

**THE ADDED VALUE OF  
PATIENT  
ORGANISATIONS**



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# Executive Summary

The European Patients Forum (EPF) commissioned this report to give an overview of the role of patient organisations in Europe, to highlight their value as legitimate stakeholders in civil dialogue in health-related policies and to draw attention to the challenges patient organisations are facing. The objective of the report is to emphasise the contribution of patient organisations in representing and voicing the situation of a specific population that would otherwise not be represented. The main activities of patient organisations are set out in four different areas: policy, capacity-building and education, peer support and research & development (both health and pharmaceutical).

## Policy

Patient organisations are invaluable partners in the policy process, providing input through stakeholder advisory groups, expert panels, European and/or national government public consultations or institutional meetings. Patient organisations are able to help policy-makers understand the experience of living with a disease or a condition. They use this “end-user perspective” to promote the interests of patients at all stages of policy development and in a range of institutional settings. Their contributions are valuable from the cross-sectoral approach of Health in all Policies (HiAP) to health and other essential services design. Through representation, mobilisation and empowerment, patient groups combine individual and social actions to gain political commitment and public support for specific patient and general population health issues. They are experts in channeling the voice of patients by representing patients’ interests in a united, coherent and consistent way which enhances the overall balance and nuance in policy-making.

## Capacity Building and Education

Patient organisations work to strengthen the organisational and governance structures of their constituencies with the aim of helping patient groups become more resilient, sustainable and effective. Patient organisations also invest in capacity-building for policy-makers, industry, academia and the media. They undertake educational initiatives to disseminate information from end-users to policy-makers and authorities and vice-versa, as well as between different stakeholders. They work to promote health literacy to help patients make sound health care decisions for themselves. They produce and review health-related information with the aim of making sure that information provided to patients is of high quality and accessible.

## Peer Support

Patient organisations provide peer mentoring, counseling or listening services, and legal and financial support. In their role as peer supporters they co-deliver self-management education and deliver various forms of support into the wider patient community.

## Research and Development: Health and Pharmaceuticals

Patient organisations are increasingly active research collaborators, including through setting of research priorities and data collection. They are avid advocates for greater involvement of patients in the early stages of pharmaceutical research and development, as well as Health Technology Assessment (HTA), and argue for a similar approach in research and the development of disruptive innovation. They help patients navigate the complexities of the regulatory process for medicines and raise awareness about this with the pharmaceutical industry.

## Challenges

Patient organisations face many challenges when performing these activities. The lack of resources and funding is an ongoing problem in all areas of their work. There is also an overarching issue of credibility impacted by the professionalisation of the sector that can disconnect it from its base and threaten its representativeness. Furthermore, there is a systemic failure of cooperation and a culture and tradition of tokenism when it comes to working with patient organisations. A regulated and coherent legislative space that would ensure a right of access to independent, timely and adequate resources is also missing on the European level. This means that the contribution of patient organisations to health (and society) in Europe is potentially greater than it is today.

## Key messages

Patient organisations need to keep their eyes on the future by focusing on their core principles: those of representing, mobilising and empowering patients and advocating for their rights. They could consider ways to educate external stakeholders about what they do, the significance of the added value that they bring, and why and how they work with industry partners. They can use the revolution in communications, technology, innovation, research and development to advance their connectivity, engagement and resilience in a changing world. But patient organisations cannot face these challenges alone: other actors have an important role to play as well. The following recommendations can be taken into account by other stakeholders.

## All stakeholders should

1.

Acknowledge that patient representatives must be treated on a par with other types of experts, such as scientists, industry representatives and medical professionals, and should be compensated for their expertise and time accordingly;

2.

Recognise that accepting some funding from industry does not automatically imply a conflict of interest;

3.

Challenge the institutional tradition of tokenistic involvement of patient groups in the development of health policies and the design of health and other essential services;

4.

Invest in meaningful patient involvement and work with patient organisations to develop and implement good practices, including on compensation, facilitation/practical support, and capacity-building on both sides.

### European and national decision-makers should



- Not automatically insist on performance measurement; recognise that the usual (quantitative) impact assessment methods may not adequately capture the added value of patient input. Adopt patient involvement as a value in itself, and work together with patient organisations to develop more meaningful methods to make the added value of patient involvement visible;
- Find ways of providing unearmarked (unrestricted) funding to help patient organisations in their ability to fund their daily work; work with patient organisations to identify an appropriate, sustainable funding base;
- Enable patient organisations to provide their input into all stages of policy development and evaluation (including the setting of research priorities, and health and research budgets);
- Introduce quotas for chairmanship and/or membership of EU expert groups for patient groups and/or public health Non-Governmental Organisations (NGOs);
- Invite patient groups to become involved in the development of non-health sector policies.

### Researchers and academia/ professional educators should



- Systematically and actively involve patient groups in Research and Development (R&D) as joint grant holders or co-applicants, or members of project advisory boards or steering groups, in particular when it comes to research priorities;
- Take the views of patients into account when producing the lay summary of studies, their implications and applications for dissemination;
- Keep patients and their representatives informed on the progress of clinical trials or research in which they are involved;
- Involve patients in the development of medical and health-related curricula and Continuing Professional Development (CPD) modules;
- Invite patients to teaching and assessment activities of undergraduate and postgraduate healthcare professionals and future health policy-makers;
- Ensure that patients are meaningfully involved in the development of clinical and good practice guidelines;
- Promote patient involvement in defining professional skillsets and training needs.

## Introduction

The European Patients' Forum (EPF) is an umbrella organisation of 74 patient organisations in Europe. EPF's vision is that all patients with chronic conditions in Europe have equal access to high quality, patient-centred health and related care.

EPF commissioned this report to give an overview of patient organisations in Europe and highlight their value as legitimate stakeholders in civil dialogue in health-related policy-making. This report describes the main activities and roles of patient organisations in four different areas: (1) policy; (2) capacity-building and education; (3) peer support; and (4) research & development (health and pharmaceutical). Patient organisations face a number of challenges that are both internal and external of nature.

These challenges mean that patient groups and more generally society as a whole may not make the most of their potential and added value. This report therefore also sets out the particular challenges they face in the current political, financial and social climate.

In the last section of this report key messages are formulated not only for patient organisations but also for other important stakeholders who work with patients: decision-makers (both at the European and the national level); researchers; and academia/professional educators. These stakeholders are the main audience of this report and the key messages are written to help them understand the potential benefits of enhanced cooperation with patient groups in their daily work.

This report uses data collected in a survey conducted by EPF amongst its members. This survey was filled in by thirty respondents, the majority of which were European-level organisations. Answers to the concrete part of the survey are presented in the Annex.

Although the respondents consisted mainly of pan-European disease-specific organisations, and although their number was limited, the experiences reported are believed to be fairly representative of many organisations in the area. The report has also drawn on interviews with important stakeholders in the field of health, as well as literature research.

# 1. What is a patient organisation?

## 1.1 Historical context

Patient organisations have evolved considerably in the last 80 years. The first patient organisations were about the sharing of patients' experiences of a specific disease. These evolved into mutual self-help organisations in the 1940s and 50s. In the 1960s organisations took on the task of defending stigmatised and excluded patients. While health reform and health advocacy can be traced to the social reform movements of the 19th century, modern patient advocacy (distinguished from health advocacy by the direct participation of patients) has its origins in the HIV/AIDS activism in the 1980s, as well as breast cancer advocacy in the 1990s<sup>1</sup>.

During the 1980s a number of organisations operating within the same disease area merged to become national bodies with a stronger voice and greater political recognition. In the 1990s national groups started to come together on a pan-European basis. This was, to some extent, in response to the increasing influence of the European agenda on national healthcare and pharmaceutical policies as more countries joined the European Union (EU). In 1992 the Maastricht Treaty introduced an explicit EU competence on health by "encouraging cooperation between Member States" and "if necessary, lending support to their actions" in the field of public health (article 129(1)). With the revisions brought about by the Amsterdam Treaty in 1997, the EU was mandated to ensure "a high level of human health protection in the definition and implementation of all [Union] policies and activities" and to work with Member States to improve public health, prevent

illness and "obviating sources of danger to physical and mental human health" (Article 168)<sup>2</sup>. Following this development, in 2003 a number of pan-European patient groups realised that the next step in their evolution was to bring together groups from different disease areas and established the European Patients' Forum (EPF). EPF is now an umbrella organisation of 74 pan-European disease-specific patient organisations and national coalitions of patient groups from many EU Member States<sup>3</sup>. EPF's role is to be the united voice of patients and the key interlocutor with the EU institutions on cross-cutting issues affecting all patients.

## 1.2. Definition of a patient organisation

In order to explore the role patient organisations play and the value they have in the field of health and beyond, there needs to be a common understanding of what a patient organisation is. A universally accepted definition for a patient organisation does not exist. The European Medicines Agency (EMA) has developed a definition which is widely used:

**"Patients' organisations are defined as not-for profit organisations which are patient focused, and whereby patients and/or carers (the latter when patients are unable to represent themselves) represent a majority of members in governing bodies"**<sup>4</sup>.

A large number of patient organisations adhere to guidelines or principles which make them valued and recognised as trusted partners. A good example of a set of criteria for patient organisations are those defined by EPF to evaluate membership requests from prospective member organisations (below). These five criteria ensure that EPF is composed of "bona fide" patient organisations.

1.

### Transparency

Members generally disclose their sources of funding and make their audited financial accounts available;

2.

### Legitimacy

Members should be registered in at least one of the EU Member States;

3.

### Democracy

Patient organisations should have governing bodies which are elected by their members, who shall be patients, their carers, or their elected representatives;

4.

### Representativeness

Pan-European disease-specific organisations should have members of their own in more than half of the EU Member States. National platforms should represent at least 10 different disease groups in order to be accepted as full members;

5.

### Accountability and consultation

Statements and opinions should reflect the views and opinions of their memberships, and consultation procedures with those memberships should be in place<sup>5</sup>.

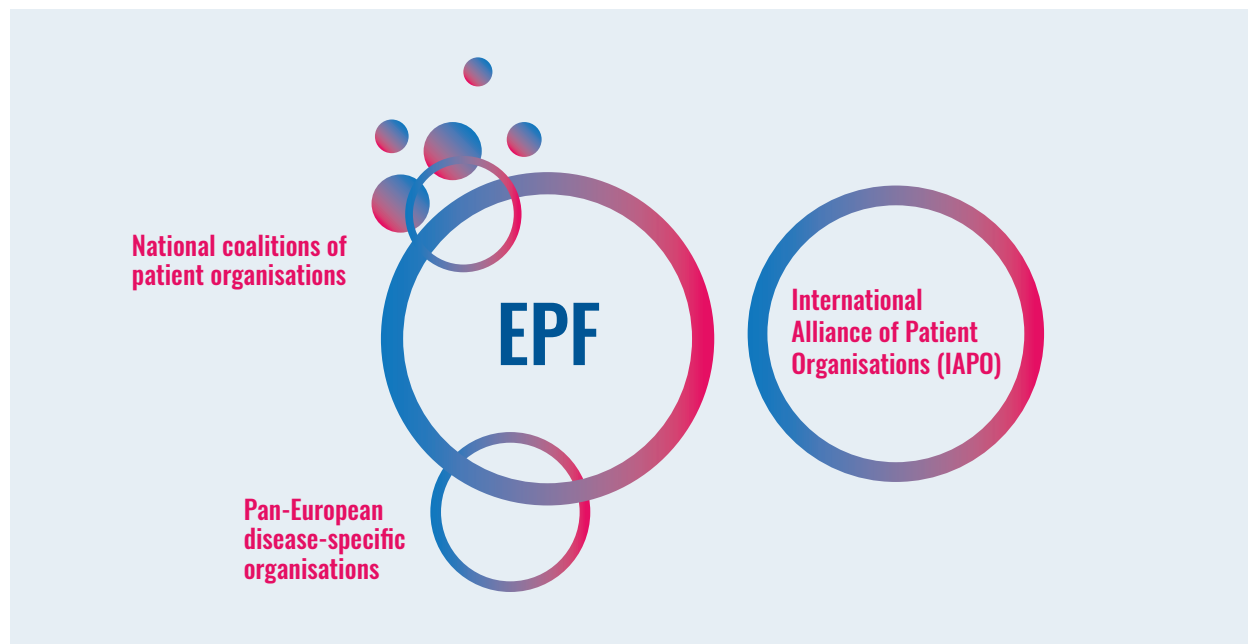
<sup>1</sup> Wong-Rieger, D. (2017), Moving from patient advocacy to partnership; a long and bumpy road, p. 1.

<sup>2</sup> Lisbon Treaty, Article 168. Available at: <http://www.lisbon-treaty.org/wcm/the-lisbon-treaty/treaty-on-the-functioning-of-the-european-union-and-comments/part-3-union-policies-and-internal-actions/title-xiv-public-health/456-article-168.html>

<sup>3</sup> EPF is not currently represented in all EU Member States as not all countries have a national coalition of patient organisations. EPF does however have reach into all MS through our disease-specific member organisations.

<sup>4</sup> European Medicines Agency, Stakeholders and Communications Division, (June 2014). Available at: [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Regulatory\\_and\\_procedural\\_guideline/2009/12/WC500018099.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/12/WC500018099.pdf)

<sup>5</sup> EPF, Full membership criteria. Accessed at 15/7/2017. Available at: <http://www.eu-patient.eu/Members/The-EPF-Members/Full-Membership/>



### 1.3 Types of patient organisations

There are many types of patient organisations: some focus on the local or regional level, while others are active at national, European or international levels. Some are coalitions or organisations working across diseases, channelling the voice of the whole patient community on cross-cutting issues, while some are condition-specific, meaning that they deal with a single disease (Alzheimer's, diabetes, multiple sclerosis, etc.) or disease area (rare diseases, cancers, mental health conditions, etc.).

**Disease-specific, local or regional patient organisations** are very close to the community they represent. They have practical knowledge of disease-specific situations and realities. They sometimes choose to become part of broader coalitions, such as national coalitions or pan-European disease-specific organisations. They do so to be able to exchange knowledge and expertise with their peers, or to strengthen their voice and outreach.

**A national coalition of patient organisations** is an umbrella organisation grouping national or regional patient organisations representing chronic conditions

or groupings of conditions. These organisations come together and form an umbrella organisation that should be representative of the collective interests of all patients in one country. The national coalition thus becomes the single point of contact for national stakeholders and decision-makers. Of course, the national coalition only works on cross-cutting issues (e.g. access to care, discrimination, structure of healthcare systems), enabling disease-specific organisations to focus on their area of specialism<sup>6</sup>.

Pan-European disease-specific organisations are another type of umbrella organisations, gathering national patient organisations active in the same disease-area. They enable the patient community to compare situations between countries, to have a better overview of the treatments and care available across Europe, and to transfer good practices whenever possible. They are also in a better place to monitor legislative developments at EU level.

