

Patient empowerment of people living with rare diseases. Its contribution to sustainable and resilient healthcare systems

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Abstract

The notion of empowerment is linked to patients' everyday life and is the base allowing for the patient engagement through which individuals and communities are able to express their needs, are involved in decision-making, take action to meet those needs. In the field of rare diseases, empowerment strategies have greater value due to low prevalence, lack of expertise, poor quality of life. Avenues to patient empowerment are: health literacy and capacity-building; shared decision-making; support to self-management. Patient empowerment is recognised as key enabler in creating sustainability as addressing challenges faced by modern healthcare systems in terms of effectiveness, access and resilience. It is recommended to develop a comprehensive EU roadmap on patient empowerment including specific recommendations, taking stock of good practices. This holistic approach should lead to a society where all actors are fulfilled human beings and unmet needs are addressed in compliance with fundamental human rights.

Key words

- patient empowerment
- rare diseases
- patient engagement
- healthcare systems
- sustainability

INTRODUCTION

History and concept

The many *origins and sources of inspiration of the notion of empowerment* can be traced back to such varied domains as feminism, Freudian psychology, theology, the Black Power movement, and Gandhism. Another important influence to the empowerment “philosophy” appears to be the work of Paolo Freire and his concept of “popular education” developed in the 1960s, which became influential in development practices in Latin America in the 1970s, associated particularly with literacy projects [1]. However, it is not until the 1970s, especially the 1976 publication of *Black empowerment. Social work in oppressed communities* by Barbara Solomon, that the term formally comes into use by social service providers and researchers. In the 1980s, empowerment was seen, for the most part, as a radical project of social transformation, to enable otherwise excluded social groups to define and claim their rights collectively [2]. A notable example related specifically to the field of health and people living with a disease, is the self-empowerment movement of people living with AIDS during the 1980s, particularly the drafting of their 1983 “Denver Principles” where the idea that personal experiences should shape the AIDS response was first

voiced [3] and in which they made their seminal declaration stating: “We condemn attempts to label us as ‘victims’, a term which implies defeat, and we are only occasionally ‘patients’, a term which implies passivity, helplessness, and dependence upon the care of others. We are ‘People With AIDS’” [4]. It is therefore in the context of various social movements that the word ‘empowerment’ begins to be used increasingly in research and intervention concerning groups experiencing marginalisation, such as African Americans, women, gays and lesbians, and people with disabilities.

From this historic overview, it appears that empowerment refers in essence to principles such as the ability of individuals and groups to act in order to ensure their own well-being or their right to participate in any decision-making that concerns them. However, empowerment is a complex concept that has been used in different ways. Gibson suggested that “Empowerment is [...] easier understood by its absence: powerlessness, helplessness, hopelessness, alienation, victimisation, subordination, oppression, paternalism, loss of a sense of control over one's life and dependency” [5]. In addition, not only are we lacking an agreed upon definition of “empowerment”, but the word itself does not translate easily or equally. For example, in both German and

French, the English expression “to empower” can be translated into two different verbs: *ermächtigen/autoriser* (which suggests “power over”) on the one hand, and *befähigen/rendre capable* (“power to”) on the other [2]. There are other possibilities for a French translation: the Quebec French dictionary uses the word *autonomisation*; the World Bank [6] uses the words “demarginalisation” and “integration”. Empowerment is also found in the literature as “renforcement des capacités et participation” [6].

Another issue of definition is the distinction between empowerment being a process/approach or being a goal/outcome, although these two concepts are not necessarily disparate: The World Bank, for instance, defines “empowerment as the process of enhancing the capacity of individuals or groups to make choices and to transform those choices into desired actions, which both build individual and collective assets, and improve the efficiency and fairness of the organisational and institutional context which govern the use of these assets” [7].

