



Patient Organisations & Pharmaceutical Medicine

The role of the Patient Organisations and the Public Sector in influencing healthcare decisions

Patient Organisations have expanded in number and complexity over the last few decades, many operate internationally. They are a powerful voice in deciding on healthcare policy and access to treatment. Importantly, many decisions are influenced or facilitated by the work of Patient Organisations. As Pharmaceutical Physicians many of us would have been involved with these types of groups in providing advice about a particular disease area and education about treatment options.

This newsletter will cover the development of these groups through the eyes of the IAPO (International Alliance of Patient Organizations). We will also look at the role and involvement of patients or patient groups within the Scientific Committees at the EMEA. As well as looking at the obligations of industry in the UK on interactions with Patient Organisations (as required by the ABPI Code of Practice).

Finally, looking more broadly, we have also included an article on the 'The Innovative Medicines Initiative.' This is a unique initiative which brings together the public sector with the European research based pharmaceutical industry in an attempt to solve the major problems besetting the discovery and development of medicines.

Dr Jit Solanki MFPM

Communications Committee



The Evolving Role of Patient Groups in Shaping Healthcare Delivery and Access to Treatment

Patient groups are increasingly recognised as an important stakeholder in the world of health and healthcare delivery. There are a growing number of groups where patients and their representatives are joining together to represent the views and support the needs of patients connected by region, disease or condition. While some groups have a history going back as far as the late nineteenth century, a boom in groups forming has occurred since 1970.

A systematic assessment of the number of patient groups, worldwide, has not been undertaken and in part this is a result of the difficulty defining and classifying patient groups. However, the International Alliance of Patients' Organizations (IAPO) conducted a survey of its membership in 2006, to better understand how many patients and patients organizations were represented through its membership. At that time, IAPO had 159 members who collectively had a network of over 4,000 organizations, representing an estimated 365 million patients. The majority of these groups were located in Western Europe and North America, at present IAPO has 28 members based in the UK. However, membership and interest in IAPO has been growing in all world regions, particularly in Africa and South East Asia.

Patient groups have evolved in very different forms, to best meet the needs of the patients they serve. Globally, patient groups can be classed as: self-help groups, small local groups of patients with a particular condition (Osaka City University Hospital Cancer Patient Support Club, Japan); local, regional and national associations of patients connected with, a particular disease or disorder, a combination of related diseases or a particular organ (American Autoimmune Related Diseases Association and the Poole Endometriosis Society which went on to become the Pelvic Pain Support Network, UK); regional or national cooperation in umbrella organizations; international umbrella organizations (Multiple Sclerosis International Federation); consumer organizations (Cochrane Consumer Network), and patient rights organizations (All Ukrainian Council for Patients' Rights and Safety).

Within these categories, there are broadly three types of patients groups:

- The organizations of people with long term medical conditions
- The organizations of parents or carers
- The organizations working for consumers of healthcare

IAPO is a unique global alliance of patient groups, like those outlined above, that represents patients of all nationalities across all disease areas. IAPO's full and associate members together work towards a vision of patient-centred healthcare throughout the world, so that healthcare systems are aligned with the needs of patients.

The majority of patient groups represent people with long term conditions whose priority is generally to live as well as possible with their condition. These concerns drive their coming together in small local groups to meet other people, discuss experiences and exchange information related to a common medical condition. In striving to manage their circumstances, groups soon realise that decisions influencing their lives are very often made on a national and international level and that policy making is not usually specific to one disease. In order to improve services and ensure their needs are met, many groups have moved into advocacy and lobbying as well as carrying out their more traditional roles.

Much of this advocacy and lobbying work focuses on ensuring that patients' views, needs and rights are at the centre of healthcare policy making, or more generally, that patients are involved in the delivery and design of their care. In a survey of IAPO's members in 2005, the policy areas of greatest importance were: access to treatment and medication, patient information, the relationships between patients and healthcare professionals and patients' rights and responsibilities. These areas form the core of many groups focus.