For the sake of clarity it is important to make the distinction between EPF members and patient organisations in general. Under its umbrella, the

European Patients' Forum gathers both national coalitions and pan-European disease-specific organisations. Local, regional and national disease-specific organisations are not eligible for EPF membership, but they can be members of national coalitions and European umbrella organisations that are members of EPF. On the international level, an equivalent of a body of European patient groups is the International Alliance of Patient Organisations (IAPO).

Patient groups are sometimes not legally formalised. They may be a sub-group active within broader organisations or alliances which include other stakeholders, such as healthcare professionals. An example of the latter is PARE, "People with Arthritis/Rheumatism in Europe", a Standing Committee within the European League Against Rheumatism (EULAR), which encompasses patient groups, health professionals and academia and acts as a forum for patient organisations without itself being a patient organisation.

<sup>6</sup> European Patients' Forum, Toolkit on building national coalitions of patient organisations, (2016).

## 1.4 How patient organisations differ from other NGOs

Patient organisations are unique amongst civil society organisations because of the duality in focus and membership. Whereas NGOs in other areas work on behalf of a wider constituency or society, members of patient organisations are very often patients, relatives of patients, or carers themselves (who indeed often work on a voluntary basis).

**“Patient organisations are a special subset of human-centric NGOs”.**

**Robert Madelin, Chairman of Fipra, former Director-General for Health and Consumer Policy**

The outreach and impact of patient organisations is very strong because they have first-hand experiences and can channel the voice of those they represent. This should, in an ideal world, also mean they have considerable credibility. Unfortunately this is not always the case. Section 3 on challenges will elaborate on this issue.

**“Patient organisations have a lot of influence and their outreach work is very influential. There is a lot of commitment, which their power and dynamism stems from.”**

**Sirpa Pietikäinen, Finnish Member of the European Parliament**

## 1.5 Patient Organisations across Europe

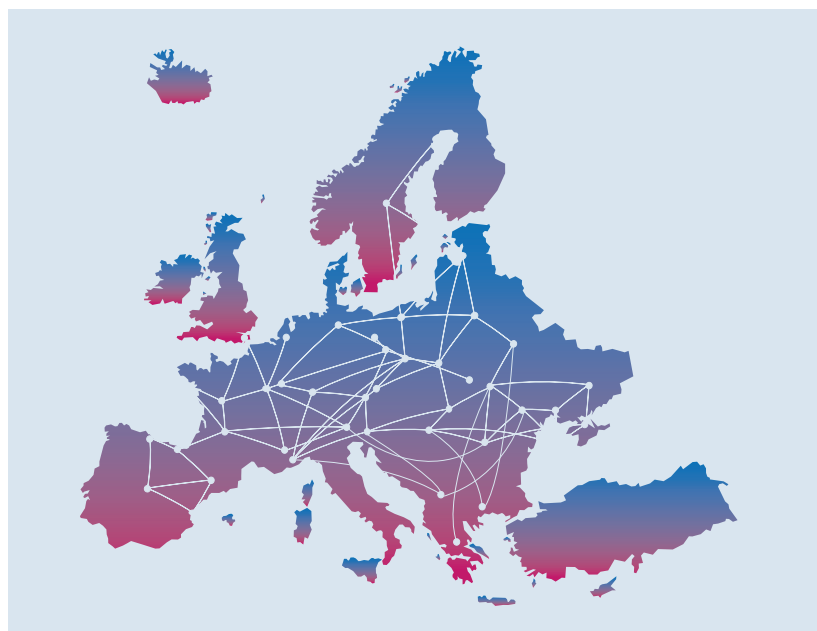
The exact number of patient organisations worldwide or in Europe is not known. In order to have an overview of their own geographical coverage EPF conducted some research and identified so-called “white zones” – the countries in which EPF is not directly geographically represented. The report<sup>7</sup> concluded that in some countries the patient movement is not formally organised in a national coalition – such as in the case of Portugal or Italy. This does not mean, however, that these countries do not have any patient organisations or that there is no active patient movement at all. It may be that patient groups have simply not (yet) come together to form a unified voice at national level.

In other countries, regionalism affects the way the patient movement is organised. In Belgium, for example, there are two patient coalitions channeling the voice of patients at the national level, representing two out of the three Belgian regions (French-speaking Wallonia and Flemish-speaking Flanders). In the Baltic and Nordic countries, patients’ interests are often represented by

a national disability organisation, which advocates for patients’ rights in addition to disabilities issues. This is the case in Latvia, Lithuania, Estonia, Sweden and Finland.

Furthermore, in some countries, there is an inter-regional dimension to the patient movement. This is the case in Scandinavia, where there is not necessarily a single organisation representing patients with a specific chronic disease at national level, but where Finnish, Swedish, and Norwegian patient organisations pool their resources and knowledge together and work together across borders to solve their common issues through ‘Nordic cooperation’<sup>8</sup>.

With regard to European disease-specific patient organisations, the second type of EPF members, the “white zones” report referred to above showed that some chronic diseases are not represented at European level. This does not mean that there is no patient organisation representing this disease-area at other governance levels in Europe.



<sup>7</sup> Unpublished.

<sup>8</sup> European Patients’ Forum, Membership Development, White Zones Report, (2016).



## 2. Value and activities of patient organisations

“The governments of Member States should ensure that citizens’ participation should apply to all aspects of healthcare systems, at national, regional and local levels (...) and create legal structures and policies that support the promotion of citizens’ participation and patients’ rights, if these do not already exist.”

Council of Europe Recommendation No.R(2000)5 on the development of structures for citizen and patient participation in the decision-making process affecting healthcare<sup>9</sup>.

Policy-makers at European institutions and World Health Organization (WHO) Europe have been explicit in their calls for more patient involvement<sup>10</sup>. The European Commission’s White Paper “Together for Health: A Strategic Approach for the EU 2008-2013” (October 2007) highlights that the participation and empowerment of citizens and patients needs to be regarded as a core value in all health-related work at the EU level<sup>11</sup>. Building on the work done on the Citizens’ Agenda, community health policy must take citizens’ and patients’ rights as a key starting point<sup>12</sup>. This includes participation in and influence on decision-making, as well as competences needed for wellbeing, including health literacy. Patient involvement is also one of the operating principles put forward in the Council Conclusions on Common Values and Principles in the European Union Health Systems (June 2006): “All EU health systems aim to be patient-centred. This means they aim to involve patients in their treatment, to be transparent with them, and to offer them choices where this is possible,

e.g. a choice between different health care service providers”<sup>13</sup>.

Social and political scientists have also joined patient organisations in calling for a redistribution of power between patients, experts and specialists in policy- and decision-making, which concerns patients as end-users. An example can be found in a 2012 article by Truglio-Londrigan: “A qualitative systematic review of internal and external influences on shared decision-making in all healthcare settings”<sup>14</sup>. Patients and the ones representing them should be seen as stakeholders in the medical and healthcare fields and “be entrusted with a share of the decision and control power in these fields, on an equal footing with biomedical institutions, pharmaceutical firms and health administrations”<sup>15</sup>. They should be assigned an actual role instead of a tokenistic one.

The fact that patient organisations have an undeniable *raison d’être* does not prevent them from having to quantify their added value. An aspect of patient input that remains difficult

to quantify is the ability of patients to provide an overarching perspective and first-hand experience on what it is like to live with a disease. However, patients are the undisputed experts on this and should be formally recognised as such.

Furthermore, other stakeholders are not routinely challenged on their representativeness or their ability to distinguish between personal and collective views. Recommendations from the European Medicines Agency (EMA) Patients’ and Consumers’ Working Party (PCWP) topic group on measuring the impact/value of patient involvement in EMA activities reiterate that patient experts should not be subjected to scrutiny that is not applied to other experts, and conclude that despite the fact that [there is] “little research on methodologies in this area that could be extrapolated and used to measure patient input”, efforts should continue to measure and make visible its added value, including via qualitative methods, in order to show the overall benefits of patient involvement<sup>16</sup>.

<sup>9</sup> Council of Europe, Recommendation No.R(2000)5 on the development of structures for citizen and patient participation in the decision-making process affecting healthcare.

<sup>10</sup> WHO European Region, Kickbusch, I. & Gleicher, D., Governance for Health in the 21st Century. Available at: [http://www.euro.who.int/\\_data/assets/pdf\\_file/0019/171334/RC62BD01-Governance-for-Health-Web.pdf](http://www.euro.who.int/_data/assets/pdf_file/0019/171334/RC62BD01-Governance-for-Health-Web.pdf)

<sup>11</sup> European Commission, Together for Health, A Strategic Approach 2007-2013, COM (2007) 630 FINAL (23/10/2007). Available at: [https://ec.europa.eu/health/ph\\_overview/Documents/strategy\\_wp\\_en.pdf](https://ec.europa.eu/health/ph_overview/Documents/strategy_wp_en.pdf)

<sup>12</sup> European Commission, Citizens Agenda, Single Market Review. Archived 12/8/2009. Available at: [http://ec.europa.eu/citizens\\_agenda/single\\_market\\_review/index\\_en.htm](http://ec.europa.eu/citizens_agenda/single_market_review/index_en.htm)

<sup>13</sup> The Council of the European Union, Council Conclusions on Common values and principles in European Union Health Systems (2006/C 146/01), (22/06/2006). Available at: <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2006:146:0001:0003:EN:PDF>

<sup>14</sup> Truglio-Londrigan M., et al. (2012), A qualitative systematic review of internal and external influences on shared decision-making in all healthcare settings. *JBI Libr Syst Rev.* 2012;10(58):p. 4633-4646.

<sup>15</sup> Akrich, M., The dynamics of patient organisations in Europe, (2008), p. 27.

<sup>16</sup> European Medicines Agency, Consolidated report on the activities of topic groups established in 2015, (23/05/2017). Available at: [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Annual\\_report/2017/05/WC500228518.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Annual_report/2017/05/WC500228518.pdf)

## 2.1. Principles of advocacy for health

As early as 1978 the World Health Organization (WHO) introduced the concept of participation and involvement of people in their planning and implementation of their healthcare. The modern use of the term “advocacy for health” gained momentum after the first international conference on health promotion (Ottawa Charter on Health Promotion, 1988)<sup>18</sup>, and later when the WHO’s “Health Promotion Glossary” (1998) made a first attempt to define the term as “A combination of individual and social actions designed to gain political commitment, policy support, social acceptance and systems support for a particular health goal or programme”<sup>19</sup>.

Despite significant progress made in the field of health since the inception of the term, it still accurately describes the nature of advocacy actions: “Acting by and/or on behalf of individuals and groups to design living conditions that create and support health outcomes and healthy lifestyles”. It can take on many forms including the use of the mass media, multimedia (including digital) and social media, direct political lobbying, and community mobilisation through, for example coalitions of interest around defined issues - such as patient organisations. There are three overarching principles which inform patient advocacy: representation, mobilisation and empowerment<sup>20</sup>.

“The people have the right and duty to participate individually and collectively in the planning and implementation of their healthcare.”

Alma Ata Declaration, Principle IV (1978, WHO)<sup>17</sup>



### Representation

When individual patients are not able to represent themselves or when the whole group of patients elects one person to represent their interests, one talks about representational advocacy.

The motto of the global worldwide disability movement “nothing about us without us” has been adopted by many patient organisations. It embodies the principle that no health policy, programme or other initiative that has a potential impact on patients should be undertaken without the participation of patients. In this regard, patient organisations add essential value by enabling patient to “speak with one voice”, which improves their visibility and credibility, and ensures a coordinated presence of patient-related issues in health and non-health policies and programmes at the different governance levels where patient organisations are active.

Having a “structured patient community” that acts as a single established forum able to form and articulate consensual opinions on

patient-related issues in a uniform manner is valuable for other stakeholders as well: they can identify one single point of contact, either for one disease-area or for one country, they do not have to meet with multiple organisations, and the information they receive is structured, coherent and non redundant.

A good example of the rationale behind the representational advocacy of patient coalitions at national level is outlined in EPF’s Toolkit on Building National Coalitions of Patient Organisations, where the advantages of “joining forces” are highlighted: building on the expertise of others, broadening the understanding of the patient community, transcending institutional boundaries, and achieving quicker and more sustainable progress in fulfilling one’s mission through optimised use of their own resources<sup>21</sup>.

An organisation which is unique and an example in its representational advocacy, is the European Network of (Ex-)Users and Survivors of Psychiatry (ENUSP). Self-governed by people

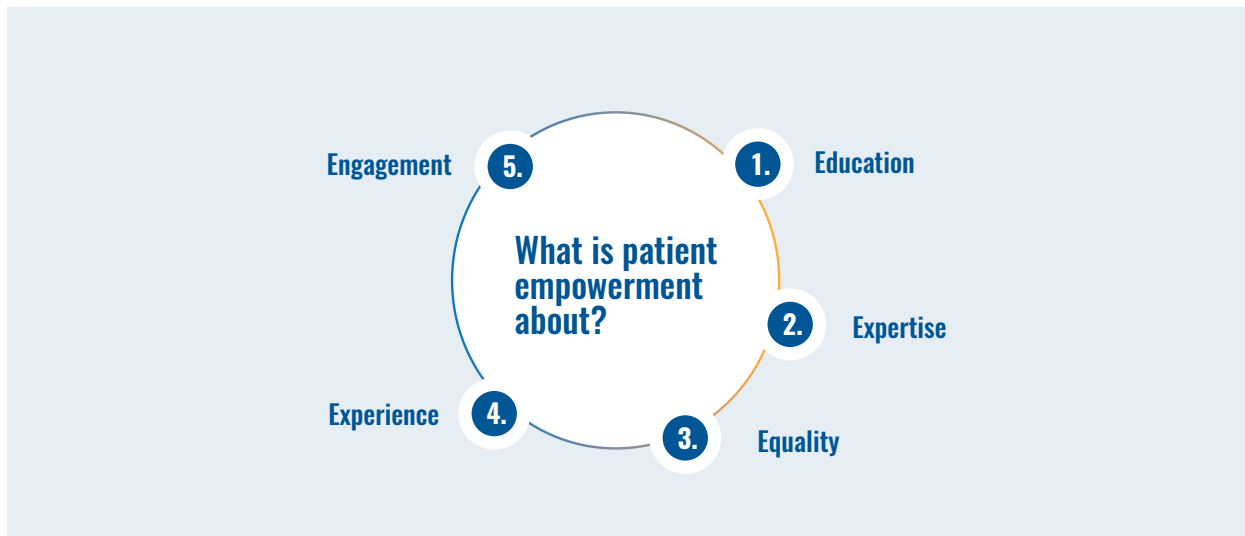
<sup>17</sup> World Health Organization, The Alma Ata Declaration, Principle IV, (1978)

<sup>18</sup> World Health Organization, The Ottawa Charter for Health Promotion, First International Conference on Health Promotion, Ottawa, (21/11/1986). Available at: <http://www.who.int/healthpromotion/conferences/previous/ottawa/en/>

<sup>19</sup> World Health Organization, Health Promotion Glossary, (1998). Available at: [http://apps.who.int/iris/bitstream/10665/64546/1/WHO\\_HPR\\_HEP\\_98.1.pdf](http://apps.who.int/iris/bitstream/10665/64546/1/WHO_HPR_HEP_98.1.pdf)

<sup>20</sup> Centre for Society Orientation. Available at: <http://www.cod.rs/en/what-we-do/advocacy-and-lobbying/>

<sup>21</sup> EPF, Toolkit on Building National Coalitions of Patient Organisations (2016). Available at: [http://www.eu-patient.eu/globalassets/library/toolkits/epf-toolkit\\_nationalcoalitions.pdf](http://www.eu-patient.eu/globalassets/library/toolkits/epf-toolkit_nationalcoalitions.pdf)



with psychiatric experience and fully independent of funding from the pharmaceutical industry, it provides (ex-)users and survivors of psychiatric treatment means for direct self-representation<sup>22</sup>.