In the field of healthcare, the term *patient empowerment* gained prominence as part of a move away from paternalism towards more equitable and collaborative models of healthcare delivery, with the potential for improving cost-effectiveness of care, especially for people affected by long term conditions [8]. In this sense, empowerment is viewed as a process of “activating” patients, who as a result of “rejecting the passivity of sick role behaviour and assuming responsibility for their care (...), are more knowledgeable about, satisfied with, and committed to their treatment regimens” [9]. The European Patients’ Forum explains this distinction between the individual (patient) level and the collective (patient community) level as the difference between gaining personal skills in the former, and creating mutual support groups, identifying issues through the sharing of information and creating a coalition with potential for advocacy in the latter [10]. Another important nuance highlighted in the EU-funded EMPATHiE project (“Empowering Patients in the Management of Chronic Diseases”) is the fact that “an empowered patient has control over the management of their condition in daily life” and they “develop self-confidence, self-esteem and coping skills to manage the physical, emotional and social impacts of illness in everyday life” (www.eu-patient.eu/contentassets/543c15ed8f8c40f692030a0a0d51b8e2/empathie_frep_en.pdf). Therefore, patient empowerment can go beyond solely changing the way one copes and manages healthcare strategies, but also enter the realm of everyday life.

The fact that patient empowerment as a process can occur at the individual level and at the collective level, as well as in the medical context and outside of it, means that patient empowerment can have an impact at: *micro* (patient) level, in terms of improved health, greater satisfaction with the treatment option and better quality of life and psychological state; at *macro* (community) level in terms of quality of health and social services and design of interventions responding better to needs; and at *policy* level in terms of policy prioritisation, design, and cost-effectiveness [10]. In view of these elements, four components can be considered as fundamental to

the process of patient empowerment: 1) understanding by the patient of his/her role; 2) acquisition by patients of sufficient knowledge to be able to engage with their healthcare provider; 3) patient skills; and 4) the presence of a facilitating environment. In sum, although the term can have different meanings and interpretations, *patient empowerment* generally refers to the process that allows an individual or a community to gain the knowledge, skills, and attitude needed to make choices about their care.

In the field of *rare diseases* this process of empowerment takes on an even greater value. First off, because, due to the low prevalence and the lack of expertise and adequate information, the patient is forced to become knowledgeable about his own disease state. Moreover, people living with a rare disease are often at high risk of experiencing poor quality of life, including increased levels of anxiety, depression, pain, fatigue and limited ability to participate in society. The results of the Rare-Barometer survey “Juggling day care and daily life” [11], developed in the framework of the InnovCare project (www.innovcare.eu), with over 3000 respondents, provides detailed evidence of this reality. This has made it clear that, while each disease may have its own set of characteristics and health impacts, people living with a rare disease often share similar experiences and challenges – including limited information, no treatment, lengthy times to diagnosis and isolation. In addition, it is often the very rarity of rare diseases that has left them largely disregarded by the research or medical community and policy makers.

To overcome these difficulties, empowerment, defined as an action-oriented notion with the focus on removal of formal or informal barriers, and on transformation of relations between communities and institutions, becomes a necessity for people living with a rare disease. It becomes crucial to look at this group collectively, rather than at each rare disease independently, to identify the common problems and gather the actors (critical mass) for joint actions (collaborative work) towards the strengthening of the actors’ abilities and their recognition in the social field. This realisation has led to patient empowerment being effectively applied in the rare diseases community over the last few decades. Empowerment strategies in the rare diseases community include improving access to information on the basis of indigenous knowledge, finding supportive groups, and establishing dialogues, and they have led to reduced social exclusion and health disparities, and built a sense of community [12]. These strategies occur at both the individual (patient) level as well as at the community level.

Firstly, it is important to highlight that *individual* empowerment of people living with a rare disease has been mediated by the rapid growth of web-based health-related information, notably by:

- the possibility of establishing or joining online cross-border communities for people with the same disease or the same problem, of which the platform of online patient communities RareConnect.org is a prominent example in terms of both disease and geographical coverage;



- and by the availability of help lines and other support services often offered by patients' organisations. In particular, help lines, supported by the European Network of Help Lines for Rare Diseases (www.eurordis.org/content/help-line-services), play an essential role in providing the often lacking information that patients face, especially in the absence of disease-specific patient organisations.

In addition, another key component of individual empowerment is the ability to self-care, including the self-management of the condition. This ability of the patient to deal with all the elements of its condition, including symptoms, treatment, physical and psychosocial consequences, and lifestyle changes, requires the *coordinated* approach between physicians, patients, care-givers, and health care organisations. Self-management support allows the development of confidence, self-efficacy and skills to control daily life. In the case of people living with rare diseases, it is often the case that patients themselves and in particular, family members, act as primary carers. However, research reveals that self-management and the collective support required to achieve it is far from being an easy task [11].