Despite the apparent logic of the evolution in patient groups' role, the extent to which patients are involved in healthcare policymaking is sporadic, often limited and its impact patchy. A number of factors, including questions of legitimacy and value, lack of methodologies, representation and systems culture have combined to create barriers to comprehensive and meaningful patient involvement. Nonetheless, many successful projects exist across the healthcare sector that demonstrate the value, to all stakeholders, of patient involvement.

A review of 12 pieces of research into public and patient involvement published by the UK Department of Health (DOH, 2004) found overwhelmingly positive outcomes. The most common was an improvement in patient satisfaction but other benefits were: increases in confidence, trust and understanding for patients, the

public and providers. Some of these benefits are hard to quantify but will affect overall quality, continuity and effectiveness of care.

Engaging patients and considering their experiences and knowledge is useful because it helps ensure that policies reflect patient and caregiver needs, preferences and capabilities. Patients' experiences and knowledge can shed light on how to develop integrated and comprehensive healthcare management systems which involve the patient and his or her family in their treatment, along with multi-disciplinary healthcare professionals. This helps ensure that individual patients will have the opportunity to operate in an environment that will allow them to decide about their own treatment, should they wish to. This leads to positive personal and health outcomes which make patient-centred healthcare, achieved through patient involvement, an appropriate and cost-effective way to address the needs of patients.

The added legitimacy that involving patients in decision-making is now recognised to bring, has led patients' organizations to have a small but growing influence on many healthcare providers and stakeholders, globally. This influence has ranged from designing local healthcare delivery models for long term conditions with healthcare professionals and local government, to partnering on the development of legislation to shape the research agenda and develop incentives for new medicines for patients with rare disorders.

Such cases demonstrate the increasing sophistication with which patients' organizations are able to represent patients that belies a chronic lack of funds and resources. Nonetheless, if the challenges facing healthcare systems worldwide are to be met, patient organizations need tangible assistance to continue to have a positive influence shaping the healthcare of the future.

References

Department of Health (2004) Patient and Public Involvement in Health: The Evidence for Policy Implementation.

International Alliance of Patients' Organizations (2005) Member Consultation – May 2005. Available online: www.patientsorganizations.org/consultationmay2005

International Alliance of Patients' Organizations (2007) Creating a Patient-Centred Family Healthcare Practice: A Practical Experience of Patient-Centred Collaboration. Available online: www.patientsorganizations.org/casestudies

European Organization for Rare Diseases (Eurordis) (2007) Orphan drugs: The role played by Eurordis. Available online: http://www.eurordis.org/article.php3?id_article=1529

Jeremiah Mwangi
Senior Policy Officer

International Alliance of Patients' Organizations
703 The Chandlery
50 Westminster Bridge Road
London SE1 7QY
info@patientsorganizations.org
www.patientsorganizations.org



The involvement of patients on the regulatory approval of medicines (the experience from the patients in Committees)

Scientific Committees at the EMEA are now integrating patient representatives as members. Historically, the first one to do so was the Committee for Orphan Medicinal Products established in 2000. The most recent Committees also include patient representatives: the Paediatric Committee (PDCO) and the Committee on Advanced Therapies. The appointment of patients' representatives to the PDCO has been delayed due to a complex system of appointment by both the European Parliament and the Commission, and, as a consequence, a year after its establishment, the PDCO has only recently received the appointment of patient representatives.

When revising the pharmaceutical legislation in 2001 no place was made for patients in the Committee for Medicinal Products for Human Use. This is unfortunate and the reason why the EMEA with the CHMP created a specific Working Party with Patients and Consumers.

The most significant experience to date comes from the Committee for Orphan Medicinal Products. The three patients' representatives' roles were not explicit in this committee, but they have taken their roles very seriously. They have created their sphere of activity and defined what they expected to contribute to and receive from the Committee. Since the beginning, they have contributed actively to the Committee's work as full members, not only receiving scientific evaluation and deciding on opinions, but also consistently emphasising and safeguarding the interests of patients

and in the process playing the role of watchdogs. They drive the initiatives of the Committee on various aspects of fair and speedy access to medicines across Member States and

interactions with stakeholders. They keep the pressure on other members of the Committee to maintain the focus on the primary objective of this Committee, which is to make medicines available for patients affected by rare diseases.