### Mobilisation

By involving people in patient organisations' activities in such a way that they are encouraged and supported to join the cause, mobilisation expands the rationale for action from a narrow single-issue cause towards convincing others/non-patients that the issue is important for the wider community or population as well. It has been argued (among others in the 2010 book "The spirit level" by Pickett and Wilkinson) that more equal societies with more inclusive public policies do better in overall terms. The mobilisation of communities of interest has an important role to play in promoting more inclusiveness and hence equality. This is also in line with the motto "Society is only as strong as its weakest link"<sup>23</sup>.

Reaching a broad audience means tailoring advocacy and policy messages to decision-makers, the media (mass and specialised), donors and funders, individual patients, academia and other potential partners from the general advocacy community.

In reference to the current 'alternative facts', in a 'post-truth' society where experts are often discredited and/or ridiculed, this role of acting as multiplier of evidence-informed policy messages obtained through consensus with a wider group of patients becomes increasingly relevant.

### Empowerment

Advocacy through representation and mobilisation enables patients to be agents of change in political and practical discourse. Actions involved – collecting and exchanging information, awareness raising, encouraging other stakeholders to participate – allow for a sense of ownership over the outcomes achieved (or not). This particularly applies to groups that have traditionally been excluded from policy- and decision-making processes such as patients and marginalised populations.

Empowerment is "a multidimensional process that helps people gain control over their own lives and increases their capacity to act on issues that they themselves define as important." Collective 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"<sup>24</sup>. This is the empowerment principle of advocacy.

## 2.2. Policy

**"Patients, being a crucial stakeholder in the health system, absolutely have to be involved. The involvement of structured patient organisations is part of that."**

**Robert Madelin, Chairman of Fipra, former Director-General for Health and Consumer Policy, European Commission**

On the wide spectrum of policy-making (whether it is local, national or European), patient organisations have a particular contribution to make in helping policy-makers understand patient priorities and experiences of living with a disease/condition<sup>25</sup> recognising the added value of patient organisations is recognising that this perspective would be lacking without their input.

Patient organisations contribute to policy at all stages by being a trusted, pro-active, involved and communicative partner in the

<sup>22</sup> European Network of (Ex-)Users and Survivors of Psychiatry (ENUSP). Available at: <http://enusp.org/who-we-are/>

<sup>23</sup> Pickett, K. & Wilkinson, R. (2010), *The spirit level: why equality is better for everyone?* Penguin Books. Second edition.

<sup>24</sup> PaSQ, adapted from Luttrell et al. and the Duque project, (2009). Available at <http://www.duque.eu>.

<sup>25</sup> For example through the European Parliament Interest Group on Patients Access to Healthcare or the European Commission's EU Health Policy Forum.

policy-making process. Often complex and lengthy, the process of policy formulation, drafting and implementation requires coordinated, coherent and timely input from all relevant stakeholders at every stage if it is to achieve its defined goal. Patient organisations can contribute to this process by offering their perspectives in consultations, by suggesting refinements at a later stage where they think policy could be improved, and by evaluating both the quality of implementation and the eventual impact of the policy.

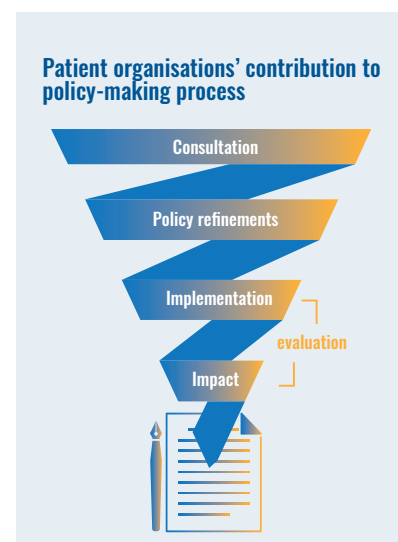
Patient organisations are often members of stakeholder advisory groups, expert panels, European and/or national government public consultations or institutional meetings where they provide a united voice to promote the best interests of patients vis-à-vis decision-makers. Examples of this are the coordination by the European Federation of Allergy and Airways Diseases Patients Associations (EFA) of the European Parliament's Interest Group on Asthma and Allergy, the response written by EPF to the Public Consultation from the European Commission on the EU Pillar of Social Rights and the comments sent in by the International Federation for Spina Bifida and Hydrocephalus (IFSBH) on the EU Disability Strategy. In a similar way, many national patient groups regularly contribute to national health policy consultations and initiatives.

Almost half of the respondents to the EPF survey believe that they are always or most of the time successful in having a concrete positive impact on shaping policy. Another 48% believe they achieved this at least sometimes.

Patient organisations have broadened their views beyond classic health policy by contributing to the Health

in All Policies (HiAP) approach alongside other health stakeholders; further enabling their participation in this process will enhance the overall balance and nuance in policy-making. HiAP is a cross-sectoral approach to public policies that “systematically takes into account the health and health systems implications of decisions, seeks synergies and avoids harmful health impacts, in order to improve population health and health equity”<sup>26</sup>. Strong patient and public involvement is fundamental to HiAP approach, and the role of patients herein is broad: they reinforce the need for the systematic evaluation of consequences of public policies on health determinants (for both patients and the population at large) by contributing to Health Impact Assessment (HIA) exercises; they bring a strong focus on human rights and social justice; they act to improve the accountability of all-level policy-makers for health impacts; and they negotiate for long-term changes.

Patient groups are well-positioned to identify and steer “consensual fields of actions” from their own needs assessment analysis (cross-sector wins such as the economic contribution of improving re-employability of persons with chronic conditions), they co-generate evidence by making use of the vast expertise of patients they have to hand or re-balance power relations in policy-making. In a context where the demand for and costs of healthcare are rising whilst resources are scarce, health technology assessment (HTA) and economic evaluations (EE) have become an integral part of healthcare decision-making<sup>27</sup>. HTA is used to assess the relative effectiveness of a new medicine (and increasingly on treatment, devices etc.) compared to existing ones, supporting decisions on pricing and reimbursement that



are meant to be fair for patients and for society.

In its broad form, HTA is meant to be “a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seek to achieve best value”<sup>28</sup>.

The importance of incorporating the patient perspective into HTA is increasingly recognised as potentially enriching the content of HTA reports and recommendations<sup>29</sup>.

The integration of patient-reported and patient-relevant outcomes measured in HTA is considered necessary in order to arrive at an accurate assessment of a medicine's added value<sup>30</sup>. Patient organisations are usually either contacted by HTA agencies (if they have a relationship) when the agency is seeking submissions or they respond to public consultations<sup>31</sup>.

<sup>26</sup> World Health Organization, Health in All Policies, Seizing Opportunities, Implementing Policies, Leppo K., (2013). Available at: [http://www.euro.who.int/\\_data/assets/pdf\\_file/0007/188809/Health-in-All-Policies-final.pdf](http://www.euro.who.int/_data/assets/pdf_file/0007/188809/Health-in-All-Policies-final.pdf)

<sup>27</sup> Tritter, J., Koivusalo, M., Ollila, E. & Dorfman, P. (2009) Globalisation, markets and healthcare policy: redrawing the patient as consumer. Routledge. Available at: [https://books.google.be/books?id=zg0tZUNzIryC&printsec=frontcover&source=gbs\\_ge\\_summary\\_r&cad=0#v=onepage&q&f=false](https://books.google.be/books?id=zg0tZUNzIryC&printsec=frontcover&source=gbs_ge_summary_r&cad=0#v=onepage&q&f=false).

<sup>28</sup> European Network of Health Technology Assessment (EUNETHTA) definition.

<sup>29</sup> Dipankui et al. (2015) “Evaluation of patient involvement in a health technology assessment”, *Int J Technol Assess Health Care*. 2015 Jan;31(3):166-70.

<sup>30</sup> EU HTA Network: “Strategy for EU Cooperation on Health Technology Assessment”, 2014, p. 8. Available at [http://ec.europa.eu/health/technology\\_assessment/docs/2014\\_strategy\\_eucooperation\\_hta\\_en.pdf](http://ec.europa.eu/health/technology_assessment/docs/2014_strategy_eucooperation_hta_en.pdf)

<sup>31</sup> Scott A and Wale J, “Patient advocate perspectives on involvement in HTA: an international snapshot”, *Research Involvement and Engagement* (2017) 3:2.

But patient involvement in HTA is still a limited practice, and so far there is no agreement on the best method of involving patients<sup>32</sup>. At the international level, the HTAi Patient and Citizens' Sub-Group is working to promote methodologies to incorporate patients' perspectives in HTA, share good practices and strengthen HTA by the systematic incorporation of patient perspectives<sup>33</sup>. A first comprehensive guidebook for patient involvement in HTA was recently published<sup>34</sup>. The European Patients' Academy (EUPATI), a project coordinated by EPF, has also developed guidance for patient involvement in HTA<sup>35</sup>.

As the end-users of health services, the patient perspective should be intrinsic to health services design. Coulter (2002) suggested that the twenty-first-century health service user is at once "a decision-maker, a care manager, a co-producer of health, an evaluator, a potential change agent, a taxpayer and an active citizen whose voice must be heard by decision-makers"<sup>36</sup>. Planning and provision of patient-oriented health-care services and co-design should be based on the opinions, needs, and preferences of patients, their carers, and the wider community: this is a key challenge in the health systems of developed countries<sup>37</sup>. The OECD, which collects health care quality indicators for the European Commission, recently launched the PaRIS initiative (endorsed by Member States in January 2017), which aims to put the patient perspective in the centre of healthcare reforms<sup>38</sup>. The essence of the initiative is the collection of comparable cross-country indicators on patient-reported

outcome measures (PROMs) and measures for patients' experiences of care (PREMs). Building on this crucial piece of work, another important future area will be the development of PROM/PREM indicators for persons with multiple chronic conditions.

The OECD has invited patient organisations to participate, inter alia through EPF, as it values the input patient representatives can provide in the design phase of such questionnaires, to ensure they really measure the outcomes and experiences that "matter to patients" and can realistically be implemented. Quality of care and patient safety can be hugely improved by patients

implementation at Member State level of the Directive on Cross-border Healthcare that was adopted in 2011. Patient organisations helped policy-makers understand the impact of the Directive on patients by checking how it was actually implemented by national governments and by providing feedback to the institutions<sup>40</sup>. In this case, the 2013 European Commission's report for the European Parliament and the Council on the Directive notes the concern of patient organisations that "patients are faced with 'a labyrinth of confusing... information' with regard to cross-border healthcare" and noted their policy recommendations for addressing this<sup>41</sup>.

**"I think that (patient organisations) have a very important role (...). Firstly, they advise and connect and give support to patients at the grassroots level. Secondly, they gather specialist information from the patients' point of view. (...) Finally, their role is crucial in advocacy and in pushing forward the development of priority European health and medical policies."**

**Sirpa Pietikäinen, Finnish Member of the European Parliament**

participating at an organisational level (hospitals, institutions etc.), in patient councils and in patient safety initiatives. The participation of patients in improving patient safety is increasingly recognised as important, and there are examples of innovative practice in involving patient organisations.

Sweden and Belgium are two countries where systematic patient involvement is being explored<sup>39</sup>. An example of patient involvement in policy is the

Evidence reviewed by RAND in their research "Involving the public in healthcare policy" supports the notion that public involvement initiatives or activities can have an effect on the health care policy process through influencing strategic decisions such as decisions of service delivery or priority-setting. There is also empirical evidence supporting the notions of the developmental role of public involvement in improving lay participants' knowledge of subject areas and/or decision-making

<sup>32</sup> EPF survey: "Patient Involvement in Health Technology Assessment in Europe," 2013. Available at [www.eu-patient.eu/whatwedo/Initiatives/](http://www.eu-patient.eu/whatwedo/Initiatives/)

<sup>33</sup> Health Technology Assessment International. Available at: <http://www.htai.org/interest-groups/patient-and-citizen-involvement/pcig-home/pcig-objectives.html>

<sup>34</sup> Facey, Karen, Ploug Hansen, Helle, Single, Ann (Eds.) Patient Involvement in Health Technology Assessment. Adis, 2017.

<sup>35</sup> <https://www.eupati.eu/health-technology-assessment/guidance-for-patient-involvement-in-hta/>

<sup>36</sup> Coulter, A. (2004) When I'm 64: Healthy choices. In: Health Expectations, 7, pp. 95–97.

<sup>37</sup> Vahdat, S. et al, Patient Involvement in Health Care Decision Making: A Review, Iran Red Crescent Med J. (2014 Jan); 16(1): e12454. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3964421/#A12454R15>

<sup>38</sup> OECD Ministerial statement: "The Next Generation of Health Reforms" at <https://www.oecd.org/health/ministerial-statement-2017.pdf> and PaRIS initiative: [http://oecdobserver.org/news/fullstory.php/aid/5790/The\\_PaRIS\\_initiative:\\_Helping\\_healthcare\\_policies\\_to\\_do\\_better\\_for\\_patients.html](http://oecdobserver.org/news/fullstory.php/aid/5790/The_PaRIS_initiative:_Helping_healthcare_policies_to_do_better_for_patients.html)

<sup>39</sup> WHO (2013) report "Exploring patient participation in reducing health-care-related safety risks"; Reports of the sub-groups of the PSQC WG on education in patient safety and reporting-learning systems, April 2014; The Health Foundation (2013) Evidence scan: Involving patients in patient safety; EPF

<sup>40</sup> EPF, Crossborder Healthcare Available at: <http://www.eu-patient.eu/whatwedo/Policy/Patients-Mobility/>.

<sup>41</sup> European Commission, Report from the Commission to the European Parliament and the Council, COM (2015) 421 FINAL, (4/9/2015) Available at: [https://ec.europa.eu/health/sites/health/files/cross\\_border\\_care/docs/2015\\_operation\\_report\\_dir201124eu\\_en.pdf](https://ec.europa.eu/health/sites/health/files/cross_border_care/docs/2015_operation_report_dir201124eu_en.pdf).

processes, and of increasing awareness among decision-makers and/or service providers of ways of operating in the healthcare sector<sup>42</sup>.