Secondly, it is crucial to point out the key role of *patients' organisations*. "Rare disease patient organisations have been created as a result of experience gained by patients and their families from being so often excluded from health care systems and thus having to take charge of their own disease themselves. The scientific community's inadequate knowledge of rare diseases and the scarce attention given to them by national competent authorities and the pharmaceutical industry has led to the creation of associations of patients and parents. Rare disease patient associations aim at gathering, producing and disseminating the limited existing information on their disease and making patients and parents voices' heard. Their actions have already ensured progress in healthcare and social assistance provided to patients and carers, development of treatments, and in raising public awareness of rare diseases" [11]. In this sense, patient organisations act at both the *individual* and *collective* levels of patient empowerment. In fact, the very nature of rare diseases patient organisations places "patient empowerment" at the core of their mission. For EURORDIS-Rare Diseases Europe, the EU alliance of rare disease patient organisations, "patient empowerment" is one of the three key components of its tripartite mission and is centred on information sharing, community-building, networking opportunities for members and capacity-building for patients.

MATERIAL AND METHOD

The document is the result of a review of literature on topic, patient advocates' testimonials, EU policy documents and legislation, results of surveys performed under the RareBarometer survey programme (www.eurordis.org/voices), as well as consultation with EURORDIS (www.eurordis.org) European Public Affairs Committee (EPAC). The brief, after shedding light on the concept of "patient empowerment" and its historic development, tries to define the way in which patient empowerment is implemented and to identify the link

between patient empowerment and equity, resilience and sustainability of healthcare systems.

RESULTS

a) What is an empowered patient?

The Alma Ata Declaration issued by the World Health Organisation (WHO) as far back as 1978 stated that: "The people have the right and duty to participate individually and collectively in the planning and implementation of their healthcare" [13]. In 2006, the Council of the European Union declared that patient involvement is a common operating principle in the health systems of the European Union [14]. However, today this is still not the patient's experience on a daily basis. But, what is an empowered patient? And what is the distinction between an empowered and an engaged patient?

In health care, the rapid proliferation of health information on the Internet has resulted in more patients turning to the Internet, particularly social networks such as PatientsLikeMe (www.patientslikeme.com/), Connected Living (www.connectedliving.com/) or RareConnect, specifically for rare diseases) as their first source of health information. This rapid evolution of digital technologies has shifted the focus to sharing and co-creation of knowledge. This has radically changed the way patients interact with institutions, with the media and with other stakeholders in the reference context.

However, the information patients access independently will vary in quality. This wealth of information can be very powerful, but it can also be confusing, in particular if the individual patient is not able to use effectively the information. In this respect, empowering patients means enabling them to make good use of their available knowledge by providing tools, techniques, and support that allow them to actively participate in the management of their health and life if they wish so. However, empowerment is not a simple process, nor is it necessarily linear, and it is not a process that can be imposed, only facilitated [10].

Moreover, it is not always easy to distinguish clearly between patient empowerment and patient involvement/engagement. Generally speaking, only once a patient is empowered, can a patient be engaged, the difference between the two being that the former (patient empowerment) is the common base allowing for the latter (patient engagement). The concept of patient involvement refers specifically to the rights and the benefits of patients to have a central position in the healthcare process, it goes beyond the availability of information or health literacy: it is about the interaction between the patient and the healthcare provider and encompasses a wide range of different aspects.

Recently, the EU-funded EMPATHiE project developed the following working definition of an empowered patient which combines both terms: "An empowered patient has control over the management of their condition in daily life. They take action to improve the quality of their life and have the necessary knowledge, skills, attitudes and self-awareness to adjust their behaviour and to work in partnership with others where necessary, to achieve optimal well-being. Empowerment interventions aim to equip patients (and their informal caregivers

ers whenever appropriate) with the capacity to participate in decisions related to their condition to the extent that they wish to do so; to become “co-managers” of their condition in partnership with health professionals; and to develop self-confidence, self-esteem and coping skills to manage the physical, emotional and social impacts of illness in everyday life” [10].

Additionally, and in line with the scheme illustrated by Christopher Rissell [15], the European Patients’ Forum (EPF) proposes a definition of empowerment that includes the significant role of patient organisations in advocating for the patient perspective at policy level, this incorporating both the individual (patient) and the collective (patient community) dimension of empowerment.

