



**ACCESS TO
THERAPEUTIC INNOVATION**



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Executive summary

There are multiple explanations to inequity on access, multiple views as to what constitutes a priority in healthcare, and multiple perceptions of what qualifies as an innovation. Together among this multiplicity of ideas, there is a clear need for an improved approach to enhance patient access to the best innovation that medical technology has to offer. A first step in connecting the dots is the development of a tool that would assist in identifying and prioritising the areas of health where EU collaboration could be appropriate to reduce health inequalities between and within Member States.

The Sub-Committee on Access to Innovative Therapies has developed a "matrix" approach designed to define the level of EU intervention required to enhance the access of innovative therapies across Europe. This tool could be used to assess current and future EU policies or to support the development of an EU budget in the field of health. By assessing the medical unmet need and the access issues, the matrix identifies where national and where European integration on a specific disease area may be most appropriate.

Based on this initial research, the further development of improved access for patients and a reduction of unnecessary barriers are envisaged through a three-step approach, including concrete actions at both EU and Member State level.

- 1. UNDERSTAND: Creation of a European Access Observatory (EAO) to annually assess the uptake of new products and therapeutic alternatives in Member States.*
- 2. MAXIMISE: Leverage valuable EU initiatives to address the existing barriers to access.*
- 3. ANTICIPATE: Development, in collaboration with healthcare stakeholders and policymakers, an EU-level analytical tool to support the prioritisation and decision-making specifically focused on addressing access questions in health policies.*

I. INTRODUCTION

Europe's population of 500 million citizens is ageing rapidly, with the European Commission and the Economic Policy Committee estimating that the demographic burden will increase exponentially over the next three decades. As the working age population is set to decrease by 16%, the elderly population is projected to increase by 77% by 2050 - a change that will require more resources. Job market reform will be needed to ensure sufficient contributions are made into the social protection systems of Member States. Healthcare budgets are under pressure, and high rates of chronic diseases, which swallow 80% of healthcare budgets, demand smarter spending to mitigate the management costs. The sustainability of current approaches is under scrutiny, and solutions are needed if the patients of tomorrow are to benefit from cost-effective innovative therapies, advances in medical technologies, and ways of communicating medical information. Discussions are underway at European and national level about how to create the right environment for viable healthcare systems that incorporate European notions of equity and solidarity, and that can overcome the lack of policy coordination in the EU and with member states, currently resulting in an inefficient use of resources.

Within this broader discussion of Europe's healthcare systems, access to therapeutic innovation is of particular concern. Inequalities between and within Member States have grown in recent years, as is evident from the Euro Health Consumer Index 2014 (Bjornberg, 2015). Recent public health issues, such as the H1N1 pandemic or the launch of innovative but expensive Hepatitis C therapies, have highlighted questions about European collaboration on access. They further serve to demonstrate that Member States face some common challenges, but that their ability to respond effectively to those challenges is influenced by differences in healthcare systems, health policies, and socio-economic conditions.

Europe-level discussions have increasingly been addressing inequalities in access. The role of the EU has been limited to that of a coordinator, facilitator, or idea generator, but with access issues still unresolved, the status quo on health policy is being challenged by some stakeholders keen to promote reflections on whether access to innovative therapies should fall under the influence of the European Union.

There might indeed be a case for greater collaboration at European level in promoting access to therapeutic innovation. However, there are multiple elements contributing to inequity in access, multiple views as to what constitutes a priority in healthcare, and multiple perceptions of what qualifies as an innovation. Access is not a single problem, nor is it one on which stakeholders share a definition; it is a set of distinct problems that require distinct solutions, and clearer identification of where the added-value of European action and policy response might be the most appropriate.

To re-evaluate the role of Europe in improving access to therapeutic innovation, it is necessary to first understand the current hurdles to access and then develop the tools needed to overcome them. The European Health Parliament Sub-Committee on Access to Innovative Therapies will consider these points with the aim of:

- Developing a tool allowing for the categorisation of health and access issues according to some pre-defined criteria; and
- Providing a set of recommendations based on our findings.

II. SITUATIONAL ANALYSIS

The health policy landscape is evolving and an increasing number of initiatives at the EU level or through joint national initiatives are delivering policy responses in the healthcare sector.

Within the EU-wide policy framework, this ranges from European Commission and European Parliament initiatives to stakeholder coalition responses, which are focused on driving more efficient, effective, and streamlined approaches to access. Yet an important question still remains: Are such initiatives demonstrative of a harmonised integration framework and successful in increasing integration at EU level?