The results of the survey led by EPF on the main activities of patient organisations show that, on average, organisations contribute to three or more major EU public consultations (the stage prior to policy development) per year on top of the input they give to internal, national, regional or local consultations. Furthermore, it shows that patient organisations are involved in general “advocacy activities” and “awareness raising”. Most respondents (80%) reported being active in advocacy activities at European level, while 43% focus mainly on domestic initiatives (and some of them are active on both). The level they operate on depends on their nature: pan-European organisations logically tend to work more with EU institutions while national coalitions focus their efforts on the national level – where they have a better knowledge of the situation, legislative tools, etc.

This reinforces the importance of communication between organisations active at different levels in order to coordinate advocacy efforts. The top four advocacy activities identified concern monitoring policies and informing members, responding to consultations, meeting decision-makers and participating in advisory bodies either of the EU or national governments: this concerns 76% of the respondents<sup>43</sup>. When it comes to the EU institutions, patient organisations mostly meet with representatives of the European Parliament (62%) and within the European Commission with representatives of Directorate-General (DG) Sante (55%) followed by DG Research (38%) and other EU’s agencies (38%). About half (52%) are

in contact with national authorities. Cooperation with other stakeholders within the patient organisations’ area of activity is considered important for 52% of the respondents with 28% regularly carrying out such cooperation. Only 17% of patient organisations that responded to the survey indicated having experience in leading a working group within an EU or national institution, suggesting either a lack of capacity (skills, workload, finances) or a lack of formal recognition and institutional trust in the ability of patient organisations to perform these kind of roles. (See Annex for all the results of the survey).

## 2.3. Capacity building and education

### 2.3.1 Capacity building

Capacity building delivered by and for patient organisations refers to intentional and coordinated efforts focused at strengthening organisational management and governance structure, making patient groups more

resilient and sustainable, enabling them to achieve their mission and increasing their effectiveness, performance and impact. Such efforts can take place at local, regional, national, or European level where patient organisations help the patient movement at large to structure itself, gain credibility and professionalism, and expand on their ability to meaningfully contribute to European and national health policy and advocacy, as well as participatory democracy. They can be aimed at patients, the public, patient organisations and the community, or at other stakeholders (including health professionals and researchers as well as industry). Thus, EPF recognised in 2009 that those wishing to work with patients also needed help with capacity-building – the result was the Value+ Handbook on meaningful patient involvement for project coordinators, leaders and health promoters<sup>44</sup>.

Examples of activities aimed at patients, the membership of patient



<sup>42</sup> Conklin A., et al, RAND Europe, (2010) Involving the public in healthcare policy- An update of the research evidence and proposed evaluation framework. Available at: [http://www.rand.org/content/dam/rand/pubs/technical\\_reports/2010/RAND\\_TR850.pdf](http://www.rand.org/content/dam/rand/pubs/technical_reports/2010/RAND_TR850.pdf).

<sup>43</sup> Among the respondents who indicated being active in advocacy activities.

<sup>44</sup> European Patients’ Forum. Available at: [http://www.eu-patient.eu/globalassets/projects/valueplus/doc\\_epf\\_handbook.pdf](http://www.eu-patient.eu/globalassets/projects/valueplus/doc_epf_handbook.pdf)

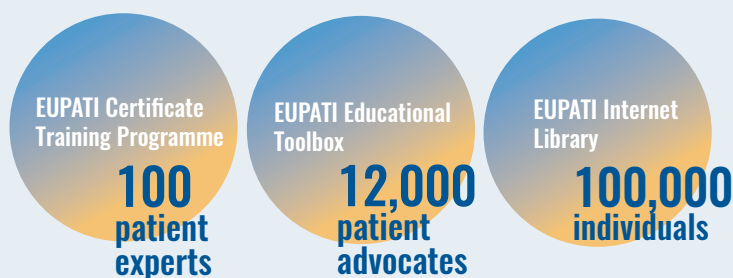
organisations or other health advocates in the community are leadership development, strategic and organisational planning, programme design and evaluation, fundraising, financial planning and management, or strategic communications. They can be quite specific; for example the European AIDS Treatment Group (EATG) toolkit supports communities to demand optimal HIV treatment from their local authorities, insurers and businesses<sup>45</sup>. They can also be more general: sharing good practices, tools and methodologies of good organisational governance or for working in partnerships with the pharmaceutical industry. A good example of a well-established, effective and highly-appreciated capacity building activity is the EPF Capacity Building Programme (launched in 2012)<sup>46</sup>. Designed to respond to the needs and concerns identified by EPF membership, it supports patient organisations in strengthening their role as players in the health care policy and service delivery environment. Flexible and 'tailor-made' assistance is offered to increase the organisational capacity and advocacy skills of patient groups (at national level in the Central and Eastern European region and at EU level), enhance the capacity of all partner patient organisations and their knowledge on specialised topics (patient safety, health technology assessment, etc.), and build leadership skills among young patient advocates<sup>47</sup>.

Another example of increased cooperation and involvement of the patient community in this area is the European Patients' Academy on Therapeutic Innovation (EUPATI)<sup>48</sup>. This pan-European project is a public-private partnership implemented by

## What is EUPATI?

The European Patients' Academy on Therapeutic Innovation (EUPATI) is a Public Private Partnership within the Innovative Medicines Initiative (IMI) Joint Undertaking. For more information please check [www.eupati.eu](http://www.eupati.eu)

## EUPATI audiences



a collaborative multi-stakeholder consortium from the pharmaceutical industry, academia, not-for-profit, and patient organisations. EUPATI's approach is to provide training to patients in order to increase their capacity and capability to understand and contribute to medicines research and development and also to improve the availability of objective, reliable, patient-friendly information for the public. In addition to the activities targeted at the patient community itself, it is not uncommon for patient organisations to invest in capacity building for health professionals, policy-makers, industry, and academia. In that case, the focus can be on upgrading these stakeholders' skills in communication and evidence-based messaging or on how to meaningfully involve patients in clinical and social research in thematic training modules and seminars. These activities are undertaken to increase general coherence, trust and mutual understanding within and outside the sector.

Patient groups also play a fundamental role in capacity building with the media to promote a positive depiction of patients that is stigma-free and factually accurate in popular media stories. The activities of European Organisation of Families Affected by Mental Illness (EUFAMI) are exemplary in this regard; they developed a campaign promoting an "International Media Guide for Mental Health" with an annual award-ceremony among European media outlets<sup>49</sup>.

## 2.3.2 Education

Closely related to capacity-building activities are educational initiatives undertaken by patient organisations. Disseminating and multiplying information throughout the healthcare sector and beyond, whether bottom-up (end-users to policy-makers and other stakeholders), top-down (policy-makers and other stakeholders to end-users) or horizontally (between various stakeholders) has become an increasingly important part of patient organisations' daily work.

<sup>45</sup> European AIDS Treatment Group (EATG), Toolkit. Available at: <http://www.eatg.org/news/new-toolkit-supports-communities-to-demand-optimal-hiv-treatment/>.

<sup>46</sup> EPF, Evaluation of the 2012-2014 EPF Capacity Building Programme, Summary of the Findings (2015). Available at: [http://www.eu-patient.eu/globalassets/who-we-are/transparency/cbp\\_evaluation\\_summary-of-findings\\_2015.pdf](http://www.eu-patient.eu/globalassets/who-we-are/transparency/cbp_evaluation_summary-of-findings_2015.pdf).

<sup>47</sup> EPF, Capacity Building Programme, Available at: [http://www.eu-patient.eu/whatwedo/Capacity-Building-programme/EUPATI\\_Closing\\_report\\_of\\_EUPATI\\_2012-2017\\_\(2017\)](http://www.eu-patient.eu/whatwedo/Capacity-Building-programme/EUPATI_Closing_report_of_EUPATI_2012-2017_(2017)). Available at: [https://www.eupati.eu/closing-report-eupati-2012-2017/#2\\_](https://www.eupati.eu/closing-report-eupati-2012-2017/#2_)

<sup>48</sup> Results\_and\_successes\_of\_the\_EUPATI\_Project.

<sup>49</sup> International Media Guide for Mental Health. Available at <http://eufami.org/wp-content/uploads/2017/03/lexicon.pdf>.

On the one hand, patient organisations “translate” or disentangle complex information – whether scientific or policy-related – into readily understandable, relevant and accessible resources for the patient community in order to support patient advocacy and build health literacy. On the other hand, they gather and collate information/knowledge derived from the patient communities and individual patients, and feed this collective knowledge (sometimes referred to as “patient evidence”) to relevant stakeholders. Patient organisations also often have considerable outreach to the general public as sources of lay-friendly information on various health-related issues.

Examples of horizontal dissemination of information are the EURORDIS “Living with a Rare Disease” online library and TV<sup>50</sup> or the European Haemophilia Consortium’s (EHC) “Haemophilia stories”<sup>51</sup>, both of which depict the impact of health and non-health policies on the daily lives of their specific patient communities.

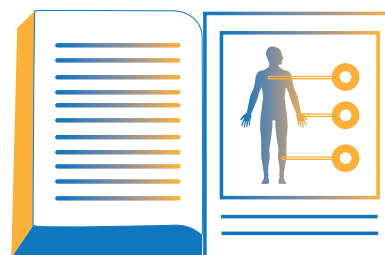
Acting as educators to other stakeholders can also entail organising high-level conferences, seminars<sup>52</sup>, roundtables and thematic workshops. Patient organisations can use these to set the agenda and ‘the rules of the game’ by steering discourse, changing mindsets and seizing the political window of opportunity. This educational approach can help in tackling challenges like convincing multiple stakeholders of the importance of a HiaP approach. An

interesting example of educating other stakeholders is the European Multiple Sclerosis Platform’s (EMSP) Practical Toolkit for Employers on Supporting People with Multiple Sclerosis in the Workplace<sup>53</sup>. This practical guide for businesses offers concrete examples of ways to “enhance or build their policies in terms of recruitment, attendance management and return-to-work procedures”, developed by patient groups themselves.

Patient organisations also carry out activities aimed at increasing patients’ (and the general population’s) health literacy, including treatment literacy. Health literacy, defined by the WHO (2013) as “the ability to make sound health decisions in the context of everyday life: at home, in the community, at the workplace, the health care system, the marketplace and the political arena”<sup>54</sup>, is a key determinant of health and well-being. Low health literacy often leads to the underestimation of health problems, a lack of adherence to treatment and/or self-management of chronic conditions, and the subsequent aggravation of health inequities.

Better health literacy can support prevention and healthy lifestyles, prevent medical mistakes, and improve the effectiveness of health systems thanks to active patients and citizens<sup>55</sup>. Patient organisations can help make specialist disease-specific information accessible and comprehensible for patients. Patient groups offer much more credible and therefore compelling health

**Patient organisations “translate” or disentangle complex information – whether scientific or policy-related – into readily understandable, relevant and accessible resources for the patient community in order to support patient advocacy and build health literacy.**



and/or treatment information than that provided by industries directly linked to commercial determinants of health and disease (food and drink, tobacco and alcohol, for example). The information on healthy diets and diabetes epidemiology and treatment provided by a diabetes patient group is likely to differ from that provided by a company producing sugary beverages. Patient organisations produce and review existing health-related information, sometimes translating it into local languages or making it more context-relevant<sup>56</sup>. They also develop guidelines for communicating information to patients in a user-friendly way and set standards for high-quality health education materials. Health literacy efforts are also aimed at educating patients on

<sup>50</sup> EURORDIS, Living with a Rare Disease. Available at: <http://www.eurordis.org/living-with-a-rare-disease>

<sup>51</sup> European Haemophilia Consortium, Haemophilia Stories: Documentary and Short videos, (2015). Available at: <https://www.ehc.eu/document/haemophilia-stories-short-videos/>.

<sup>52</sup> For example EPF’s conference on patient empowerment, cross-border healthcare or patient safety. Available at: <http://www.eu-patient.eu/Events/past-events/>.

<sup>53</sup> European Multiple Sclerosis Platform, Practical Toolkit for Employers on Supporting People with Multiple Sclerosis in the Workplace. Available at: <http://www.emsp.org/wp-content/uploads/2016/01/PPP-Working-Out-MS-in-the-Workplace-Toolkit-Jan2016.pdf>.

<sup>54</sup> World Health Organization, Health literacy: the solid facts. WHO Regional Office for Europe. Copenhagen, Denmark. (2013). Available at: [http://www.euro.who.int/\\_data/assets/pdf\\_file/0008/190655/e96854.pdf](http://www.euro.who.int/_data/assets/pdf_file/0008/190655/e96854.pdf)

<sup>55</sup> The Health Literacy Coalition, Health Literacy Infographic (2016) Available at: <http://www.eu-patient.eu/globalassets/policy/healthliteracy/health-literacy-infographic.pdf>

<sup>56</sup> Health literacy proofing, including adherence to scientific correctness.



their rights as healthcare consumers, and on general anti-discrimination and equal treatment rights (not exclusively restricted to health), particularly among disadvantaged and marginalised groups<sup>57</sup>. Finally, information linked to health, disease or treatment may simply need to be translated into the concrete implications on patients' daily life in areas such as nutrition, employment, social inclusion or reimbursement. For these reasons health information should not only be written for patients; it should be developed with patient input<sup>58</sup>.

Information linked to epidemiology, treatment or quality of life may be complex (scientifically, legally, or financially) and both the production and the dissemination often requires a multi-stakeholder approach involving health professionals, advocacy groups and community-based organisations, groups at risk of low health literacy, researchers, European institutions and national governments, the general public, news and digital media, and the business community. Ideally, initiatives to build health literacy need to be grounded in the settings of people's everyday life, linked to their community, and rooted in collaborative learning and social support to sustainably take off - and patients are legitimate stakeholders in making sure these initiatives reflect their reality and their needs<sup>59</sup>. An example of concrete work to increase patient's treatment literacy is the EATG HIV/AIDS Treatment Literacy Training Manual (2010), accompanied by a Treatment Advocacy Manual (both in

English and Russian since it is mostly aimed at the newer Member States and EU neighbourhood countries in Central and Eastern Europe)<sup>60</sup>.

EPF's response to the European Commission's public consultation on the guideline for the drafting of the "Summary of Clinical Trial Results for Laypersons" is a good exposition of the principles which should underlie this work<sup>61</sup>. The document stressed the importance of transparent and publicly accessible lay-, patient- and non-health professional-friendly information, and underlined the importance of communicating this in an unbiased, comprehensive and relevant way<sup>62</sup>. EPF emphasised that simple language of the lay summary of complex scientific information should not come at the price of factual inaccuracy or the introduction of bias. The EPF recommendations contributed to the formation of a multi-stakeholder working group, including several patient representatives, which developed a European good practice guideline for lay summaries<sup>63</sup>.

In the era of digital health information, health literacy has become all the more pressing: the internet has made a lot of information available but patients may have difficulty making sense of it all and working out which of the many available sources are credible sources of information - in particular those concerning treatment options or self-care. This is where patient organisations can add tremendous value.

