The Importance of Patient Groups and Collaboration

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1. Introduction

Historically, patient groups (PGs) were formed to offer mutual support not only for patients with serious or chronic debilitating disorders, but also their relatives and friends, who in many cases undertook primary responsibility for day-to-day care. As these groups expanded, they focused first on access to professional and institutional care as needed and to improving insofar as possible patients’ quality of life and a dignified and peaceful end. The next logical step soon followed when PGs became legal charities to raise funds to raise public awareness of particular diseases, to lobby policymakers to direct public attention and resources to obtain better access to treatment and to promote the search for more effective remedies. In essence, they became patient advocacy groups (PAGs) providing a public voice for patients and lobbying for their particular interests.

There is little doubt that PAGs have made major contributions to patients’ well-being and quality of life. In some cases, PAGs have developed resources of their own that have made material contributions to discovery of more effective treatments and sometimes to cures. However, the majority of such development has and continues to depend upon commercial interests, academic institutions or publicly funded resources.

Not only because both charitable and public financial resources are limited, but also because the research infrastructure required for both
fundamental and applied research into complex disorders, often with poorly understood aetiology, is equally limited, there is competition for the available resources. This occurs against a background where world-wide expenditure on health care falls well short of demand and, in many cases, short of actual need as well. For the general well being, it is essential that the economic, social and policy issues that arise are settled in such ways that deliver an optimal balance between patients' needs and available resources. Patient advocacy groups hold the keys to this balance, but if these are to properly applied, PAGs must broaden their perspectives to consider a patients general welfare not just that related with a specific disease that, in many cases, will be complicated by comorbid or consequential disorders.

2. The Burden of Diseases

The World Health Organisation (WHO) has developed methods to assess generally the burden of disease on societies Murray and Lopez, 1996). The diseases underlying this burden vary significantly from region to region with the burden in developing countries arising mainly from perinatal disorders, malnutrition, communicable disease and violence whilst in developed areas the burden arises mainly from non-communicable diseases (Murray and Lopez, 1996).

The WHO summarises the burden of disease in terms of DALYs – Disability Adjusted Life Years – which, in turn, is the sum of YLL – Years of Life Lost – and YLD – Years of Life with Disability. YLL
represents the reduction in life expectancy whilst YLD represents the number of years lived with disability adjusted for the incidence of disease and the severity of associated disability, a factor ranging from none (0) to death (1) (Olesen and Leonardi, 2003).

Considering the European region (roughly, all European countries west of Russia plus a few others) Olesen and Leonardi (2003) using the WHO data have calculated that “brain diseases” are responsible for 23% of the years of healthy life lost and 50% of YLDs. In this context, “brain disease” included neurological, neurosurgical and psychiatric disorders together with half of the burden of injuries and congenital problems. Considering the key summary measure of lost health, DALY, 35% were related with brain diseases (Olesen and Leonardi, 2003). Based on these data, these authors suggested that a third of the curriculum at medical school should deal with the brain and that a like proportion of life-science funding should go to basic and clinical neuroscience and that overall a third of health-care expenditure should be allocated to prevention, diagnosis and treatment of brain disease.

Given the brain disease underlies a significant proportion of health care costs in Europe, a proportion probably similar in other developed areas of the world, it will be incumbent upon patient groups to prioritise the actual needs rather than the demands of patients so that resources that may be available are applied most effectively. In this respect, all
PAGs, not just those representing neuroscience, hold the keys to resolving insofar as possible the resource / demand dilemma.

3. Responsible Advocacy

In representing patients, PAGs have a duty to listen to their patient’s wants, to filter trivial wants and to translate the remainder into prioritised needs considering in that process what is most probably achievable in the short, medium and longer term. For such an assessment, scientific and medical expertise will be required and for this most PAGs will require collaborators. Having established a set of priorities that is scientifically, clinically and financially credible in terms of a specific disease, a PAG has an equal duty to consider whether the resource demands of their priorities are not disproportionate in light of the burden of disease. Whilst PAGs naturally have a self-centred focus on specific diseases, this must necessarily be tempered by a wider social responsibility to share equitably the limited resource pool. PAGs must share with governments, policy makers as well as with medical and scientific communities the responsibility to achieve an optimum balance between resources and the needs of all patients not only the few.

Even though brain disorders underlie more than a third of Disability Adjusted Life Years (DALYs), they remain rather a “poor relation” when it comes to research. If health care is to have any hope of efficient delivery, public policy and research must target such” big ticket” issues
as brain disease. Whilst a policy change of this sort would constitute something of a paradigm shift, especially in Europe, it requires no less a shift in the perspectives of the PAGs whose “…principal goals of health care advocacy are to raise awareness of specific issues and make them national priorities… (Carroll, 2004),

Instead of competing with one another for scarce resources, PAGs must recognise that many specific disorders have important—perhaps critical—features in common and that synergy, especially at the level of fundamental science, is very likely. Rather than competing on narrowly defined disease entities, the PAGs must coalesce in broadly based consortia that have a much greater opportunity to influence public policy and to define the objectives of basic research that is certainly necessary to achieve break-through therapies for many if not most brain diseases. Application to specific disorders could then follow.