A. Horizon 2020

Horizon 2020 is the largest EU Research and Innovation Programme thus far, with nearly €80 billion available in funding over a period of seven years (2014 to 2020), as well as leveraging some private investments. The programme releases calls within specific areas, including medical technologies and pharmaceuticals (European Commission), with two especially relevant packages currently underway focusing on: "Health, Demographic Change and Well-being" and "Future and Emerging Technologies".

Within this framework the second Innovative Medicines Initiative (IMI2) has a €3.3 billion budget for 2014-2024, roughly half from Horizon 2020 and the remainder from companies that belong to the European Federation of Pharmaceutical Industry Associations and other life science industries and organisations. The supporting companies do not receive any EU funding, but they contribute to specific projects, for example, by donating their researchers' time or providing access to research facilities or resources. The IMI2's annual Work Plan for 2015 includes more information about strategic objectives, specific challenges and funding.

B. European Semester

Despite the limited competencies seceded by Member States to the European Union, the European Semester has since 2011 provided enhanced recommendations as to how national healthcare systems can be reformed in a fiscally efficient manner. While not all countries were originally part of the initiative, each edition has brought additional countries into its scope.

But there is continued reluctance at Member State level to adopt these recommendations, because of concerns over subsidiarity, or over further cuts in the wake of the post-crisis austerity measures. In addition, existing recommendations are overly focused on financial and quantitative targets, with little considerations given to more qualitative targets and long-term reforms (e.g. increasing uptake of generic medicines to enhance access to therapies, as was the case in Ireland). The most recent recommendations from the European Parliament Committee on the Environment, Public Health and Food Safety highlighted the risk that the European Semester was promoting short-term thinking about investment rather than considering health as a long-term investment and a value in its own right (ENVI Committee, 2015).

C. Patient Access Partnership on Equity of Access to Quality Healthcare

The European Patients' Forum and the Bulgarian National Patient Organisation joined forces to invite key stakeholder to initiate the Patient Access Partnership, conceived to reflect on sustainable provision of equitable patient access to quality healthcare in the EU. The objective is facilitating the dialogue between European and national health stakeholder on access, identifying hurdles for patients, assessing gaps within EU Member States, collaborating with EU-led or national initiatives, and developing innovative and sustainable solutions.

The initiative is supporting a European Parliament Interest Group on Access to Healthcare, hosted by MEP Andrey Kovatchev (EPP, BG). This group offers an open platform for exchange, with the vision of transferring the best practices identified by members of the partnership.

D. Health Technology Assessment (HTA)

Initiatives

Collaboration on access has gained greater attention at EU level, but these discussions are not entirely new; they are part of a trend toward a more prominent EU role. The first milestone toward a more unified pharmaceutical market was reached through the regulatory integration that came with the establishment of Centralised Procedures and common EU legislation on pharmaceuticals with a -defined assessment methodology. But differences in national systems, particularly in methodologies and evaluation for reimbursement decisions, constitute additional hurdles in terms of equal access. Discussions on health technology assessment (HTA) and the merits of cooperation emerged, and several projects have been financed through the EU research programme, FP7, which is now Horizon 2020. One of the outcomes is EUnetHTA, and this has been transformed into a “joint action,” originally envisaged to run in two cycles between 2010 and 2015, but now about to be extended into a third cycle. This increased cooperation has led to concrete deliverables for pharmaceuticals including: common HTA methodologies, joint pilots, IT tools, and training facilities. Perhaps more importantly, it helped to create an HTA community across national organisations, which culminated with the set-up of the European HTA Network.

The Health Technology Assessment Network was created on the basis of Directive 2011/24 on the Application of patients’ rights in cross-border healthcare and in October 2014 formally adopted the Strategy for EU Cooperation on HTA as based on input from formally appointed representatives from Member States, plus Norway and Iceland. While the network intends to enhance cooperation between national and European bodies under the auspices of the overarching vision that “evidence is global, decision is local” (HTA Network, 2014), the European Network on HTA (EUnetHTA) still exists and supports the work for HTAN on a technical level through the development of good practice methods, processes, and core models for assessment of various technologies.

HTA collaboration has also been reinforced with initiatives led by the European Medicine Agency (EMA). Parallel scientific advice is one such example, which allows EMA and HTA agencies to be equal partners in a multistakeholder procedure, leading to early-dialogues between the pharmaceutical manufacturer, EMA and HTA bodies. The lack of a joint report was at first considered a procedural weakness, but in practice this offers flexibility and holds out the prospect that the procedure will lead to useful advice for the manufacturer, despite differences between national HTA bodies. In addition, the recent revision of the EU Pharmacovigilance legislation led to the creation of the Post Authorisation Safety Studies and Post Authorisation Efficacy Studies, which further allows for the reassessment of new innovative medicines over their life cycles.