Another enabling function of patient organisations in the educational area is helping patients understand which clinical trials are available and what it will mean for them to be a participant in a trial. There has been important progress in the development of new and precision therapies for cancer, for example. As a result, multiple clinical trials can be in operation at the same time, which gives patients many opportunities to take part in one. Even though researchers should explain in detail to patients what it means to be a participant in such trials, legal concerns and professional jargon often make the wording unnecessarily difficult to understand for the layperson. Patient organisations have an important role to play in guiding patients through some of these complexities, and all the more in advocating for the information related to patient participation in clinical trials to be made available in an understandable and non-ambiguous way.

The results of the EPF survey show that 50% of the respondents are currently involved in "capacity-building activities" of some sort, with another 10% who either plan on delivering some in the future or who have done so in the past. Most of the activities consist of training modules, thematic conferences and seminars, with a small percentage of study visits or individual coaching. The majority of the capacity-building activities are aimed at members of the organisations themselves and a small percentage at the wider patient community or general public (see Annex).

<sup>57</sup> Health literacy increasing activities may include information to patients related to their socio-economic determinants of health, for example issues related to legal difficulties in medicines reimbursement, poverty, social exclusion, homelessness or domestic abuse as a result of a chronic disease or disability.

<sup>58</sup> Denegri and Faure (2013). "It's plain and simple: transparency is good for science and in the public interest." *Trials* 2013, 14:215

<sup>59</sup> De Wit, L. et al. (2017). Community-based initiatives improving critical health literacy: a systematic review and meta-synthesis of qualitative evidence. *BMC Public Health* 2017. Available at: <https://bmcpublichealth.biomedcentral.com/articles/10.1186/s12889-017-4570-7>

<sup>60</sup> European AIDS Treatment Group (EATG), Toolkit. Available at: <http://www.eatg.org/wp-content/uploads/2016/06/Treatment-Literacy-EN.pdf>.

<sup>61</sup> EPF, EPF's response to the European Commission's public consultation on the Summary on Clinical Trial Results for laypersons", (2016). Available at: <http://www.eu-patient.eu/globalassets/policy/clinicaltrials/epf-response-to-the-lay-summary-public-consultation--august-2016.pdf>

<sup>62</sup> Other examples include: 1) [www.patientslikeme.com/clinical\\_trials](http://www.patientslikeme.com/clinical_trials); 2) disease-specific sites such as European Huntington's Disease Network (<http://www.euro-hd.net/html/network>); 3) HealthTalk online (<http://www.healthtalk.org/peoples-experiences/medical-research/clinical-trials/topics>); 4) NHS Choices has extensive lay-friendly content on research (<http://www.nhs.uk/conditions/Clinical-trials/Pages/Introduction.aspx>); 5) The James Lind Alliance and UK DUETS database address treatment uncertainties from patients' and other perspectives (<http://www.library.nhs.uk/DUETS/>; <http://www.lindalliance.org/>).

<sup>63</sup> The guidelines were published on 3 August 2017 and can be found here: [https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017\\_01\\_26\\_summaries\\_of\\_ct\\_results\\_for\\_laypersons.pdf](https://ec.europa.eu/health/sites/health/files/files/eudralex/vol-10/2017_01_26_summaries_of_ct_results_for_laypersons.pdf)

## 2.4. Peer support

The role that patient organisations play in the area of peer support consists of providing knowledge, sharing experiences, and offering emotional, social or practical help to individual patients. It can take a number of forms such as mentoring, counseling or listening.

Beyond these traditional roles, more and more disease-specific patient organisations at the national level step in to provide legal and financial support for individual patients and co-deliver self-management education and develop support for patients into the wider community. Training individual patients as community-based peer counselors could be considered as such an outreach programme. In the field of legal support, an interesting example is the sharing of legal best practices, defending patients' rights and monitoring compliance with EU legislation by Member States done by the the European Cancer Patient Coalition (ECPC) Legal Network for Cancer Patients<sup>64</sup>.

The results of the EPF survey show that 37% of the respondents currently offer support of some sort to individual patients. This mostly concerns social support (36%), legal (27%) and administrative help (27%)<sup>65</sup>. Furthermore, some communication-related activities are identified as peer-support as they tend to increase individuals' opportunities

to navigate the "information jungle" and enhance their health literacy. This emphasises the close link between some of the capacity-building and educational initiatives discussed above, as well as the role played by some patient organisations in offering holistic and sustainable support to patients on an individual basis.

## 2.5 Research and Development: health and pharmaceuticals

When looking at the unique value of patient organisations with regard to research, it is important to make a clear distinction between health and social research, and pharmaceutical research and development (R&D).

### 2.5.1 Health research

Patient-centric or patient-led research gives a unique social relevance to new and innovative ways of science co-creation. Patients are well-placed to provide more qualitative empirical data that enriches the political and practical narrative within the health research area through contextual background, wider community development, and empowerment.

There is a multitude of surveys and registers created by (mainly pan-European) disease-specific organisations that are instrumental in the area of data-collection. For example, Alzheimer Europe<sup>66</sup>

publishes yearly reports on the state of Alzheimer disease policies and treatments across Europe; the last one is a comparative benchmarking exercise on national dementia policies and strategies<sup>67</sup>. EURORDIS, the European Organisation on Rare Diseases, carried out a Europe-wide survey on the social impact of rare diseases that was filled in by 3000 rare disease patients and their carers<sup>68</sup>. Similarly, EMSPs' Voice of MS patients survey was completed by 2700 people living with multiple sclerosis (MS) in 33 countries across Europe<sup>69</sup>. The involvement of patient organisations in data collection helps researchers and decision-makers to define and share priorities and allocate adequate research budgets.

The geographical transferability of research data is becoming an increasingly important consideration<sup>70</sup>. Better transferability of data should also make it easier to use health data gathered in other fields of research such as behavioural sciences, health psychology, anthropology, philosophy and sociology. Patient organisations already actively collaborate in research by maintaining registries, assembling biobanks and gathering information.

Digitalisation has given birth to a new type of research: that of digitally enabled and patient-led research. The increase in community/patient/citizen-led science has given important impetus to new ways of data collection and interpretation. Innovation in

<sup>64</sup> European Cancer Patient Coalition (ECPC) Legal Network for Cancer Patients. Available at: <http://www.ecpc.org/activities/legal-network-for-cancer-patients>

<sup>65</sup> From a statistical point of view, however, the number of respondents is quite low to be able to derive generalisations.

<sup>66</sup> Alzheimer Europe is a non-governmental organisation aimed at raising awareness of all forms of dementia by creating a common European platform through coordination and cooperation between Alzheimer organisations throughout Europe. Alzheimer Europe is also a source of information on all aspects of dementia.

<sup>67</sup> Alzheimer Europe, Report "European Dementia Monitor 2017-Comparing and benchmarking national dementia policies and strategies". Available at: <http://alzheimer-europe.org/Publications/E-Shop/European-Dementia-Monitor-2017/European-Dementia-Monitor-2017>

<sup>68</sup> Bearryman, E., EURORDIS, Survey (2017). Available at: <http://www.eurordis.org/news/3000-rare-disease-patients-carers-voice-difficulties-balancing-care-life>

<sup>69</sup> European Multiple Sclerosis Platform, Voices of MS patients (2016). Available at: <http://www.emsp.org/projects/voice-ms-patients>

<sup>70</sup> Goeree, R., et al. Transferability of health technology assessment and economic evaluation: a systematic review of approaches for assessment and application, *Clinico-econ Outcomes Res.* 2011; 3: 89–104. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3169976/>.

the world of data collection is well under way via smartphones, personal genomics, wearable devices and digital health records, as well as through websites such as “Patients Like Me” where people can share their health data to track their progress, help others and improve research. Nesta, an innovation foundation, states that: “New data is produced, owned and controlled by patients, and it will be accessed on their terms – as active participants rather than passive subjects. The richest opportunities will arise when patients act as citizen scientists, actively measuring and interacting, within communities where they have a powerful voice in the direction and conduct of learning. In other words, the next generation of research will be patient-led”<sup>71</sup>

Patient organisations are often represented in European research consortia, where they provide added value by identifying patient priorities, bringing the patient perspective on living with the condition and the impact of different treatments, and using their extensive networks to ensure patient involvement at different levels.

Examples of general health-related projects with a substantial role for patient organisations are the European Joint Action on chronic diseases (CHRODIS), - the European Network on patient safety and quality of care (PaSQ), - the PRO-STEP tender study on self-management, and SUSTAINS on eHealth and SmartCare on integrated care.

Patient involvement in peer-reviewed journals and in the dissemination of scientific knowledge is slowly gaining traction. In 2014, the British Medical Journal (BMJ) adopted its groundbreaking “patient partnership” strategy<sup>72</sup>, which means, among other things, that authors wishing to submit articles to the journal are asked to co-produce these with patients; scientists are asked to show how they involved patients in setting the research question, outcome measures and other aspects of the study; and patients now review papers alongside the standard peer review process. In addition, the BMJ has patient editors and runs a series of articles and blogs, such as “What your patient is thinking”<sup>73</sup>

The BMJ took this step because partnering with patients, families and carers is seen as an ethical imperative and essential to improving the quality, safety, value, and sustainability of health systems. The journal also has an international patient advisory panel<sup>74</sup>. It is also important to mention patients and patient groups’ involvement in the co-design, development, application and monitoring of disruptive innovations for healthcare<sup>75</sup>.

Such innovations are increasingly attractive to health technology researchers as they promise relatively cheap and quick solutions to a number of challenges. An example of this is the EMSPs’ collection of MS-related health applications on treatment and management practice, cognitive

enhancement strategies and practical exercises<sup>76</sup>.

The study undertaken by the European Commission in 2016 on “Big Data in Public Health, Telemedicine and Healthcare” sees tremendous potential in exploiting ‘Big Data’ in health through almost limitless digitalisation of health<sup>77</sup>. Since the appetite for digitalisation is only growing, it is of great importance for patient groups to promote the interests of patients whilst also acting as watchdogs of their privacy and safety by demanding and promoting good governance and regulation of data access and use. By reaching out to policy-makers and regulators, patient organisations can use the “Big Data” discourse to have patient benefits reflected in better pharmacovigilance and patient safety.

In the EPF survey, 70% of the respondents declared that they are involved in one or more of these forms of data collection. This data is either used for general health research purposes, for enhancing the knowledge base of patient organisations themselves, or for pharmaceutical R&D. The respondents in the survey declared that they specifically use their data for the following purposes: patient experience surveys, data collected for peer-reviewed medical journals, complaints, collecting best practices and identifying best examples of legislation.

<sup>71</sup> Loder, J., Nesta, Patients become citizens Scientists, Available at: <http://www.nesta.org.uk/2016-predictions/patients-become-citizen-scientists>

<sup>72</sup> British Medical Journal (BMJ). Available at: <http://www.bmj.com/campaign/patient-partnership>;

<sup>73</sup> British Medical Journal (BMJ). Available at: <http://www.bmj.com/specialties/what-your-patient-thinking>;

<sup>74</sup> British Medical Journal (BMJ). Available at: <http://www.bmj.com/about-bmj/advisory-panels/patient-panel-members>

<sup>75</sup> Disruptive innovation vs. sustained innovation in healthcare is a system or a device that disrupts the way we use technology in healthcare industry - replacing existing technology and creating a new market demand. Common examples include smartphones and tablets, but also such innovations like interoperability, 3D printing, digestible sensors or home diagnostics.

<sup>76</sup> European Multiple Sclerosis Platform, Resources and Apps. Available at: <http://www.emsp.org/resources/apps/>.

<sup>77</sup> European Commission, Study on Big Data in Public Health, Telemedicine and Healthcare, (2016). Available at: [https://ec.europa.eu/health/sites/health/files/ehealth/docs/bigdata\\_report\\_en.pdf](https://ec.europa.eu/health/sites/health/files/ehealth/docs/bigdata_report_en.pdf)

## 2.5.2 Pharmaceutical Research and Development

Historically, patients have not played a major role in product development beyond participation in clinical trials; however, this paradigm is changing. The landscape for research and development of new medical products is evolving and continues to become more patient-centric – at least on the level of rhetoric, though not always in practice. Patient involvement and input into the early stages of research and development of therapies is increasingly recognised as being as critical to improving health care as patient engagement after approval<sup>78</sup>.

Researchers and drug developers are increasingly engaging patients before products (including therapeutical products and medical devices) enter the market. This is to ensure that new therapies are designed to meet the needs and priorities of patients, and that the clinical trials conducted to inform regulatory approval, subsequent cost-benefit assessment and eventual clinical use, capture information that is highly relevant and specific to the end-users – resulting in an accurate assessment of the extent to which the new therapy offers additional benefits for patients. Although efforts among stakeholders have been fragmented

and uncoordinated to date, a path forward is beginning to emerge<sup>79</sup>

The European Medicines Agency (EMA) Patients' and Consumers' Working Party (PCWP) topic group on measuring the impact/value of patient involvement in EMA activities has been recommending more patient involvement in the regulatory process in their report and national medicines agencies are following these recommendations<sup>80</sup>. Patient groups have taken a leading role in this by building capacity and raising awareness among themselves and other stakeholders to facilitate patients and community involvement in the development and review of clinical trials protocols, as demonstrated by the "Activist's Protocol Review Toolkit" promoted by the Treatment Action Group and its European branch the European AIDS Treatment Group (EATG)<sup>81</sup>.

Patient organisations are giving their constituents information on where to turn for participation in patient access programmes and generally helping them understand the complexities of the regulatory process. They engage in awareness raising with the pharmaceutical industry and are working together with national medicines agencies. According to a survey carried out in the United States

by Eye for Pharma, just over half of their respondents (patient advocates) have established advocate criteria that enable them to provide the patient perspective for biopharmaceutical trial design<sup>82</sup>. Despite the increasing recognition of the critical role of patient involvement in R&D there are however still fundamental barriers preventing a real shift to full integration of patients as a central part of this process. The next section of this report considers these, amongst the other challenges that patient organisations face.

