance: 24-30, Agosto 2005
4. Mosconi P, Poli P., Giolo A., Apolone G. (2005), "How health consumers fell about clinical research: a questionnaire survey", European Journal of Public Health 15: 372-379
5. Mosconi P, Apolone G. (2005), "Consumers and the drug regulatory process", EUROPA DONNA News, Summer/Autumn: 12
6. Mosconi P, Colombo C. (2005), "Costruire un'alleanza strategica tra associazioni di pazienti & cittadini e comunità medico-scientifica: il progetto PartecipaSalute", Clinical Governance 2: 37-42 Maggio 2005
7. Apolone G., Bassi M., Buscone N., Mosconi P, Rizzini P., Scarpino V., Velo G. (2005), "Informazione scientifica sul farmaco: riflessioni e indirizzi per una buona pratica", Economia Politica del Farmaco n.5: 30-35, Giugno 2005
8. Marsico G. et al (2004), "Epidemiologia qualitativa delle pratiche dei Comitati Etici. Progetto Trasparenza". Giornale Italiano di Farmacia Clinica; 18: 1-7

Contacts

Dr. Daniela Pierannunzio – Dr. Annalisa Trama

National Institute of Health, National Center for Rare Diseases,
Viale Regina Elena, 299 - 00161 Rome (Italy)

+39 06 4990 4368/4371 - @ daniela.pierannunzio@iss.it annalisa.trama@iss.it
www.iss.it/cnmr