Patients' representatives bring a certain level of idealism in the discussions, whilst being grounded in reality. They don't like to wait to get medicines, but at the same time bring a constructive approach to public health. They retain a critical albeit constructive approach to the work done by regulators and remind them of their responsibilities.

In many difficult discussions regarding products with either efficacy regarded as too limited by regulators or rare safety issues regarded as major by regulators, they reminded regulators clearly that they, themselves, could and would make the decision to take or not to take a medicine. This brings an additional view to the regulator's perceptions of medical need as well as the patients' needs. This is particularly true for rare diseases where patients' knowledge and understanding about their disease is often more extensive than that of non-specialists.

As a spin off of their presence in the Committee or in the dedicated EMEA Working Party there is feedback provided to patient organisations, helping them to understand what regulators are doing, to appreciate the scientific level of discussions within the Committees and the independence vis-à-vis industry. Finally, patients' representatives are working with regulators on communication, transparency and information to patients in lay language, which ultimately links regulatory work and patient care.

They are also bringing a wider perspective to the Committee, as care is not just about medicinal products. For this reason, they are active in other areas such as the European Task Force on Rare Diseases, or proposing areas for research (including translational research) in collaboration with Directorate General Research.

*Dr Agnès Saint Raymond
Head of Sector
Scientific Advice, Paediatric and Orphan Drugs
European Medicines Agency*



Patient organisations & the ABPI Code of Practice

One of the major themes of the changes to the 2008 edition of the ABPI Code of Practice (The Code)¹ is transparency. This defines the requirement for the nature of the relationship between pharmaceutical companies and patient organisations (POs). As pharmaceutical physicians are the stewards of the Code and, in many companies, of compliance in general, we must take responsibility to meet the stated need of building professional relationships and to ensure that the specifics of a company's interaction with a PO are accurately described in the written agreement which must exist.

Other Codes and guidelines cover the activities of POs but these are beyond the remit of this article.

When the Code first came into being 50 years ago, and until recently, it had little to say on this subject. As it is not legal to promote products to the general public, our relationships with POs must, by definition, be non-promotional. The new Code's emphasis in this area of activity reflects the current increased concern with matters beyond promotion.

The Code has now clarified that its guidance on meetings and hospitality (Clause 19) refers to non-promotional activities as well so that constraints exercised on interactions with healthcare professionals (HCPs) during promotional activities now apply equally to HCP interactions in a non-promotional context such as advisory boards and also to interactions with the public, including POs. All such activities, therefore, must be carefully examined and certified according to Clause 14.

The need to approve and certify the agreement with a PO makes perfect sense. It cannot be good practice for either party to enter into an undefined relationship. A major part of many pharmaceutical physicians' roles is to approve written copy and we should certainly welcome the opportunity to work closely with our commercial and Legal colleagues to ensure that the relationship with a PO is appropriate and that the documentation reflects that. The agreement to supply "core funding" may be a simple one and only the written agreement requires certification but an agreement to cooperate on a specific project must define the extent of the relationship in detail and whether the company is involved or not in the activity will decide on whether certification of all the materials is required. The detail of the process is well described in guidance issued by the

PMCPA². All materials must include an acknowledgement of the role of the company.

The agreement cannot specify an exclusive relationship although, should the circumstances occur, there is no prohibition on this occurring. One may however doubt the value of such an arrangement to either party.

Pharmaceutical physicians must ensure that their company has a list of POs receiving significant (described as £500 or more ex. VAT) funding from their company. This list must be in the public domain, by means of the company website or the annual report. The support will also be declared by the POs according to their specific regulations.

Sharing promotional material with patients is of course illegal but it may be appropriate to share such material with a PO when requested in certain circumstances, such as to support a PO giving input into the NICE process. The pharmaceutical physician should be involved in such transactions and any specific materials generated would require formal certification.