<sup>78</sup> Pogorelc, D., MedCityNews, What's behind the FDA's push for more patient engagement, (2013). Available at: <http://medcitynews.com/2013/05/whats-behind-the-fdas-push-for-more-patient-engagement-and-its-not-that-everyone-else-is-doing-it/>

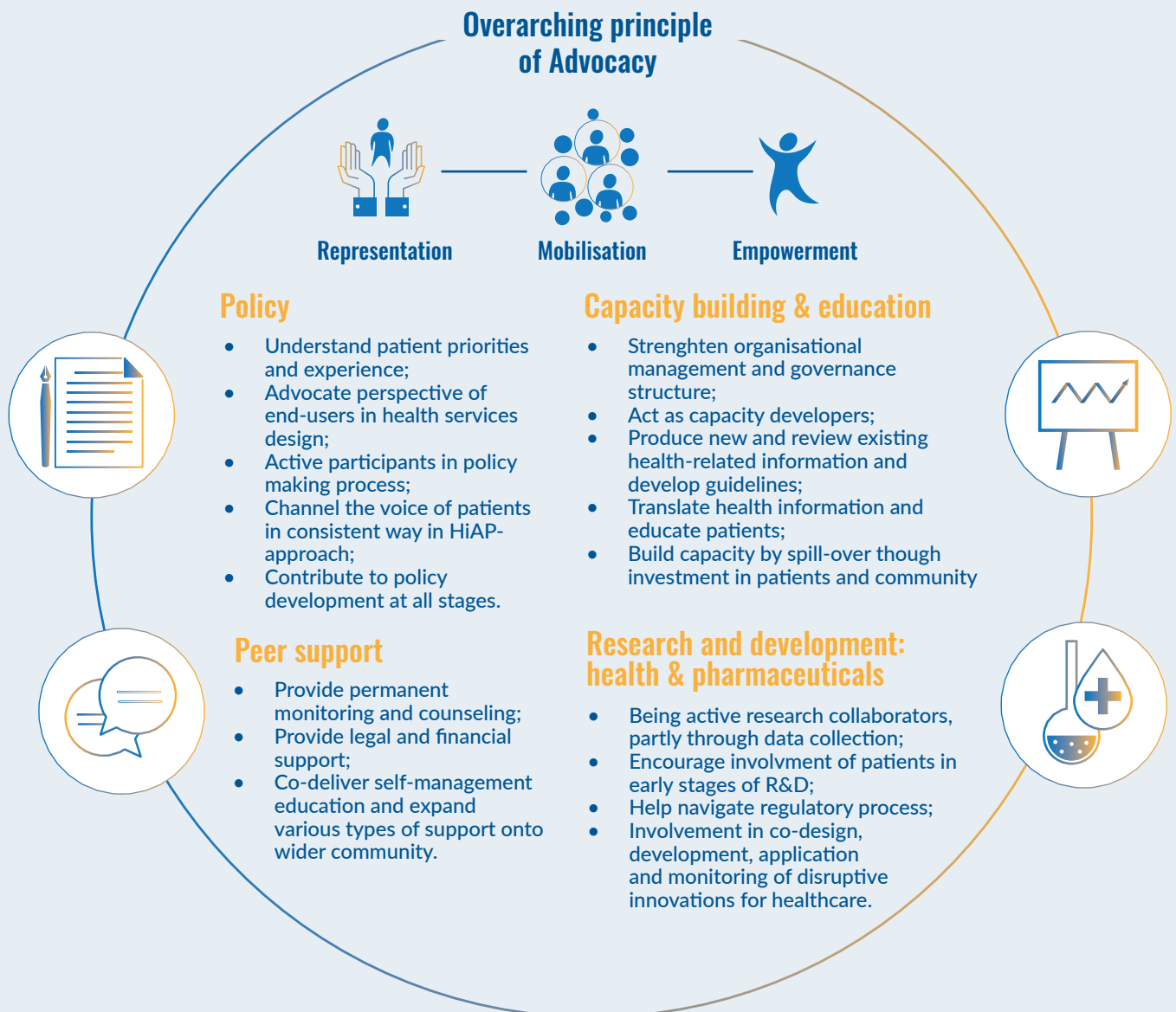
<sup>79</sup> National Health Council, Dialogue/Advancing meaningful patient engagement in Research, Development, and Review of Drugs, (2015). Available at: <https://www.nationalhealthcouncil.org/sites/default/files/PatientEngagement-WhitePaper.pdf>

<sup>80</sup> European Medicines Agency, Consolidated report on the activities of topic groups established in 2015, (23/05/2017). Available at: [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Annual\\_report/2017/05/WC500228518.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Annual_report/2017/05/WC500228518.pdf)

<sup>81</sup> Treatment Action Group, An Activist's Protocol Review Toolkit, (2017). Available at: <http://www.eatg.org/news/tag-an-activists-protocol-review-toolkit/>

<sup>82</sup> Eye for Pharma, 'What pharma can learn from patients: a Global patient advocate survey', (2016). Available at: [http://social.eyeforpharma.com/pharmaceutical-indepthanalysis?qt-in\\_depth\\_analysis\\_tabs=3#qt-in\\_depth\\_analysis\\_tabs](http://social.eyeforpharma.com/pharmaceutical-indepthanalysis?qt-in_depth_analysis_tabs=3#qt-in_depth_analysis_tabs).

## Value of patient organisations



# 3 Challenges for patient organisations

Patient organisations face a considerable number of challenges in their work. These challenges can be internal (resources -both human and financial-, organisational professionalism and lack of performance measurement) and external (lack of legally-acknowledged right to public funding, systemic failure of collaboration, tokenism and the legislative gaps).

## 3.1 Internal challenges

### 3.1.1 Performance measurement

Healthcare systems in Europe need to be built in cooperation with patients as end-users, and on the principle of shared responsibility for preventing diseases at community and society level: the value of patient involvement in policy and research should be clear to all, and such involvement should become an objective in itself. While patient organisations are asked to measure their performance and impact, the question of why patient organisations are subject to impact measurement in the first place is rarely asked. Not only is this notoriously difficult to do, as numerous research projects have shown, but it is also unfair: this level of scrutiny is not routinely applied to other health stakeholders such as the pharmaceutical industry, health insurers or health professionals' associations. To date, a number of systematic literature reviews have reported the various ways in which patient groups' involvement makes a difference to health research and policy-making<sup>83</sup>. However, this evidence has been criticised as



being weak and anecdotal<sup>84</sup>. As they are just one actor amongst many in complex policy-making processes, it is hard to identify and quantify the exact impact of patient groups on the final outcome. For this reason, it is difficult to engage in a counterfactual analysis of the impact on any given policy of no patient involvement. Most popular impact assessment tools do not measure the weight of the contribution of a single stakeholder in a final outcome; neither do they consider measuring the concrete effect of meaningful involvement of patients or civil society representatives as such.

When it comes to counterfactual analysis, the closest possible activity would be foresight or modelling studies, which are not often utilised.

Recommendations from the PCWP topic group on "Measuring the impact/value of patient involvement in EMA activities" clearly state that "looking at patient input from a more holistic view enables a shift from a rather narrow (quantitative) measuring of impact to a wider approach that highlights and identifies the added value of patient involvement, which includes qualitative methods"<sup>85</sup>.

<sup>83</sup> Brett J., Staniszewska S, et al. Mapping the impact of patient and public involvement on health and social care research: a systematic review. *Health Expect.* 2014;17(5):p. 637–50.

<sup>84</sup> Staniszewska S., et al. Developing the evidence base of patient and public involvement in health and social care research: the case for measuring impact. *Int J Consum Stud.* 2011;35(6):p. 628–32.

<sup>85</sup> European Medicines Agency, Patients' and Consumers' Working Party (PCWP) / Healthcare Professionals' Working Party (HCPWP) topic group, Consolidated report on the activities of topic groups established in 2015, (2017). Available at: [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Annual\\_report/2017/05/WC500228518.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Annual_report/2017/05/WC500228518.pdf).

### 3.1.2 Organisational professionalisation and know-how

A major internal challenge for patient organisations is that they often lack the mechanisms to develop or strengthen their strategic planning, improve their organisational development and capacity for fundraising, and professionalise their organisational behaviour. This can be for several reasons, such as a lack of earmarked funding, understaffing, a lack of particular skills, or an underdeveloped knowledge base (where the patient organisation does not have access to information on how to strengthen and professionalise its organisation)<sup>86</sup>.

Language and communication often constitutes another important challenge for patient groups in taking full advantage of their opportunities. The technical and bureaucratic language used by EU institutions and national authorities means that it can be time-consuming and frustrating for organisations to work out what is going on and how they can be involved. In pan-European work, the language barrier is less of a problem than it once was, but still remains a challenge for some organisations - some 10% of the respondents of the EPF study still say so. This can be a problem for the effective cascading of information up and down the structure of the organisation (where the “top” level often works in English but the grassroots-level patient advocates do not). Language can also

be a barrier to getting a response from the grassroots community to consultations on EU topics, or in finding a suitable representative to attend an international meeting as these are often conducted in English.

## 3.2 External challenges

### 3.2.1 Culture and tradition of tokenism

The survey performed and the interviews with stakeholders carried out in preparing for this report confirmed a relatively strong negative institutional culture and tradition of tokenism when it comes to involvement of patient organisations. Even though the situation has improved in recent years, much work, both on the side of patient groups and institutions, still needs to be done in order to fully acknowledge - and make the most of - the unique value of patient organisations.

### 3.2.2. Legislative gaps

As well as a culture of tokenism, there is a lack of EU legislation on meaningful patient involvement and systematic cooperation in contexts beyond specific clinical or medical issues (i.e. those tied to the treatment of individual patients), making the work of patient organisations difficult. If legislation does exist, it is either not properly enforced or it is too restrictive. Almost a quarter of the respondents of the EPF survey identified these issues as a weakening

factor in their daily work. While there are various legislative initiatives and frameworks at national level across Europe, no systematic overview has been made to date. Involving patients in health policy and health-related decision-making (in line with HiAP and ‘beyond-GDP’ discourse for example) has slowly but steadily taken off although there is still quite a patchy legislative landscape and a long way to go before patient involvement will come to its full fruition. This includes references to patients in legislation governing health or research budgets and priorities, transparency and governance issues, gender equity or food systems’ production and consumption (to name but a few examples). This lack of unenforced or restrictive legislative space makes it difficult for patient organisations to have a workable mandate.

### 3.2.3 Resources and funding

Another challenge for patient organisations at the European, national and local level is the lack of explicitly earmarked budgets devoted to systematic involvement of individual patients and patient groups. Until recently, most health budgets focused on addressing diseases: deliberating who to involve and how in tackling their determinants, management and treatment is a relatively new exercise in health policy-making. Adding to this problem is the fact that patient representatives, often operating on a voluntary basis, are often seen as *pro publico bono* - and not expert -

<sup>86</sup> EPF is working on Transparency guidelines (expected in November 2017) to provide guidance to patient organisations, to ensure the highest possible standards for the network and to increase the level of trust from other stakeholders in patient cooperation.

figures and subsequently are denied the right to financial compensation for their time and contribution to various activities.

Patient organisations often identify the lack of resources in the widest sense of the word as preventing them from reaching their full potential. Of the respondents involved in the EPF survey, 40% claimed that they are lacking the appropriate unrestricted and/or core funding with additional 13% lacking resources and capacity to do work beyond delivering “project-related products” which attract specific funding.

**“The challenges most patient organisations have is that they do not have a lot of independent resources. The resources are tied to a project. As a result they have many demands with a very limited amount of resources.”**

**Adrian van de Hoven, Director  
General Medicines for Europe**

Several interviews with relevant health stakeholders also confirmed that the lack of (independent and unearmarked) funding for patient organisations is a significant barrier to optimal organisational performance.

EPF made the following recommendations for the resourcing of patient groups in its 2016 Roadmap for Action on Patient Empowerment: “at European level, a strategy should be developed and implemented for sustainable ‘core grants’, including adjustment of the financial legitimacy criteria to enable more patient groups to become eligible; at national level,

Member States should explore together with patient organisations and private sector partners innovative and ethical options for funding patient groups that will enable them to function and maintain their independence from industry and from the government”<sup>87</sup>.

### 3.2.4 Credibility

Patient organisations face a lack of formal recognition as credible stakeholders and partners in health policy debate in general, and in non-health policies in particular. Patients’ experiences are often discredited as either lacking objectivity and independence from the views of the pharmaceutical industry or lacking a wider perspective beyond that of their disease/condition. EMA’s own research on “Professionalisation and representativeness among civil society representatives” asserts that NGOs need a certain level of professionalism to be able to work with international governance<sup>88</sup>. However, it also recognises that with greater professionalisation, there is a risk of losing representativeness. It is assumed that groups put the majority of their time and resources into activities aimed to influence and not into membership-focused activities.

As groups acquire the highest level of integrity and competence - become more ‘professional’ - they lose the ability to speak for their constituents due to changes in their internal organisation. This can ultimately lead to a tendency of groups towards less reliance on grassroots and increasing dependence on experts as well as elite-level contacts over time<sup>89</sup> referred to as professionalisation. Patient organisations need to find ways of ensuring that they can reap the benefits of greater professionalisation - most obviously,

greater effectiveness - without losing their essential representativeness.

In the face of diminishing funding from public health and health research budgets, patient organisations look to private sources of funding. Whilst a minority has decided not to accept any funding from industry, most patient groups receive some funding from the pharmaceutical industry - either on a project basis or for operational activities. This can decrease public trust in the independence and the representativeness of those organisations, and indeed the sector in general and have a negative impact on their credibility. This is partly due to a lack of understanding by external stakeholders of why and how patient organisations work with industry partners, and how they guard themselves against conflicts of interest or loss of independence. Awareness in the patient community of the vital importance of transparency and ethical conduct - on both sides - has grown as patient organisations have become more professional.

To mitigate perceived conflicts of interest, many patient organisations have established clear rules for transparency, ethical conduct and rules of engagement. In 2007 EPF, together with the European Federation of Pharmaceutical Industries and Associations (EFPIA), developed the “Code of Practice on Relationships between the Pharmaceutical Industry and Patient Organisations”<sup>90</sup>

The Code aims to ensure transparency in the relationship between patients and the pharmaceutical industry in a strong framework based on mutual respect that preserves the independence and autonomy of both parties.

<sup>87</sup> European Patients’ Forum, Available at: <http://www.eu-patient.eu/campaign/PatientsprescribE/roadmap-for-action/>.

<sup>88</sup> Gadd, C., European Medicines Agency, Professionalisation and representativeness among civil society representatives. Available at: [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Presentation/2012/05/WC500127916.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Presentation/2012/05/WC500127916.pdf).

<sup>89</sup> <https://ecpr.eu/Filestore/PaperProposal/e727a8d0-557d-486e-8922-af563929f245.pdf>.

<sup>90</sup> European Federation of Pharmaceutical Industries and Associations, Available at: [http://transparency.efpia.eu/uploads/Modules/Documents/code\\_po2011.pdf](http://transparency.efpia.eu/uploads/Modules/Documents/code_po2011.pdf).



# 4. Conclusions and key messages

## 4.1 Scope of the report

This report has been written in order to show the unique value that patient organisations have. It is based on data collection through a survey done by EPF amongst its members, desk research and interviews with important stakeholders in the field of health. The aim of the report is to get a clear overview of the role patient organisations play in the field of health in Europe and what they can do in order to further their invaluable efforts for patients on a daily basis. This last section gives an overview of the main activities undertaken and the challenges faced by patient organisations. It also proposes a number of key messages for patient organisations, European and national policymakers, academic and industrial researchers involved in projects, and academia and professional educators.