The Innovative Medicines Initiative (IMI) also finances projects relating to HTA and relative effectiveness, for instance with the IMI Get Real projects, contributing to the generation of real world data.

E. Price & reimbursement and access initiatives

Apart from HTA collaborations, discussions on collaboration in the field of pricing and reimbursement (P&R) have also been taking place. The Network of Competent Authorities for Pricing and Reimbursement (CAPR) is a platform between Member States that was set up at the initiative of the Slovenian Presidency. The objective of the network is to identify and address common issues in the field of P&R. Participation in the network is voluntary, and like the EUnetHTA platform, a key added-value of the platform is bringing together national authorities in the field of P&R.

Another initiative, led by the European Commission's DG Enterprise, was the Process of Corporate Social Responsibility in the field of Pharmaceuticals. Divided into several work streams, its outputs included a report on the use of Managed Entry Agreement (MAE) in Europe, as well as on access to biosimilar medicines, featuring a consensus paper – What You Need to Know about Biosimilar Medicinal Products.

Another work stream within the project considered orphan drugs. By way of the Mechanism of Coordinated Access to Orphan Drugs (MOCA), it identified options for collaboration, including horizon scanning, early dialogue, possible collective value assessment or HTA, and joint pricing or procurement. Perhaps the most tangible output was the Transparent Value Framework – a multi-criteria decision analysis tool designed to assess the value of an orphan drug while accounting for its specificities, with the aim of facilitating pricing decisions.

F. Access to Innovation in Health and Social Care

The European Parliament Interest Group on Access to Innovation in Health and Social Care is an initiative that began for the 2014-2019 term with the aim of bringing together Members of the European Parliament, as well as other healthcare stakeholders, to improve patient access to innovation in health and social care. The group was launched in May 2015, intends to develop policies to make public health a priority in the European Parliament, prioritising innovation and ensuring that patient-centricity is central to healthcare systems and the provisions of solutions.

G. Universal Access to Health roundtable

A year after the EU elections, the European Patients Forum, the European Generic and Biosimilar Medicines Association, the International Association of Mutual Benefit Societies, and Doctors of the World held a roundtable on Universal Access to Health, as a follow-up to the dialogue initiated a year earlier. Through this platform, MEP Karin Kadenbach (S&D, AT), Boleslaw Piecha (ECR, PL) Alojz Peterle (EPP, SL), as well as healthcare stakeholders, addressed the ways in which universal access to healthcare across the EU can be ensured. A consensus among the group was that beyond open dialogue, it is essential to ensure tangible and concrete follow-up actions to harness this partnership in tackling access to health.

H. European Patients Academy on Therapeutic Innovation (EUPATI)

Initiated under the first IMI, EUPATI is a five-year project running until 2017, developing education material, training courses, and a public online library as a means of educating patients and the lay public on sustainable infrastructure and up-to-date information on therapeutic innovation in a scientifically objective and reliable way. Ultimately, EUPATI aims at empowering patients, placing them at the heart of healthcare systems as well-informed and trained patient advocates. Most recently, EUPATI published best practice guidance on how to engage patients. It has also published an online e-Learning tool, and it organises webinars and works with medicines agencies on considering the role of patients in R&D.

III. SOLUTIONS

Background and objectives

European integration and collaboration in the context of access to innovative therapies may be the answer to calls for improved equality across Member States, but this would require a drastic redistribution of competences in the field of health. Even if applied in limited instances on very specific areas of health, a shift from complete national – or regional – authority over disease management to a system in which the European institutions would have a bigger role is remote from today's reality. Yet from the perspective of the European Health Parliament Sub-committee on Access to Innovative Therapies and as evidenced through our qualitative and quantitative research, there is demand among European policymakers and, more indicatively, European citizens, for a tool that would assist in identifying and prioritising those areas where EU collaboration could be appropriate and could support national healthcare systems.

For this report, the Sub-Committee has developed a Multi-Criteria Decision Analysis (MCDA) tool. As a concept, it is not new, but thanks to renewed attention, it has been rejuvenated in the form of the Mechanism of Coordinated Access to Orphan Drugs (MOCA). This led to the creation of the Transparent Value Framework, a multi-criteria tool to assess the value of an orphan drug. While MOCA is a European initiative, Member States have also considered similar approaches. For instance, in Belgium, the Federaal Kenniscentrum – Centre federal d'expertise (KCE) published a report on Incorporating societal preferences in reimbursement decisions – Relative importance of decision criteria according to Belgian citizens (Cleemput I, 2014). This study aimed at measuring public preference weights for reimbursement based on two deliverables, (i) an extensive literature review on societal preferences, (ii) a study amongst the general population.