In summary, the pharmaceutical physician is pivotal to the relationship between a pharmaceutical company and POs because of the particular nature of the professional relationship demanded by the Code and the company's own compliance process. The role of the pharmaceutical physician as the steward of the Code and compliance can ensure that the relationship is acceptable and transparent.

¹ ABPI Code of Practice: http://www.pmcpa.org.uk/files/sitecontent/ABPI_Code_of_Practice_2008.pdf

² PMCPA Website: Patient Organisations and the Code; <http://www.pmcpa.org.uk/?q=patientorganisationsandcode>

*Dr Peter Bowen-Davies FFPM
Chairman, FPM Communications Committee*



**ARE YOU
INVOLVED
IN THE
CONDUCT OF
PHASE I
STUDIES?**



Human Pharmacology

New training programmes for all involved in Phase I

The Faculty of Pharmaceutical Medicine of the Royal Colleges of Physicians of the United Kingdom is now enrolling trainees in its new training programmes in Human Pharmacology.

The Diploma in Human Pharmacology is a two-year programme of structured training in the workplace and courses followed by an examination for doctors intending to work as investigators for studies involving the first administrations of potential new medicines to humans.

The Certificate in Human Pharmacology is a one-year part-time programme of courses and an examination for scientists in the pharmaceutical industry, universities and regulatory authorities who have an interest in early clinical drug development.

Enquiries for the Diploma in Human Pharmacology and Certificate in Human Pharmacology should be made to the Faculty of Pharmaceutical Medicine, 1 St Andrews Place, Regent's Park London NW1 4LB
Tel: +44 (0)20 7224 0343
Email: fpm@fpm.org.uk
Web: www.fpm.org.uk

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Public-Private Partnership

– The Innovative Medicines Initiative

The Innovative Medicines Initiative is the most ambitious public private partnership ever undertaken in the life science sector. It brings together the European research-based pharmaceutical industry, which will fund its own participation, and the academic/public/SME sectors, funded by the European Commission, in a unique partnership designed to solve the major underlying problems besetting the discovery and development of new medicines. It focuses on stimulating the European life-science sector, both public and private, to collaborate in developing tools and technologies to improve the safety and efficacy of medicines through programmes of pre-competitive research. Developments in knowledge management are also vital to underpin the research projects and an extensive programme of education and training opportunities has been devised to develop the skills needed to exploit these discoveries.

The pharmaceutical industry is facing many issues: increased downward pressure on drug prices; escalating R&D costs; failure to reduce attrition along the development path; lack of willingness (in the rich countries) or inability (in the poor countries) to pay for innovation; adverse public opinion and the poor image of the industry. Despite the rather disappointing contribution of recent much heralded scientific advances to the process of R&D, the industry still believes that its recovery lies in science and technology as documented in the FDA's report of 2004 (Challenge and Opportunity on the Critical Path to New Medical Products) as well as the Strategic Research Agenda of the Innovative Medicines Initiative. The particular focus on translational medicine and the emphasis on predictive biomarkers of safety, efficacy, disease progression and treatment responsiveness are essential to reduce attrition and to produce better medicines more cost effectively. Better understanding of disease mechanisms will be needed to validate these new tools and to move from treatment to prevention of disease. To this end, productive partnership with the academic and biotech sectors will be necessary as the pharmaceutical industry looks to outsource more of its activities and to rely increasingly on the academic/biotech sector to provide it with the new tools and early stage molecules it will develop into drugs. The days when the pharmaceutical industry thought it could do all its own basic research are long gone. However, the promise of new scientific tools will not be realised if they are restricted to in-house drug discovery and if the development and approval path follows the conventional process that has been used for decades. It is essential

that the new tools become part of the development process and that we work with the regulatory agencies for greater acceptance of surrogates of efficacy, less formulaic clinical trial paradigms and better approaches to pharmacovigilance and benefit/risk analysis. True partnership and greater transparency are also the keys to reviving the tarnished image of the industry.