4. **Need for collaboration and partnerships**

PAGs must recognise that their roles and responsibilities, if first to their members, are not just for this particular group of patients, but also for society – for intelligent and equitable policy, for politics, friends, families, supporters, employers, scientists, health-care professionals, and no less for those who produce and supply pharmaceuticals and devices. We are all fellow travellers on a challenging journey. And we will all be patients.
If PAGs are to fulfil their wider responsibilities to public welfare, they must achieve a difficult balance between representing the needs of their patient “constituency” and the needs of the wider community of patients and society generally.

There can be no doubt that the primary objective for any patient advocacy group is to listen to and recognise the needs of its members. There is otherwise no point in its existence. However, the needs of patients and their best interests are unlikely to be most effectively served by a narrow focus that excludes the interests of other patients or those of the larger society. Whilst its member patients may applaud such an exclusive focus simply because their own disorder looms largest in the framework of scientific or medical issues that urgently require resolution, such resolution is most likely to be achieved not only through collaboration with the whole range of individuals and organisations with specific expertise, but also with other PAGs and groups who share similar representative and advocacy objectives.

5. Need for Research Partnerships and Coalitions

Because PAGs in general have neither the expertise nor the financial resources to conduct primary research themselves, when representing the interests of patients, PAGs must seek and facilitate collaboration. Although PAGs have recognised this, collaborations are usually limited to individuals or entities that are able and willing to focus narrowly on the particular disease entity the PAG represents.
For example, in a recent workshop for PAGs sponsored by ASENT (American Society for Experimental NeuroTherapeutics) a speaker suggesting ways to engage academic partners (Benderly, 2004) recommended that PAGs should try to attract promising young academic researchers who were just starting their careers. A small grant might be used to direct their attention toward areas specific to the PG. Having thus “captured” an academic, the PAG could encourage continued loyalty by contributing to the individual’s professional career by supporting grant applications to other funding bodies, by offering opportunities to publish and by other similar means. However, such methods are inherently competitive, pitting PAGs against one another in the attempt to harness the next generation of scientific and clinical expertise.

Rather than competing, albeit with the best of motives, greater progress is likely to arise from collaboration between PAGs. In many chronic, disabling conditions and certainly in neurology and psychiatry the disparate disease states as defined in the currently nosology have more in common than not and separating one from another can be very problematic in vivo. On this basis, common pathological mechanisms and perhaps of aetiology seem likely and the disease-focussed PAGs should join forces to support the search for those underlying mechanisms that could allow earlier identification of risk or that may be interrupted or modified before irreversible damage has finally overwhelmed the compensating resources of the brain.
For example, a number of neurodegenerative disorders associated with dementia (Alzheimer’s disease, Dementia Lewy Body type, Fronto-temporal dementia, Prion disease) have a different predominant pathology, but share a common mechanism of production of the hallmark pathology, namely, protein misfolding. (Hammarstrom et al, 2001; Hardy, 2003), What distinguishes their specific development is unclear, but is probably important in the search for therapies or prevention. Coalitions of all PAGs representing the disparate diseases should be formed to support research in such cases. It would also be reasonable to expect that an alliance of PAGs representing disparate diseases with some common features could encourage “thinking outside the box” that often seems to produce breakthrough concepts.

6. **Partnerships for Policy Change**

Beyond partnerships of PAGs to promote progress on issues of common interest, even wider alliances with other professional organisations undoubtedly improve both credibility and leverage when advancing the social and economic justifications for directing public policy and resources. Whether or not within an alliance, PAGs must be effective at enabling, at bringing people of disparate interests together to spawn original thinking and to induce change.

When attempting to influence both public perception and by extension public policy the size of the problem obviously matters. Groups representing a large patient population with significant demands
on state or other resources will have greater success in getting their message across. They will be more able to highlight the burden of illness substantiated by economic evidence including both direct and indirect personal, financial and societal costs.

Having got the attention of the public and policy makers, the PAGs must mediate between conflicting interests and perspectives. For example, they need to engage clinicians and health economists (who are not natural bedfellows) with patients who have a sharp focus on their distress, but very little idea of the cost. Such engagement presents real potential for conflict, as the various interests are not easily reconciled.

Even apart from economics, patients and doctors have different perspectives and priorities. PAGs have an important mediating role in listening to patients and helping them to express their needs clearly and to encourage partnership in their care. Doctors typically focus on the specific disease, the site of pathology and upon symptomatology. They measure illness, its progression and response to treatment, for example, according to neurological parameters. Patients, by contrast, focus on the effect of the illness and its treatment on their daily lives (mood, pain, sleep, bodily function, disability). Patients look at things in terms of their self-respect (work, independence) and relationships with others. From that highly personalised perspective, specialised clinical assessments are largely meaningless and the primary debilitating
effects often arise not from the underlying disease, but from such comorbid conditions as depression or anxiety, from employment or financial concerns, or from sensitivity to public stigma.

