
EMP's first steps in the field of clinical trials: ECRD Conference

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Abstract

The development process of clinical trials has for long been the territory of the researchers, healthcare providers and regulators. Patients were only involved as “subjects” and had a passive role. However, in the last decennium, patients and their advocates have been moving more towards an active participation in the development of clinical trials. Many stakeholders realize that by actively involving patients, a patient tailored system of care can be achieved. However, it can be quite a challenge for patient organizations, who often work with volunteers and limited manpower to act as an equal partner in this specialized area where deadlines, standards and certain work principles have to be met. Moreover, patient representatives need to acquire enough “literacy” in this highly scientific environment. This document presents the case of a young patient organization EMP and the way EMP handled this challenge. It explains how a patient organisation can proceed step by step to meet the exigent criteria and how literacy can be gained through different training programs. The document further explains how EMP started active involvement in the development of clinical trials, by initially only focusing on activities that were within realistic reach of the group. These first prudent steps gave the patient representatives further knowledge and research skills; it gave them confidence and it created a relationship of trust with the other stakeholders. These are probably strong foundations for more extensive collaboration in the field of clinical trials in the future.

1 INTRODUCTION

EMP (European Myeloma Platform) is a European umbrella organization for national multiple myeloma patient organizations.

Multiple Myeloma (MM) is a rare cancer of the bone marrow, a disease for which treatment options have been limited for a long time. However, a lot of research has been done in the past decennium and treatment options are improving. One of EMP's basic goals is to support the research and to further improve the access to new treatments and medications for the disease.

However, our organization did not want to tackle this new terrain impulsively. Indeed, the research and medical arena is a highly scientific environment and, although patients and their representatives have a unique and valuable experiential knowledge, they do not necessarily have the specific scientific training, useful for the work. We wanted to proceed in a structured way and before exploring the possibilities of partnership with other stakeholders in the field, we first wanted to get to learn the other potential partners better and to install a dialogue with them.

2 STRATEGY

2.1 Exploration of the MM health care environment

In order to have a good insight in the health care environment related to MM and to better understand the other stakeholder's point of view on patient involvement in health care, EMP organised, the “No Policy without Patients” symposium, a multi-stakeholder-meeting, in October 2008 in Maastricht. This event was organized together with CKP (Contactgroep Kahler en Waldenström Patiënten, the Netherlands). The symposium served as a communication platform between the different partners in health care: The patients and the patient organizations, the regulators, politicians, the healthcare professionals, the researchers and the industry.

2.2 Observations

An important conclusion on which all stakeholders agreed is that access to new treatments and medications can be greatly improved by involving patient representatives in the area of clinical trials. This way, a patient tailored system of care can be achieved. The experiential knowledge of patients completes the scientific and medical knowledge of researchers. Therefore, patient

organizations should develop working relationships with the industry and with academic research teams.

Many areas of useful patient involvement in clinical trials were discussed:

- the review of medical literature for patients (informed consent, patient information of the trial)
- the review of clinical trial protocols
- the dissemination of information on trials to patients in lay-language
- the distribution of information and data on novel trials within the patient community
- the evaluation of the effects on the quality of life of new treatments or drugs
- representation by patients in ethical committees
- advice on the selection and the development of clinical trials
- advice on risk management programmes
- the support of funding for research and drug development

For a young patient organization such as EMP, it was unrealistic and too ambitious at that stage to get involved in all of the above-mentioned areas at once. In order to be efficient and deliver quality in the work, a patient organisation should define priorities and proceed step by step to become involved in areas that are within reach of the patient organization's actual capacity, taking into account the current strength and possibilities of the group.

Another important feedback from the other stakeholders was that patient organisations have to meet certain criteria in order to become an equal partner in the field, such as having a strong organisational structure and a certain level of literacy (see 2.3).

2.3 Action Plan

With these conclusions, an Action Plan was set up for EMP. The Action Plan stated that our first step in the field of clinical trials would be to establish good working relationships with the (academic) researchers and the industry. Our initial collaboration with these partners would be the review of the medical literature for patients and to review the protocols of the clinical trials.

Indeed, feedback from many patients learns us that the trial documents (the informed consent and the patient information) are not always easily understandable for laypersons. The review of those documents by patients ensures that the documents are patient-friendly.

Review of the protocols of clinical trials by patients is useful to make sure that the treatments and the procedures are acceptable and comfortable for patients. Indeed, treatment protocols do not always consider the psychosocial aspects or the issues surrounding the quality of life.

Another important conclusion of the symposium was that, in order to achieve a positive evaluation of patient involvement in the field of clinical trials, certain conditions have to be in place. It is an absolute requirement that patient organizations can rely on a strong organizational structure with smooth communication lines and distinguished tasks and responsibilities. This because the patient organization, as an equal partner, has to act according to the standards, timeframes and working principles of their interlocutors.