Europe's problems are compounded by poor public sector investment in life-science R&D, a highly fragmented research environment, and a focus on cost reduction rather than value to the patient. This has led to a loss of competitiveness of the life-science R&D sector compared with other countries, notably the US, China and India, and a loss of highly qualified people to better funded and more supportive environments (i.e. the US). The high expectations of European citizens for state-supported, first-class healthcare are completely at odds with the failure of the majority of member state governments to invest adequately in research. However, private sector investment in life science R&D is a significant contributor to Europe's research base. Consequently IMI is seen as a strategically important initiative in a key area of science that can contribute to the achievement of the Lisbon Agenda of 2000 and can perhaps stimulate the member states to invest further in this field.

As a mark of its importance, IMI was approved as a Joint Technology Initiative in December 2007, permitting it to operate eventually as an autonomous Community Body. This was a major achievement because IMI's success depends as much on its organisation as on its science. Failure to enthuse the private partners to sponsor projects and commit their in kind contributions, or a failure of the public/SME sector to respond would be the death of the initiative and the organisational aspects have been scrutinised by all sides to see whether they meet their particular needs. So far, so good. The research-based companies who are members of EFPIA responded with pledges of over €170m of in-kind contributions for the first call of IMI. This effectively released €125m of taxpayers' money from the European Commission to fund applicant consortia responding to the 18 topics of the first call. The response has been enthusiastic judging from the number of applications received.

Having said that, IMI still faces major challenges in its early years. The two stage application process has yet to be fully tested in the field although the advantages

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Public-Private Partnership

– The Innovative Medicines Initiative *continued from previous page*

it offers in terms of reduced bureaucracy seem to be widely recognised and welcomed. Despite a huge effort over more than a year to design an IPR policy that meets IMI's specific needs, there is still considerable misunderstanding and mistrust of its application in some member states and in some sectors. Therefore it remains to be seen whether project agreement negotiations will be problematical and cause delay to the start of the research work. The fixed overhead of 20% has caused dismay in many universities and there is concern that some important organisations will refuse to participate in IMI. What this all means is that IMI is still in an experimental phase but there is flexibility built into the rules and regulations that can accommodate improvements to its operations in future years if this proves necessary.

By the end of 2008, 18 awards will have been made in important areas of brain research, safety sciences, metabolic diseases, respiratory disease and education

and training, with a total value (public and private) of over €250m. In 2009, new areas of the Strategic Research Agenda will in all probability be tackled, such as cancer, infectious diseases and knowledge management. These remain to be decided but the process has started even as the assessment of the 2008 applications begins.

This has been an extraordinary year for IMI and despite all the many difficulties and challenges attending its birth, there is a sense of achievement and pride among those who have worked so long for this moment. The science undertaken will be hugely valuable but IMI's potential to transform the working practices and attitudes of all its stakeholders may be its major contribution to drug discovery and development and its true legacy.

Dr Ian Ragan

*IMI Advisor to EFPIA,
Director, CIR Consultancy*

**The 2008 Annual Meeting will take place on Monday, 17 November,
at the Royal College of Physicians London.**

For further information please see www.fpm.org.uk

How to contact us

Address:

Faculty of Pharmaceutical Medicine
1 St Andrew's Place, Regent's Park
London NW1 4LB, United Kingdom

Telephone: +44 (0)20 7224 0343

Fax: +44 (0)20 7224 5381

E-mail: fpm@fpm.org.uk

Web-site: www.fpm.org.uk

Chief Executive

Ms Kathryn Swanston Tel Ext. 22
K.Swanston@fpm.org.uk

Membership and

Finance Administrator

Mr Barry Muzzeroll Tel Ext. 21
B.Muzzeroll@fpm.org.uk

Education Administrator

Ms Laura Thornton Tel Ext. 23
L.Thornton@fpm.org.uk

Deputy Education Administrator

Mr Konrad Obiora Tel Ext. 20
K.Obiora@fpm.org.uk

PA and Administrator

Mrs Susan Paterson Tel Ext. 24
S.Paterson@fpm.org.uk

Registered charity 1011631
(England and Wales)

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