To assure that patients have access to management of all of their problems, not simply access to specialised care—important as that is—PAGs need on one hand to engender evidence-based policies, treatment guidelines and management practices based on mass statistics whilst on the other hand representing the spectrum of disease and the real problems that particular cohorts experience. These problems often cross specialist boundaries.

7. **Advocacy is not enough**

Whilst advocacy is a necessary, important and challenging function, it is not in itself sufficient. It is equally important that PAGs also take responsibility to inform and educate their members. They must provide their members not only with encouragement and support, but also with realistic expectations. Patients have a right to see a doctor who understands their disease, but also a team that can recognise associated problems and address them. Patients have a right to expect continuity of care, that is, to see the same doctor and team at successive visits. But patients have also a duty to educate themselves about their disease, to recognise and understand its possible complications and to have reasonable expectations concerning their care and to be prepared to participate fully and effectively in their own
treatment. It is a prime responsibility of the PAGs to provide and deliver this sort of patient education.

As part of this educational responsibility, PAGs need to make it clear to their members that there is fierce competition for health care resources and that patients with a particular disorder have no intrinsic right to a disproportionate share of the limited resources. Distribution of available resources is inherently political. As such politicians and policy makers are tempted to respond to the views of the much larger population of the generally healthy who tend to consider most serious those disorders that they believe present the greatest immediate risk to them personally. Patient groups representing patients with disorders that predominantly affect the elderly, including most of the degenerative neurological diseases, are disadvantaged in the competition for resources simply because the general public consider these to be remote risks.

Neurological disorders are often considered a threat only to the old and victims are largely hidden from society, whilst psychiatric disorders are associated with public stigma and likewise hidden or denied wherever possible. PAGs representing these patients must strive to illustrate the burden of these disorders and to raise their profile in public perception. In terms of DALYs and consequent direct and indirect costs to society, brain diseases are very important. In the past, spouses or other members of the immediate family have absorbed much of this
personal as well as financial cost. However, increasing dispersion of the extended family as well as dissolution of the nuclear family coupled with increasing full-time employment for both sexes results in an increasing responsibility of the state to provide care. This is inherently expensive and exacerbated by the demographic reality of an increasing proportion of older people in the population.

It is perhaps natural and certainly understandable that diseases that lead to premature death are for most frightening. Understanding the personal impact of an illness that gradually erodes the senses, intellectual function or the ability to perform simple motor tasks is much harder for the healthy to imagine. It therefore devolves to the PAGs to use every possible tool at their disposal to educate the healthy not only to recognise their personal risk, but more important to gain some concept of the impact of living with a disability that will only become more severe. It must be obvious that the risk of a single disorder such as Parkinson’s disease is small compared to the risk of one of the range of equally or more debilitating degenerative neurological conditions. Again, fragmenting the message among many specific disorders, each equally devastating, can hardly be as effective as a coordinated message. The PAGs have a duty to their members if not to society generally to collaborate in the effort to educate the public, the politicians and the policy makers.

8. Research and development issues
In the same way, the PAGs have a duty to collaborate when representing their members in the many areas of common interest such as the ethical dimensions of stem cell or animal research. The effort to relieve the suffering of patients should not be compromised by the fact that some necessary aspects of scientific and clinical research may offend the sensibilities of those with dogmatic views however fervently held.

Collaboration of PAGs is also necessary if a uniform standard of care is to be accessible to members regardless of their disease, their station in life or where they live. There are, for examples, significant differences in reimbursement and in the time lags for access to new treatments.

Regulatory requirements and the associated time costs of drug development coupled with a high risk of failure and short patent terms constrain development of new drugs. In particular, development for relatively rare conditions is unlikely. In addition, the cost of new drugs is necessarily high to recoup development costs not only for the one registered drug, but also for the three or four that failed in development. Short patent terms mean that most cost recovery has to be achieved before the patent expires and manufacturers of generics who contribute nothing to research or development flood the market. If the PAGs wish to encourage new therapies, they need to collaborate to lobby for
regulatory changes that will encourage rather than stifle research and development.

9. **What next for patient advocacy**

Finally, whilst there is no doubt that PAGs have made major contributions to the well being of their patient groups and that continuing such contributions will be required in future, changing times now require that the PAGs should seriously consider altering their operating methods in areas of mutual interest.

In the encouragement and / or sponsorship of research into the underlying mechanisms of neurological disorders, a broader scope of enquiry that could promote lateral thinking should prove more productive than one based upon a narrow focus on a single disease entity. Close collaboration of several PAGs would be most appropriate and perhaps necessary to engender such research.

In particular, there is compelling evidence from the WHO for the importance of brain disorders, mainly neurological and psychiatric, among the diseases contributing most significantly to the global burden of disease. Fragmenting the voice of all affected patients along disease-specific lines is counter-productive in the competition for an equitable distribution of limited research, development and care-centred resources.

Whilst it may well be desirable that disease-centred PAGs should retain a measure of individual identity and autonomy, a much greater
measure of association and collaboration among the PAGs dealing with brain-related diseases will be necessary if they are to serve most effectively the needs of patients and to achieve a share of resources commensurate with the impact of brain diseases on individuals, society and the health-care systems.
10. References