Active patient involvement also requires that patient organizations are highly literate or at least that some of their representatives are. Literacy is necessary in order to grasp and discuss complex issues and to be knowledgeable in the specific disease field, clinical research, health politics and so on.

So, in order to be an equal partner in health care, a patient organization has to meet quite some exigent criteria. Most patient representatives volunteer for the work because of their confrontation with the disease and they do not necessarily have this specific background at the base. Also, volunteer organizations have more difficulties in realising these conditions than well established, large scale and financially stronger umbrella organizations with paid staff. Hence the need for coalitions, networking and collaboration among disease specific organizations and umbrella rare disease organizations.

2.4 Realisation of the Action Plan

Our membership of and our collaboration with EURORDIS was a great help in improving our knowledge and literacy in the field of clinical trials in order to give us the necessary background for the work.

Some of our representatives participated at the EURORDIS Summer School, in Barcelona.

The EURORDIS Summer School is a unique and intensive 4-day programme in which patient advocates learn about the structure of clinical trials, drug development and drug regulatory processes in Europe. During this training, participants interact with regulators, researchers and other patient representatives in order to learn from each other's experiences regarding clinical trials and drug development. The Summer School proved to be an important source of information for our representatives and a stimulating environment for future involvement in clinical trials.

EURORDIS offered even more valuable help to our organization. The Health Policy Officer François Houyez chaired an internal EMP-workshop on the development of the previously mentioned Action Plan of the Symposium. With his tailor-made advice and guidance, EMP was getting prepared for the work.

EMP's active participation in the PatientPartner Project, a 3-year EU FP7 project, gave our organization some further insight in the area of clinical trials. This project investigates, enforces and advises on the role of patient organizations in clinical trials.

Several of our collaborators further improved their knowledge by self study, by following other national and international workshops on the topic and by their interactions with other stakeholders in the field.

3 RESULTS

After this period of training and instruction, EMP representatives gathered enough background and literacy to start partnership with the researchers and the Industry.

An agreement on collaboration was signed with a European Academic Research group: EMN (European Myeloma Network), specialized in research in Multiple Myeloma. At a first stage, it was agreed that the collaboration would consist of the review by our patient organization of the protocol of the EMN clinical trials and the trial documents, addressed to patients (informed consent and patient information leaflet).

In the beginning of the collaboration, EMP was the only patient organization involved in the work. However, in order to represent all European MM patients, EMP took the initiative to ask the other MM patient organizations to join in the work (Myeloma Euronet and Myeloma UK). This has been realized in the meantime and we can say that a positive "side effect" from our involvement in clinical trials is that it brought all EU-MM patient organizations closer together!

After this positive experience, we also started up collaboration with some pharmaceutical companies, active in the field of multiple myeloma. Here also, at this stage our involvement consists in the review of the trial protocol and the patient documents.

In our collaboration with the Industry, EMP works according the principles of the EURORDIS Charter. The "EURORDIS Charter for Collaboration between Sponsors and Patient Organizations for Clinical Trials in Rare Diseases" consists of guidelines, aiming to improve the quality of clinical research in rare diseases and at enhancing a transparent and effective dialogue between sponsors and patient organizations. The Charter also details what a written Agreement of Understanding between a pharmaceutical company and a patient organization should include for a given clinical trial.

Besides these first partnerships in clinical trials, EMP is also involved in some other "Ad-Hoc" clinical trial projects:

- Providing information on clinical trials to patients, via our member associations (in order to "demystify" the subject and better inform the patients)
- Informing patients, via our member associations, about recruiting clinical trials in Multiple Myeloma
- Collaboration with the European Medicines Agency: user testing of the new website on clinical trials "EudraCT"

4 CONCLUSION

Those first projects gave our organization and its representatives considerable experience, further knowledge and insight as well as some self-assurance in this specialized area. The result of the collaboration is seen as very fruitful by the participating partners as it makes clinical trials more attractive, patient friendly and accessible to the patients, a win-win situation for every stakeholder!

Moreover, it brought several multiple myeloma patient groups together and might possibly lead to future strong coalitions. For our organization, it was the right strategy to build up our collaboration with the stakeholders step by step. We focused on a working area that was realistic for the capacity and the strength of our organization. We have learned to walk before we can start to run! These first projects allowed the different stakeholders to appreciate the benefits of patients' involvement in the field of clinical trials, to get to learn each other and to build up a working relationship based on mutual respect and trust. These are strong and necessary foundations for a more extensive collaboration and further involvement in future clinical trials.

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