PATIENTS CALL FOR MEANINGFUL INVOLVEMENT IN EUROPEAN COOPERATION ON HTA

Patients contribute to high quality scientific health technology assessment

Patients and their organisations welcome efforts set out in the EU proposal for a new legislation on health technology assessment (HTA) to adopt high quality methods and standards when assessing health technologies they use for their own treatment.

However, patients and their organisations are convinced that changes need to be made to the legislation before it is adopted to guarantee an **adequate involvement of patients in all HTA activities (joint consultations, early dialogues, scoping and assessments), which is essential to help HTA assessors determine the relative efficacy and safety of health technologies.**

1. A MATTER OF QUALITY

Via a direct dialogue with patients, HTA assessors can better understand what important questions need to be answered: for complex diseases in particular, **assessors need to hear from patients which relevant symptoms need to be improved and what impact they expect from the technology on their daily life. To fully interpret results of clinical trials and assess whether they are clinically meaningful, patients who are the end-user of the technology need to provide their views.** Failure to consult patients adequately on this impact reduces the assessors' ability to determine the therapeutic value of the technology.

The contribution of patients enables HTA bodies and experts to carry out a higher quality of work. In a domain where high uncertainties on the clinical benefit of new or older technologies exist, only direct discussions with clinicians and patients can clarify the incertitude.

2. ADEQUATE INVOLVEMENT

In contrast with the approach adopted by the European Parliament on that point, patients and their organisations consider that **submitting comments in writing on complex documents with information of varying importance after all discussions have taken place, is not an adequate involvement**. HTA relies on collective thinking, where different experts discuss different information, confront different interpretations, simultaneously and according to the same methods.

By 'adequate involvement', we mean participation in the discussions that take place prior to and while a medicine or technology is going through the HTA assessment, for example face-to-face meetings (joint consultations), focus groups with assessors for the scoping phase, telephone interviews with assessors, patients' preferences elicitations techniques, and expert or citizen panels.

EUnetHTA, the European Network of HTA Agencies, is currently piloting various methods to adequately involve patients in HTA. These methods vary in their modalities, costs, and the information they might provide.

More research is needed to determine which methods to use to involve patients in the European Cooperation on HTA.

At the national level, more HTA agencies are using different methods to involve patients:

- G-BA, the German HTA authority, made the participation of patients in HTA a condition for a quality assessment: in accordance with the regulations set forth in the German Social Code, Book Five (SGB V), leading nationwide advocacy groups that represent patient interests are entitled to take part in discussions or submit requests, and their opinion have to be included in the decision.
- In Austria, LBI-HTA organises focus groups with patients. The groups meet for 2 hours with HTA assessors and this proves to be much more efficient that sending comments in writing.
- Other Member States (Ireland, Spain, Sweden, United Kingdom) adopted guidance on patient involvement in HTA.

3. LEGITIMACY

The participation of patients in HTA committees or procedures is key for civil society to witness how HTA is conducted. This is a transparency requirement - patients and citizens have the right to verify that assessments are based on science and medical evidence.

Of all interested parties, patients have the most at risk: our health is at stake. Engaging patients in the Coordination Group and in the scientific subgroups of the European HTA Cooperation is **a necessary measure to enhance mutual trust.**

Patient involvement ensures that the dignity and legitimacy of people living with a disease is recognised. **Patients represented by the below groups are calling to participate in all forums where decisions that affect their lives are made, with equal credibility as other experts.**

The role of patients is clearly stated in most EU legislations on pharmaceuticals. At the European Medicines Agency (EMA), the European legislator decided patients are members with full rights in the Committee for Orphan Medicinal Products (*Regulation EC 141/2000 Art.4*), in the Paediatric Committee (*Regulation EC 1901/2006 Art.4*), in the Committee for Advanced Therapies (*Regulation EC 1394/2007 Art.21*), in the Pharmacovigilance Committee (*Regulation (EU) No 1235/2010 art.61*), and in the Management Board (*Regulation EC 726/2004 Art.65*).

To our opinion, *Recital 18 of Regulation EC 726/2004* is valid for all EU health agencies and institutions:

> *The structure and operation of the various bodies making up the Agency should be designed in such a way as to take into account the need constantly to renew scientific expertise, the need for cooperation between Community and national bodies, the need for adequate involvement of civil society, and the future enlargement of the European Union. The various bodies of the Agency should establish and develop appropriate contacts with the parties concerned, in particular representatives of patients and health-care professionals.*

Why should patients be full members in regulatory structures, but only be permitted to submit comment in writing in HTA procedures?

4. NOTHING ABOUT US, WITHOUT US

It is not without reason that in 1983, AIDS advocates formulated the principles of Treatment Advocacy, which applies to all patient advocacy movements, whichever their type, status, or history, as follow:

- Be involved at every level of decision-making; and
- Be included in all forums with equal credibility as other participants, to share their own experiences and knowledge.

These principles are used to be summarised in the motto *"Nothing about us, without us"*.

At present, patients have their word to say on all aspects that affect the development and the evaluation of treatments, by:

- Being members of ethics committees for clinical trials;
- Working with industry to improve the quality of clinical trials, discussing the design, the doses, the outcomes, and the protocol in all its aspects;
- Being full members of scientific committees at the European Medicines Agency;
- Being involved at national level by HTA agencies in the most modern Member States, from the selection and the assessment of evidence to the appraisal; and
- Contributing in pharmacovigilance by reporting on side effects and testifying about them.