## 4.2 Overview of activities and challenges

The four major areas where patient organisations have a unique value are: (1) policy; (2) capacity-building and education; (3) peer support; and (4) research & development (health and pharmaceutical). From each of these areas their most important activities are set out (on the right).

### 4.2.1 Main activities

#### Policy

##### Patient organisations:



- Play a major role in helping policymakers understand patient priorities and experiences of living with a disease/condition;
- Give their invaluable perspective in health services design as the end-users in health services;
- Channel the voice of a community of patients by representing their interests in a united, coherent and consistent way;
- Have a key role in developing and contributing to the HiAP approach alongside other health stakeholders;
- Provide policy input (direction, evidence, implications) and offer amendments to policy proposals through stakeholder advisory groups, expert panels, European and/or national government public consultations or institutional meetings.

#### Capacity Building and Education

##### Patient organisations:



- Strengthen organisational management and governance of patient groups, and improve their credibility and professionalism through sharing of good practices, tools and methodologies;
- Produce and review health-related information; develop guidelines for communicating information to patients in a user-friendly way;
- Improve health literacy in general through lay-friendly health information;
- Translate complex health information into lay language and formats and into local languages, making it context-relevant;
- Educate patient communities, the public and other stakeholders about patients rights;
- Channel information throughout the healthcare sector (top-down, bottom-up and horizontally);
- Educate health professionals, e.g., by participating in medical conferences.

#### Peer Support

##### Patient organisations:



- Provide peer mentoring, counseling or listening;
- Provide legal and financial support;
- Enable patients to get networked across borders, providing platforms for exchange and communication;
- Co-deliver self-management education, often in collaboration with professionals and deliver various forms of support into the wider community in which patients live.

#### Research and development: Health & Pharmaceuticals

##### Patient organisations:



- Are increasingly active research collaborators, including through data collection;
- Encourage greater involvement of patients in the early stages of research, including in the development of pharmaceuticals;
- Help patients understand and navigate the complexities of the regulatory process;
- Educate researchers on how to work with patients;
- Are involved in co-design, development, application and monitoring of disruptive innovations for healthcare.

## 4.2.2 Main challenges

Patient organisations face many challenges when performing these activities. Patient organisations must continually demonstrate their credibility, becoming professional without compromising their representativeness. They face a lack of resources and funding, particularly unearmarked funding which can be used for general operational costs. When dealing with institutions, there is often systematic failure of cooperation and a tradition of tokenism in working with patient organisations. And there is often a lack of regulated legislative space on the European level to facilitate participation and access to resources.

All of these challenges undermine patient organisations' efforts to achieve their full potential and deliver the maximum added-value to the state of health (and society) in Europe.

## 4.3 Key messages

The roles that patient organisations play are constantly changing and evolving. Currently, patient organisations are very much demonstrating their value as co-designers, facilitators, conveners, innovators, policymakers, peer supporters, researchers, communicators, data-collectors as well as service providers and advocates. They can no longer be seen as a "third sector"; rather, "they, as the whole of civil society, should be (seen as) the glue that binds public and private activity together in such a way as to strengthen the common good"<sup>91</sup>

However, in order to be able to keep playing these roles and executing their numerous activities, overcoming internal and external challenges is necessary. Patient organisations need to keep their eyes on the future

by focusing on their core principles; that of representing, mobilising and empowering patients and advocating their rights. Patient organisations could consider ways to educate external stakeholders about what they do, the added value they bring, and why and how they work with industry partners. They can use the revolution in communications, technology, innovation, research and development to advance their connectivity, engagement and resilience in a changing world.

Patient organisations can not face these challenges alone. There is an important role to play for a number of other actors. The following recommendations can be taken into account by other stakeholders in their dealings with patient organisations.

<sup>91</sup> World Economic Forum, The Future Role of Civil Society, (2013). Available at: [http://www3.weforum.org/docs/WEF\\_FutureRoleCivilSociety\\_Report\\_2013.pdf](http://www3.weforum.org/docs/WEF_FutureRoleCivilSociety_Report_2013.pdf)

# List of abbreviations

<b>AIDS</b>	Auto-Immune Deficiency Syndrome
<b>BMJ</b>	British Medical Journal
<b>CPD</b>	Continuing Professional Development
<b>CHRODIS</b>	Joint Action on Chronic Diseases
<b>ECPC</b>	European Cancer Patient Coalition
<b>EE</b>	Economic Evaluation
<b>EFA</b>	European Federation of Asthma and Allergy Patients Associations
<b>EFPIA</b>	European Federation of Pharmaceutical Industries and Associations
<b>EHC</b>	European Haemophilia Consortium
<b>EMA</b>	European Medicines Agency
<b>EMSP</b>	European Multiple Sclerosis Platform
<b>EPF</b>	European Patients' Forum
<b>EU</b>	European Union
<b>EUFAMI</b>	European Organisation of Families Affected by Mental Illness
<b>EULAR</b>	European League Against Rheumatism
<b>EUPATI</b>	European Patients Academy
<b>EURORDIS</b>	European Organisation for Rare Diseases
<b>GDP</b>	Gross Domestic Product
<b>HIA</b>	Health Impact Assessment
<b>HiAP</b>	Health in All Policies
<b>HIV</b>	Human Immunodeficiency Virus
<b>HTA</b>	Health Technology Assessment
<b>IAPPO</b>	International Alliance of Patients Organisations
<b>IFSBH</b>	International Federation for Spina Bifida and Hydrocephalus
<b>MEP</b>	Member of European Parliament
<b>MS</b>	Multiple Sclerosis
<b>NGOs</b>	Non-Governmental Organisations
<b>PaRIS</b>	Patient-Reported Indicators Survey
<b>PCWP</b>	Patient and Consumer Working Party (of EMA)
<b>PREM</b>	Patient Reported Experience Monitoring
<b>PROM</b>	Patient Reported Outcome Monitoring
<b>R&amp;D</b>	Research and Development
<b>WHO</b>	World Health Organization

# Acknowledgements

EPF and the authors would like to thank the following people for taking the time to lend their expertise and provide insights for this report.

**Nicola Bedlington** is the European Patients' Forum Secretary General since September 2014. She was previously its Executive Director. She has also worked as an expert for the European Commission on disability policy and NGO cooperation. She was also the first Director of the European Disability Forum and led the ENSI Secretariat, an OECD initiated international government network on education and sustainable development.

**Carlota Besozzi** is coordinator at Civil Society Europe. In this role she represents 28 European networks of civil society organisations working on issues of equality, solidarity, inclusiveness and democracy. Before this position, she was Director at the European Disability Forum for ten years and she also worked in the European Parliament.

**Vanessa Challinor** joined Alzheimer Europe in 2015 as a policy officer. She develops policy statements and reports on Alzheimers' disease in Europe.

**Magda Gunn** works at Innovative Medicines Initiative (IMI) as a scientific project manager. IMI is Europe's largest public-private initiative for medicine development. She is responsible for relationships with patients as well as policies on diabetes and metabolic disorders. She holds a PhD from North Carolina State University.

**Robert Madelin** joined FIPRA International, a public affairs consultancy, in 2016 as an International Chief Strategist. He is now FIPRA's chairman. He is also a visiting Research Fellow at the University of Oxford's Department of Politics and International Relations. Previously (2004-16): Robert held a series of senior leadership positions at the European Commission: as senior adviser for innovation, as director general for communications networks, content and technology (CONNECT) and as director general for health and consumer policy (SANCO).

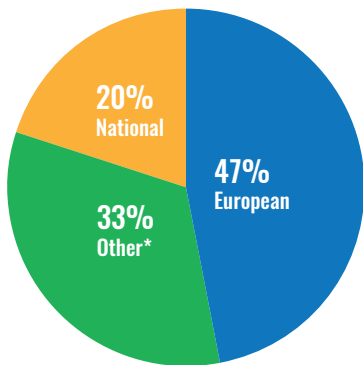
**Isabelle Moulon** is head of patients and healthcare professionals department at the European Medicines Agency (EMA). EMA is a European agency responsible for the protection of public and animal health through the scientific evaluation and supervision of medicines. Isabelle holds a PhD from Grenoble University.

**Sirpa Pietikäinen** is a Finnish Member of the European Parliament since 2008. She is a member of the National Coalition Party, part of the European People's Party. Sirpa supports European Patients' Forum activities and has promised to include the perspective of patients in her work in the Environment and Health Committee within the European Parliament. She is also Chair of the Allergy and Asthma Intergroup in the European Parliament which unites stakeholders to fight against the most prevalent chronic diseases in Europe.

**Adrian van den Hoven** is director general for Medicines for Europe, an organisation that represents the generic and biosimilar medicines industries in Europe. Prior to this role, he was director and deputy general-secretary of the largest trade organisation in Europe, BusinessEurope. He holds a PhD from the University of Nice.

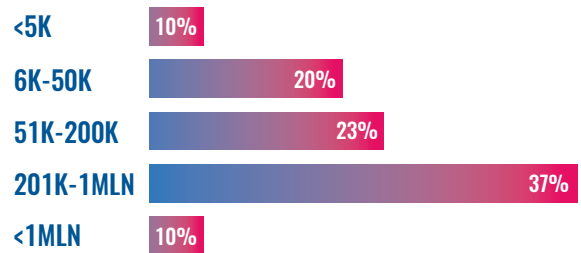
# Results of EPF Members survey

## Are you a European or national level organisation? (n=30)



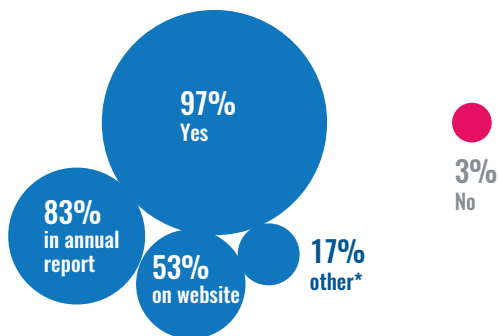
\* mostly a mix of both European and national

## What is your operational budget?



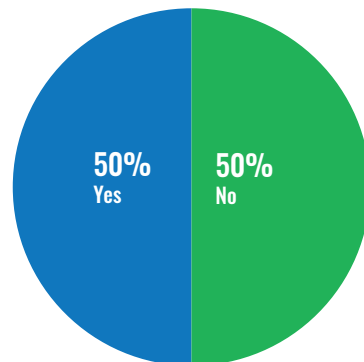
Note: due to various currency reported, no uniform understanding of 'operational budget' criterium, no uniform reporting of proportions of budget received from different sources, it was difficult to establish accurate figures for part of organisational budget received from public, private or other categories of funders

## Do you make your sources of funding public? (n=30)



\* on government site, in financial report, annual conference

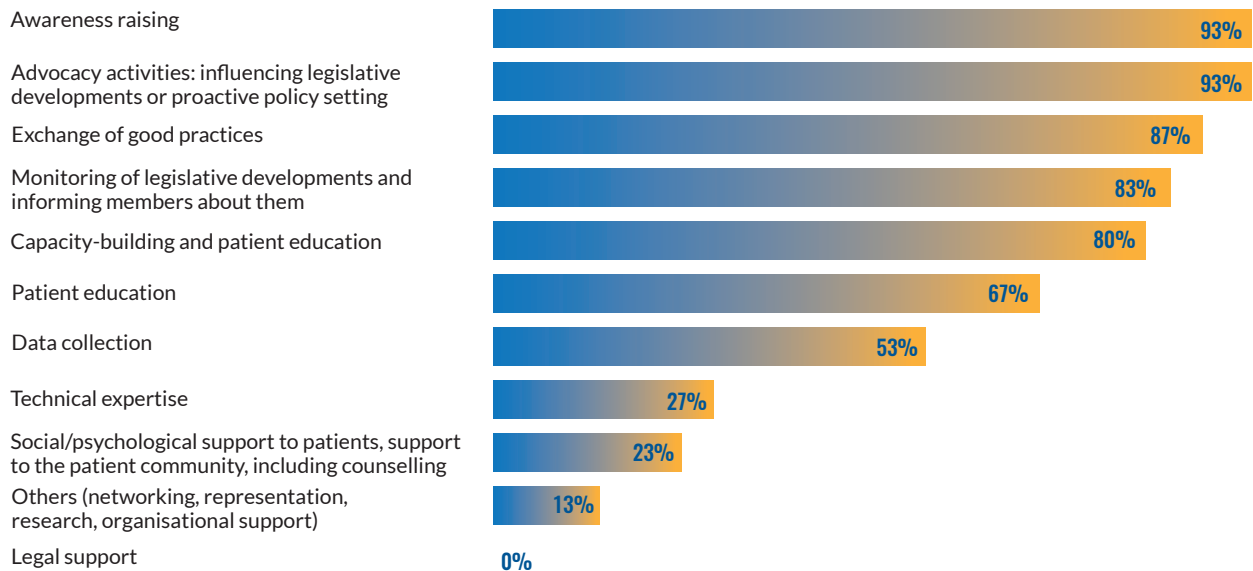
## Do you have a "code of ethics" or "guiding principles for fundraising"? (n=30)



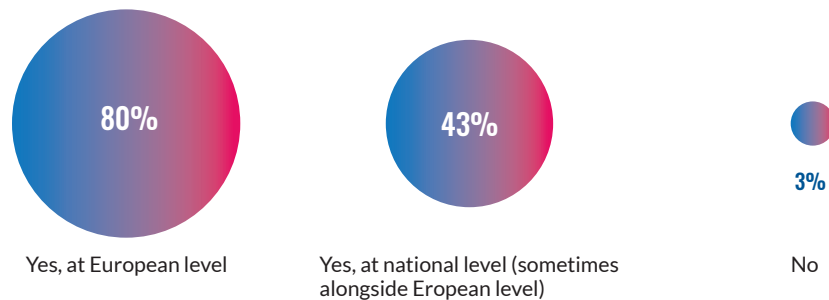
### Is your organisation registered on the EU Transparency Register? (n=30)



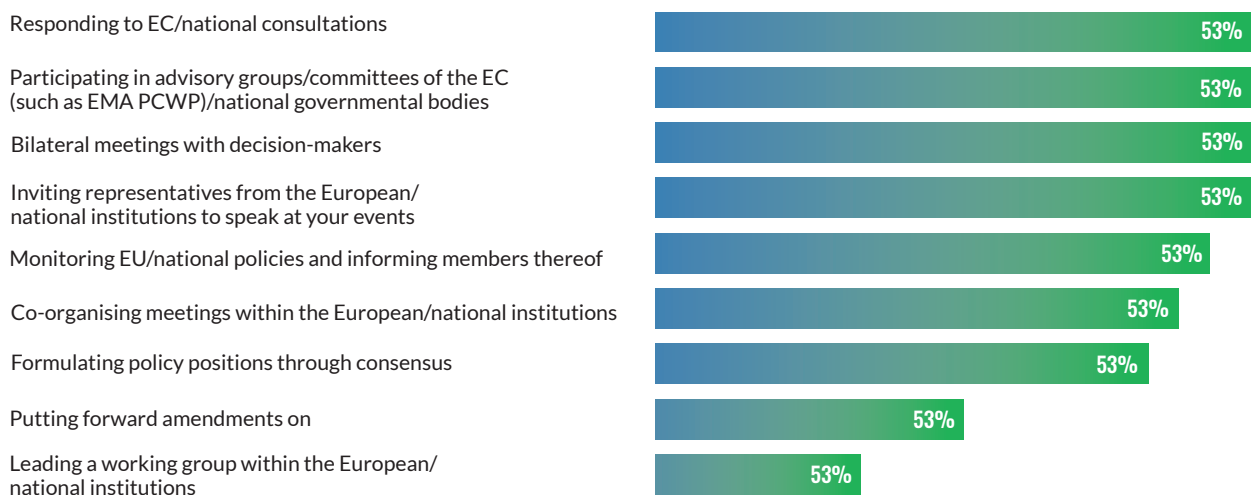
### What are the main roles/activities of your organisation? (n=30)