Several countries have put patients at the centre of their healthcare policy [16]. The NHS in the UK, for example, has promoted the concept of patient involvement using the well-known slogan: “No decision about me without me” [17]. At the EU level, a remarkable example of the adoption of the latter principle is the 2015 Addendum to EUCERD Recommendations on European Reference Networks (ERNs) for Rare Diseases (31 January 2013) whereby “Patients and patient representatives should play an integral role in the decision and opinion making process in RD ERNs and be involved in structural and clinical network activities. It is recommended that RD ERNs demonstrate meaningful patient involvement, patient-centredness and empowerment through recognition of the role of patients, as experts by experience and co-producers of knowledge, in RD ERN structural and clinical activities and therefore demonstrate meeting the legal requirements in the Delegated Acts [18]”. This principle has been translated into the governance of the existing 24 ERNs and the involvement of ePAGs (European Patient Advocacy Groups) in each ERN.

To sum up, empowerment is “a process through which individuals and communities are able to express their needs, present their concerns, devise strategies for involvement in decision-making, and take political, social, and cultural action to meet those needs [10]”. In particular, for rare disease patients, this translates in a process leading to collective intelligence to solve complex problems, it is continuous production and improvement of information, knowledge expertise as well as support and solutions [19].

Empowerment of patients is a task which involves and encourages interaction of communities, health care professionals, policy-makers and all other civil society actors with respect to health and wellbeing of individuals. “Empowering patients means providing them with the opportunities and the environment to develop the skills, confidence and knowledge to move from being a passive recipient of care to being an active partner. Empowerment needs to take place simultaneously both at the population and the individual level. It therefore requires a cultural change, involving a change in the balance of power to recognise chronic patients as experts in their own care by experience and also “co-managers” of their condition in partnership with health professionals” [20].

This change can be achieved by focusing on education and training for all stakeholders, patients, health professionals and institutions. Revitalising public health requires rethinking education of health professionals to produce a more flexible, multi-skilled workforce able to meet the growing challenges and to support patient empowerment and consumers with the adequate level of health literacy, and fostering communication, management and leadership capacities at all levels. It will be thus possible to promote innovative and high-quality, truly patient-centred, sustainable health systems of the future.

b) How is a patient empowered?

Taking control of one’s own health involves more than just gaining a voice. For this reason, there are many ways in which patients can be empowered. The following are often identified as key ones:

Health literacy, education and capacity-building.

– Although empowerment involves much more than becoming an educated/informed patient, the right information and resources are fundamental tools for empowerment. The WHO [21] highlighted that limited health literacy negatively affects health, reinforces health inequalities, especially among poorer populations, and leads to higher healthcare system costs. Improving health literacy and education not only empowers patients but also contributes to the sustainability of healthcare systems.

Health literacy is a dynamic, interactive process whereby the information is critically analysed and applied to influence or take action to change the community. Patients need to know where to find reliable information from trustworthy sources. In this sense, the European Commission’s recent paper on Digital Health Literacy focused on the use of the Internet to search for health-related information. The EU eHealth Action Plan 2012-2020 – Innovative Healthcare for the 21st Century also looks at how greater access to services and information and the use of social media for health can strengthen patient-centred care and empowerment [22].

Health literacy addresses the environmental, political and social factors that determine health. Health education also encompasses capacity-building and aims to influence not only individual lifestyle decisions, but also raises awareness of the determinants of health, and encourages individual and collective actions which may lead to a modification of these determinants. Health education is achieved therefore, through methods that go beyond information diffusion and entail interaction, participation and critical analysis. Such health education leads to health literacy, leading to personal and social benefit by enabling effective community action, and contributing to the development of social capital [23].

Patient organisations often fulfil the task of ensuring education for patients and healthcare professionals by developing and often funding helplines, information and developing ad hoc trainings for patients so that they can become fully-fledged partners in decision-making and/or in the relations with other actors they

come across in their healthcare pathway. In the field of rare diseases, trainings such as the EURORDIS Open Academy (www.eurordis.org/content/eurordis-open-academy#tabs-1) or the EUPATI programme, are extremely important to build patients capacities and to allow them to understand the scientific landscape, to discuss with researchers, medical staff and industry on equal foot, or to develop those personal skills that are essential for the interactions with other healthcare stakeholders.