The below signatories call on European Institutions to adopt appropriate measures to fully incorporate the meaningful involvement of patients in the European Cooperation on HTA. The European Cooperation should enact its own rules on how to work with different parties, adopt necessary policies to regulate potential conflicts of interests (financial, intellectual, and participatory). There should not be an a priori exclusion of a key actor for HTA: the patient.

This Statement has been co-signed by the following organisations:

AE - Alzheimer Europe

Alzheimer Europe is the umbrella organisation of 42 national Alzheimer's associations from 37 European countries. It aims to provide a voice to people with dementia and their carers, make dementia a European priority, promote a rights-based approach to dementia, support dementia research and strengthen the European dementia movement;

EATG - European AIDS Treatment Group

The European AIDS Treatment Group is a member-led network of 180 activists across Europe and Central Asia seeking to strengthen the voices of people living with or at risk of HIV infection in clinical research, as well as in the design, implementation, and evaluation of policies, laws, and programmes that affect their lives. Members – most of them living with HIV - come from a diverse range of backgrounds, i.e. social workers, researchers, medical doctors, service providers, counselors, community-peer workers.

ECPC - European Cancer Patient Coalition

The European Cancer Patient Coalition (ECPC) is the voice of cancer patients in Europe. With over 400 members, ECPC is Europe's largest umbrella cancer patients' association, covering all 28 EU member states and many other European and non-European countries. ECPC represents patients affected by all types of cancers, from the rarest to the most common.

EFA - European Federation of Allergy and Airways Diseases Patients' Associations

The European Federation of Allergy and Airways Diseases Patients' Associations (EFA) is a non-profit alliance of more than 40 allergy, asthma and chronic obstructive pulmonary disease (COPD) patients' organisations, representing over 400,000 patients in 25 European countries.

EFNA - European Federation of Neurological Associations

The European Federation of Neurological Associations [EFNA] is an umbrella group representing pan-European neurology patient groups. Our slogan 'Empowering Patient Neurology Groups' encapsulates our goals as an Association. We strive to add capacity to our members – allowing them to be the most effective advocates possible in their own disease specific areas. EFNA embraces the concept of Partnership for Progress – working at a high level with relevant stakeholders from the fields of policy, medical, scientific/research, industry, patient partners and other key opinion leaders.

EHN - European Heart Network

The European Heart Network plays a leading role in the prevention and reduction of cardiovascular diseases, in particular heart disease and stroke, through advocacy, networking, capacity-building, patient support, and research so that they are no longer a major cause of premature death and disability throughout Europe.

EMSP - European Multiple Sclerosis Platform

The European Multiple Sclerosis Platform (EMSP) represents more than 750,000 people living with multiple sclerosis (MS) in Europe. Their needs are the main focus of its advocacy and awareness-raising campaigns. EMSP relies on a growing network of 41 member organisations from 36 European countries.

EPF - European Patients' Forum

EPF is an umbrella organisation that works with patients' groups in public health and health advocacy across Europe. Our members represent specific chronic disease groups at EU level or are national coalitions of patients.

EUomo - European Prostate Cancer Coalition

Europa Uomo, the Voice of Men with Prostate Cancer in Europe, represents and supports patient groups with prostate diseases in general and cancer in particular. Our aims include increasing awareness of prostate diseases; the support of individualised treatment based on optimal medical assessment with personalised patient care and patient advocacy as a priority; focused on quality of life based on solidarity and mutual respect. Our development requires collaboration with the professional organisations to provide information on evidence-based treatment, to educate men about holistic patient care and solidarity in advocacy with other patient support groups.

IDF - International Diabetes Federation European Region

IDF Europe is an inclusive and multicultural umbrella organization of 69 national diabetes associations in 44 countries across the European region, representing people living with diabetes and healthcare professionals. Through our activities, we aim to influence policy, increase public awareness and encourage health improvement, as well as promote the exchange of best practice and high-quality information about diabetes throughout the European region.

IPOPI - International Patient Organisation for Primary Immunodeficiences

IPOPI, the International Patient Organisation for Primary Immunodeficiencies, is the Association of national patient organisations dedicated to improving awareness, access to early diagnosis and optimal treatments for primary immunodeficiency (PID) patients worldwide. Established in 1992, IPOPI works as the global advocate for the PID patient community in cooperation with its National Member Organisations (NMOs) and key PID stakeholders.

MPE - Myeloma Patients Europe

Myeloma Patients Europe (MPE) is an umbrella organisation of myeloma and AL amyloidosis patient groups and associations from across Europe. MPE was formed following the merger in 2011 of the European Myeloma Platform and Myeloma Euronet. MPE acts as an umbrella organisation for existing local and national myeloma associations and its members come from nearly 30 different countries.

EGAN - Patients' Network for Medical Research and Health

The Patients Network for Medical Research and Health EGAN is an alliance of both National Genetic Alliances and European disease specific patient organisations with a special interest in genetics, genomics and biotechnology. Especially, but not only, genetic disorders are represented within EGAN. EGAN is working for a voice in research and health policy and seeks a world in which genetic and other serious diseases are understood, effectively treated, prevented and the people affected supported.

EURORDIS - Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 800 rare disease patient organisations from more than 60 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

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