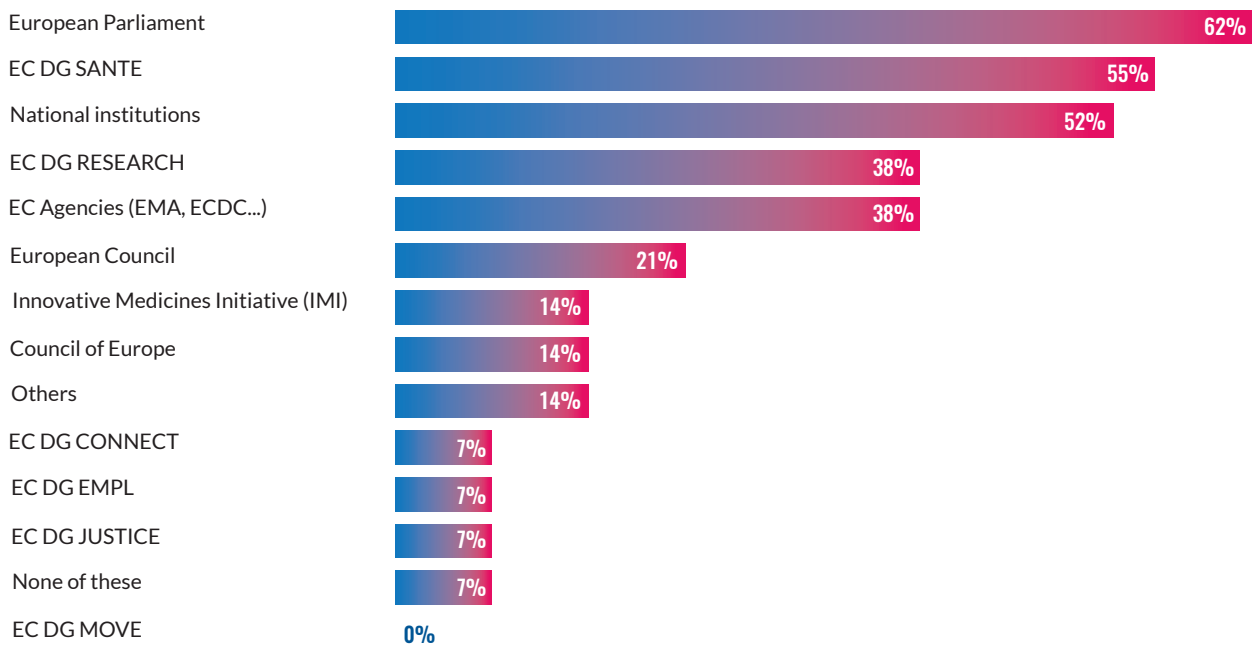
### Does your organisation conduct any advocacy work? (n=30)



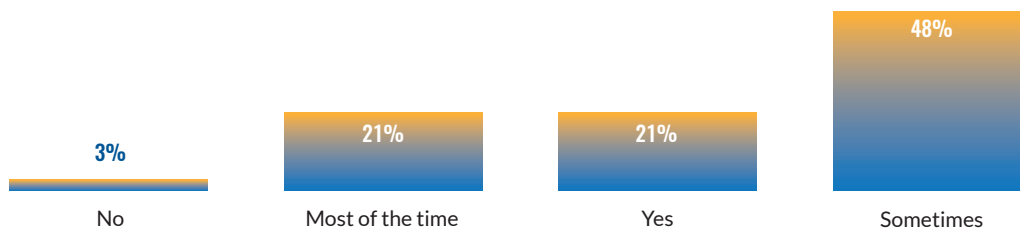
### What kind of advocacy activities does your organisation conduct? (n=29b)



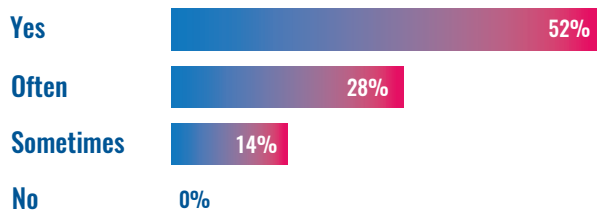
**With what institutions do you have regular contact? (n=29)**



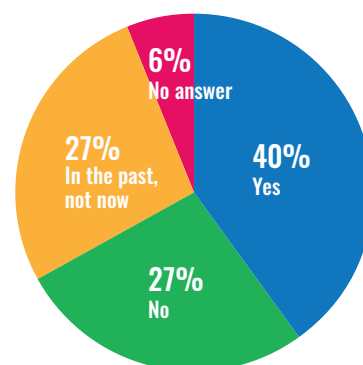
**Is your organisation successful in having its views included in recommendations/pieces of legislation/debates on the topics you advocate for? (n=29)**



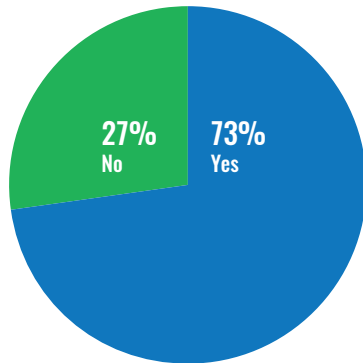
**Do you cooperate with other stakeholders within your advocacy activities? (n=29)**



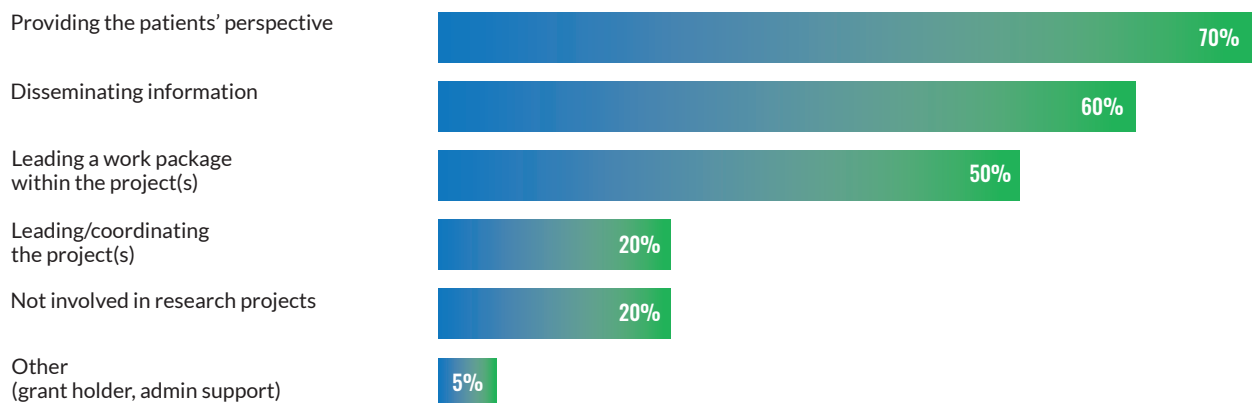
**Are you involved in EU/national research projects? (n=30)**



**Apart from research projects, does your organisation conduct/contribute to further evidence-gathering/data collection activities, such as your own or partnership projects? (n=30)**

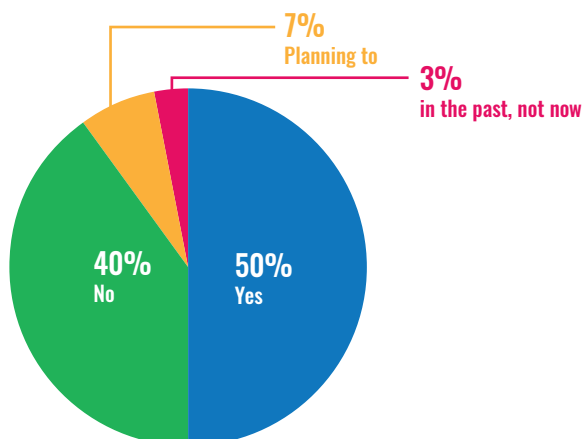


**What is/was your role within these\* projects? (n=20c)**



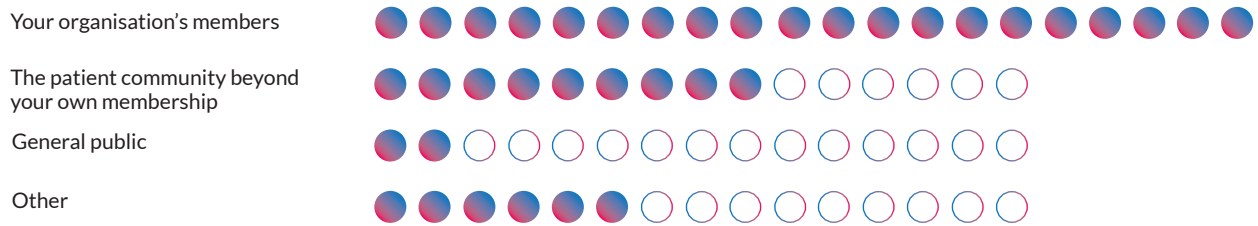
Note: \* by 'these projects' the EU/national research projects were taken into account, not other evidence-gathering activities or partnership projects

**Does your organisation conduct capacity-building activities? (n=30)**

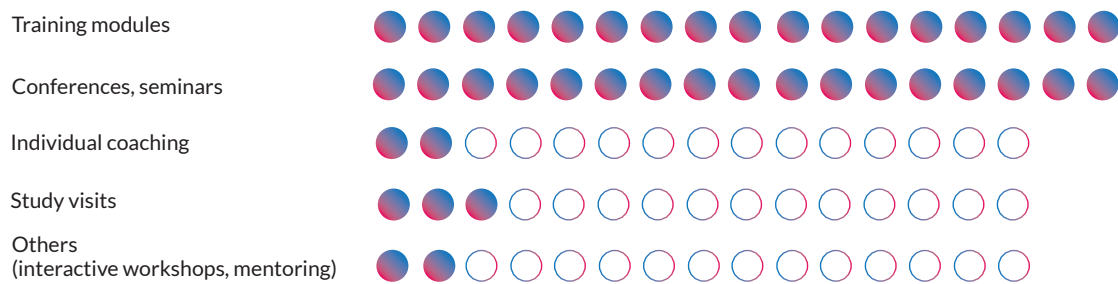




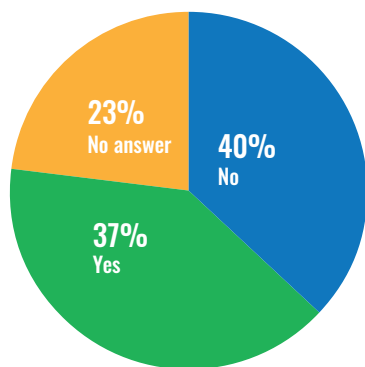
**Who are these capacity-building activities aimed at? (n=15d)**



**What do these capacity-building activities consist of? (n=15)**



**Does your organisation offer support to individual patients? (n=w30)**

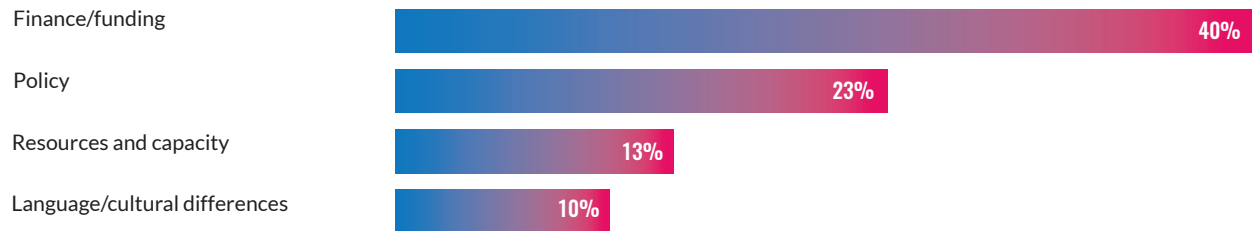


**What kind of support do you offer to individual patients? (n=11e)**



\* Information, dissemination, social media, networking opportunities

### What are the main barriers, if any, for you to be as inclusive and representative as possible? (n=30)



### If your organisation could not fulfil its role, what would be the consequences? (n=30)



## Legend

- "a" total number of respondents;
- "b" number of respondents who answered positive to question on 'conducting advocacy activities';
- "c" number of respondents who answered positive to question on 'conducting EU/national research projects';
- "d" number of respondents who answered positive to question on 'conducting capacity-building activities';
- "e" number of respondents who answered positive to question on 'offering support to individual patients';

## Respondents to the survey

EPF would also like to acknowledge and thank warmly its members that responded to the survey and therefore contributed to this report.

### AGORA

Alzheimer Europe

Association for the Protection of Patients' Rights in Slovakia (AOPP)

Dystonia Europe

Europa Donna

EuropaColon

European Alliance of Neuromuscular Disorders Associations (EAMDA)

European Cleft Organisation (ECO)

European Federation of Allergy and Airways Diseases Patients Associations (EFA)

European Federation of Homeopathic Patients' Associations (EFHPA)

European Federation of Neurological Associations [EFNA]

European Haemophilia Consortium (EHC)

European Headache Alliance (EHA)

European Institute of Women's Health (EIWH)

European Multiple Sclerosis Platform (EMSP)

European Network of (Ex-)Users and Survivors of Psychiatry (ENUSP)

European Parkinson's Disease Association (EPDA)

European Patients' Forum (EPF)

EUROPSO - European Umbrella Organisation for Psoriasis Movements

GAMIAN-Europe - Global Alliance of Mental Illness Advocacy Networks Europe

Hungarian Alliance of Patient Organisations (BEMOSZ)

International Bureau of Epilepsy (IBE)

International Federation for Spina Bifida and Hydrocephalus (IFsBH)

Lupus Europe

National Confederation of Disabled People (Greece)

National Patients' Organisation of Bulgaria (NPO)

Panyprian Federation of Patients' Association and Friends

Retina International

The Latvian Umbrella Body for Disability Organizations (SUSTENTO)

Vlaams Patiëntenplatform – (The Flemish Patient Platform)



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