Umbrella patient organisations that acting at the national level also play a crucial role in capacity-building. By pooling patients across the country together and acting as a single voice, national patient organisations are able to contribute to empowerment at all three levels (micro, macro and policy). In particular, they are able to provide input for appropriate policy, research and health provision that responds to the needs of the constituent population. In addition, this collective national grouping facilitates the interaction with similar groupings from other countries in order to exchange best practices and learn from the successes and challenges experienced in different contexts.

Shared decision-making and Integration of patients' views. – Shared decision making (SDM) has been defined as: “an approach where clinicians and patients share the best available evidence when faced with the task of making decisions, and where patients are supported to consider options and to achieve informed preferences [24]”. In sum, shared decision-making is the process by which a clinician and a patient jointly make a health decision after discussing options, potential benefits and harms, and considering the patient's values and preferences. At its core, shared decision-making rests on accepting that individual self-determination is a desirable goal and that clinicians need to support patients to achieve this goal, wherever feasible. Self-determination in the context of SDM does not mean that individuals are abandoned. SDM recognises the need to support autonomy by building good relationships, respecting both individual competence and interdependence on others.

The key to understanding this concept is to recognise that in every medical or health decision, there are at least two sources of expertise for making a good choice. The clinician has had lengthy and extensive medical training; but only the patient knows his/her attitude to risk, how the illness is experienced in his/her particular social circumstances, and his/her values and preferences. Therefore, “the Patients as Partners concept (...) takes patient engagement even further by considering the patient as a full-fledged member of the healthcare team (...) Chronically ill patients develop experiential knowledge that can enhance their self-managed care and complement the scientific knowledge of healthcare professionals. In other words, from a patient's perspective, quality healthcare decisions are based on two complementary forms of knowledge: the scientific knowledge of health professionals and the patient's own experiential knowledge” [25].

An example of success in pursuing the integration of

patients' views is the recent creation of the European Patient Advocacy Groups (ePAGs, www.eurordis.org/content/epags), patient representatives ensuring that patients are fully represented in the governance of each and every European Reference Networks, promoting and encouraging a patient-centric approach in both delivery of clinical care, service improvement and strategic development and decision-making, in line with the above-mentioned Addendum of the EUCERD Recommendation's on ERNs.

In sum, the approach to healthcare where health professionals make all decisions with little or no input from the patient has evolved over the last decades towards a patient model whereby individual patients' needs, values, and experiences are crucial for personalised and person-centred healthcare. *Patient participation or engagement is recently receiving greater attention, as it becomes increasingly evident that it can be an innovative and viable approach to ensuring appropriate care in the current environment strained by limited resources.*

Support to self-care and self-management. – Self-care is increasingly viewed as a core component of the management of long-term conditions. The majority of care for chronic conditions is provided and coordinated by the people themselves, with the support of family members and carers, at home and in the community [26]. This also applies to people living with a rare disease and their families.

Self-management is defined as the ability of the patient to deal with all that a chronic illness entails, including symptoms, treatment, physical and social consequences, and lifestyle changes. For self-management to be effective, it needs to rely on the proactive engagement of physicians, patients, care-givers, and health care organisations. The coordinated approach between all those involved will enhance patient care and more effectively treat patients with multiple chronic conditions. Self-management plays a crucial role for those patients who suffer from multiple chronic conditions or multi-symptomatic diseases, such as the majority of rare diseases. With effective self-management, the patient can monitor his or her condition and make whatever cognitive, behavioural, and emotional changes needed to maintain a satisfactory quality of life [27]. Many prevalent chronic conditions, including rare diseases, although unique in their own attributes and demands, share common challenges associated with their management, that include dealing with symptoms and disability; monitoring physical indicators; managing complex medication regimens; maintaining proper levels of nutrition, diet, and exercise; adjusting to the psychological and social demands, including difficult lifestyle adjustments; and engaging in effective interactions with health care providers.

For people living with a rare disease, however, self-management (and the collective support required to achieve it) is far from being the reality. In the survey *Juggling rare diseases and daily lives* [11], one of the highest needs expressed is the “request for services that help patients to maintain their autonomy and help self-management of the disease, including rehabilitation services

and therapies (48%), psychological support (47%), support to adapt house to the needs (30%), medical devices (28%) and adapted transports (23%). Autonomy is particularly at risk in cases in which the person needs help with their most basic and private needs or when the impairment affects his/her ability to communicate" [11].

Self-management support allows the development of confidence, self-efficacy and skills to control daily life, provided that is carried out according and in respecting each person's individual needs and preferences. Self-management support, as a consequence, *has a positive impact on the healthcare systems as it helps make best use of all available resources by improving adherence, reducing hospitalisation and emergency visits, and improving health outcomes* [10].

Self-management programs such as the Arthritis Self-Management Program (ASMP) and the Diabetes Self-Management Program in the US demonstrated the feasibility of self-management intervention programmes, leading to positive health outcomes, many of which persist for years, with an added benefit of reduced health care costs [28, 29].

DISCUSSION

How to empower rare disease patients in sustainable healthcare systems

From what is illustrated above it emerges clearly that achieving patient empowerment is a key enabler in the process of creating sustainable and equitable systems for all, thus addressing the challenges faced by modern healthcare systems (demographic shift, increased burden of chronic diseases, increasing costs of treatments and care, limited access to innovative care, intra-European difference, etc.). The European Patients' Forum's latest campaign highlight five elements of patient empowerment (1. education, 2. expertise, 3. equality, 4. experience and 5. engagement) which demonstrate that patients are active people who can, if supported and according to their individual capabilities and situation, make a difference for the sustainability of healthcare systems (www.eu-patient.eu/campaign/Patientsprescribe/).

This is recognised in the 2014 Communication from the European Commission on effective, accessible and resilient health systems where the impact of patient empowerment on health systems is explained in terms of:

1) *effectiveness*: positive clinical outcomes, improved quality of life, better use of health services, which respond the needs of patients;

2) *access*: more transparency and understanding of the healthcare system by the patient can ensue in better access to healthcare; also, empowered patients can advocate for improved access;

3) *resilience*: information flow in the system has proved to improve the resilience of health systems [30].

In fact, an important element is that results of patient empowerment beginning at "micro level" then produces "macro effects".

At the *micro (individual) level*, the first benefit experienced by the patient is improved health (i.e. quantity and quality of life years gained): by empowering patients it is be possible to achieve results in terms of more appropriate and effective use of healthcare resources,

lower use of drugs, less health inequalities among population, less treatment errors and an increased use of preventive services. In addition, as highlighted in the EMPATHiE project, additional outcomes of patient empowerment include greater patient satisfaction, professional satisfaction, increased quality of life, improved clinical outcomes and better use of health services [31]. Patients who are empowered to make decisions about their health have greater satisfaction because the chosen treatment or screening option better reflects their personal preferences, needs and values and the cost-benefit relation that the patient and her/his family see as appropriate. In the long-term this leads to considerable advantages in terms of psychological state and quality of life, as well as in savings for health service costs. For the adult population this also translates into workplace productivity gains, experienced by patients, their family and their employers.

At the *macro level*, the empowerment of patients and their representatives has a positive impact on the quality of health and social services. There is a political acknowledgement at EU level that patients should be "at the centre" of healthcare systems and that to achieve this, patients' representative organisations should be part of healthcare-related policy and decision-making [32]. Collective patient empowerment, through the meaningful involvement of patient representatives and organisations in health policy, service design and evaluation with the aim to improve the system, will be the key to fostering patient empowerment in all EU health systems and will lead to public spending on healthcare that is more explicitly linked to population health needs.

At the *highest (or policy) level*, through their representative organisations, patients can offer guidance to decision-makers on quality care that is also cost-effective, and can therefore contribute to the debate for re-shaping healthcare systems. Over the years, patient groups have been giving individual patients a stronger voice. They are also involved in disease education for patients and healthcare professionals by providing helplines, emotional support, information, access to treatment and generally fighting for patients' rights. And they conduct advocacy discussions about the healthcare system and play a crucial role to show real and concrete need of patients and citizens.

By their individual and collective actions, empowered patients have therefore a crucial role in promoting actions that lead to reduction of inequalities by correcting social injustices affecting the patients themselves and by offering them back to society [19]. Patient empowerment thus helps reduce the multiple vulnerabilities (physical, moral, social and economic) and proposes a new concept of collective and collaborative production of common goods, which needs collective and collaborative action, in order to make the best possible choices for all.

Therefore, patient empowerment addresses the serious unmet social needs of people living with a rare disease and their families which affect their dignity, autonomy and other fundamental human rights expressed in the Universal Declaration of Human Rights and in the UN Convention of the Rights of Persons with Disabilities.

In this sense, “sustainable” is what ensures equity of access so as to fulfil the human rights of people whose needs are often seriously unmet, in order to make them empowered citizens. Only with dignified and fulfilled citizens a society can be said sustainable.

CONCLUSIONS

Achieving patient empowerment is a key enabler in the process of creating sustainable and equitable systems for all. To help this process, a number of recommendations were made a few years ago through the EUROPLAN project (www.europlanproject.eu). Patient empowerment was a specific area addressed in the Recommendations for National Plans and Strategies for Rare Diseases Conferences developed by EUROPLAN [33] and in the 15 EUROPLAN National Conferences [34].

Based on the above and on other activities highlighted in this policy brief, including those carried out by EURORDIS and other patient groups, it appears that a number of potential initiatives strengthen patient empowerment in the rare disease community and deserve being supported, notably:

- Ensure an active role and effective engagement of patients at all key decision-making points at the national/regional/local level (“nothing about me without me”). Specifically, but not exhaustively:
 - Involve patients in Steering Committees or equivalent bodies overseeing the implementation and/or the monitoring /evaluation of National Plans or Strategies for rare diseases;
 - Include patients in the evaluation of the opportunity/costs of policy planning on RDs – basically any time “benefit” is to be assessed;
 - Recognise the role of patients/patient representatives in hospitals, centres of expertise and other local decision-making bodies relevant for specific diseases or groups of diseases.
- Integrate patients in clinical trial design focusing on meaningful endpoints and patient outcomes.
- Promote patients’ training, educational and capacity building initiatives, so to allow patients to become fully-fledged partners in decision-making. On clinical trial design for example, training is extremely important to allow patients to understand the scientific landscape and to discuss with researchers, medical staff and industry on equal foot.
- Support patient empowerment initiatives through public money in order to guarantee an effective contribution of patients as equal partners.
- Improve awareness-raising on the role of patients in the decision making process to improve public perception.
- Guarantee that the progress made in empowering patients and in rare disease policy in general be maintained in spite of changes in government and volatile political backgrounds.
- Ensure that medical and paramedical staff receive adequate professional training to help them identify and treat rare diseases, but also to help them interact with patients in a context of shared decision-making.
- Promote the creation of telephone help lines that play

an essential role for the provision of general and specific information on rare diseases, with the promotion of best practice exchanges and quality assurance initiatives.

- Develop or reinforce the role of “Case managers” who could address the specific range of needs of the rare disease patient and help them navigate the complex environment.
- Promote self-management initiatives adapted to people living with rare diseases, tailored to each disease or disease group, ideally designed in the context of European Reference Networks, to accompany disease-specific ‘strategies of care’ or clinical guidelines.
- Adapt social services so that they cater to the needs of patients with RDs and promote the inclusion of rare disease specificities in national social policies – allow integration of people living with rare diseases in school, workplace and social life in general.

There are currently a number of such initiatives that qualify as promoting patient empowerment and thus promoting more sustainable healthcare systems, however these are scattered and unevenly applied across the EU countries and regions.

It may therefore be argued that the ultimate recommendation could be to develop a roadmap or strategy at EU level on patient empowerment that includes the above specific recommendations and takes stock of experiences and best practices existing across the EU or beyond, encouraging their uptake in a comprehensive and systematic framework. Such a strategy would be applied to the appropriate level(s) of governance (European, national or local) to ensure that patient empowerment becomes a reality and contributes to improved healthcare across Europe’s national systems.

In conclusion, such a strategy would be one whereby the attainment of the highest possible standards of health depends on a comprehensive, holistic approach which goes beyond the traditional curative care, involving patients, communities, health providers and other stakeholders. This holistic approach should empower individuals and communities to take actions for their own health, foster equality in partnership for public health, promote intersectoral action to build healthy public policies and create sustainable health systems in the society, where all actors are fulfilled human beings and unmet needs are addressed in compliance with fundamental human rights.

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