The Sub-Committee has considered these two approaches, not to develop a MCDA model designed to assess the value/reimbursement of a pharmaceutical product, but to assess the non-product specific value of an EU intervention.

From our perspective, two elements would justify an EU priority:

- The existence of an unmet medical need; and
- The added-value of a European intervention (as opposed to national intervention).

$$P \text{ (Priority for EU)} = U_{\text{nmet}} M_{\text{edical}} N_{\text{eed}} \times A_{\text{dded}} V_{\text{alue}}$$

Such a tool could be used for several purposes:

- Assessment of current (and future) EU policies;
- Establishment of a list of priority health areas at the EU level;
- Creation of a special EU Fund to reduce health inequalities, in which:
 - > The allocation of the budget could be defined according to areas that should be a priority and where EU support is critical; and
 - > The fund could be revised on a regular basis (i.e. every 5 years).
- Support for the development/allocation of an EU budget in the field of health (in addition to appropriate expert and stakeholders consultations); and
- Identification of those disease areas where EU-level solutions to access should be considered.

Methodology

A three step approach has been applied:



The Sub-Committee also adopted a set of criteria to define both the unmet need and the added value of a possible EU intervention. The criteria selected for determining unmet medical need are largely inspired by the studies mentioned above, including the Transparent Value Framework and the KCE report.

The following criteria in terms of unmet need were identified:

- Prevalence;
- Cause of mortality;
- Five-year survival rate;
- Pain caused to the patients;
- Disability caused to the patients;
- Burden caused to the healthcare system; and
- No treatment available

The criteria for assessing the “added value” of EU intervention were based on a literature review of existing barriers to access to new therapeutic options. These criteria gather a broad range of potential barriers ranging from scientific and research challenges to economic hurdles (budgetary constraints), and including other relevant dimensions such as information of patients and physicians, as well as differences in Member States P&R decision-making processes. As mentioned in the introduction, there are many potential hurdles to access, and identifying an exhaustive and justified list is a difficult exercise in itself.

The following criteria were identified as possible barriers of access:

- Lack of scientific knowledge for the disease;
- Complexity, risk, and cost of the research;
- Limited economic appeal of investing in developing a new treatment option;
- Health budget limitations;
- Health budget limitations in the short term (“cash-flow problem”);
- Uncertainty regarding the actual expenses the product will have in real-life;
- Budgets prioritized to other disease areas / treatment options (“National priority”);
- No clear value of the treatment in terms of its price/costs (“cost-efficiency uncertainty”); and
- No clear value of the new treatment in real life (“real-life clinical uncertainty”).

Unmet Need score \ Acces Issue score	1	2	3	4	5	6	7	8	9	10
1		National policies								
2										
3										
4				Member states collaboration initiatives						
5										
6						E.U. collaboration/solidarity				
7										
8							E.U. legal Intervention			
9										
10									Total E.U. Integration	

Table 1: Theoretical Matrix

A. Weight of criteria

To make full use of the matrix and obtain a score for each disease area to help prioritise between disease areas and access issues, it is essential to estimate the relative importance of each criteria. For that purpose, the Sub-Committee has developed a standard questionnaire in which participants were requested to rank each of the criteria.

This questionnaire was shared with the members of the European Health Parliament and students from the College of Europe (see questionnaire in Annex I). The choice of this population was made on the basis of gathering the views of individuals from different Member States. However, there are some limitations as the engaged population primarily consisted of young, highly-educated Europeans with a reasonable level of knowledge and/or interest in health policies, and perhaps a favourable bias toward European integration.

As mentioned previously, enhanced integration at European level in the field of health and access is a leap forward in terms of general integration. Even in the absence of further integration, ensuring that current EU funds and efforts are allocated in the most efficient manner in light of what EU citizens consider a priority is of utmost importance in a democratic Europe. From that perspective, the tool could be used within current EU policies, with a view to prioritise EU funds and policies.

To supplement the information provided, the methodology adopted involved a third step: expert reviews, conducted through qualitative interviews. This allows the Sub-Committee to correct some of the biases that may arise.

The questionnaire was sent in March 2015, tallying a total of 71 respondents by April 2015. The matrix will be revised and updated based on new data cut-offs. The qualitative interviews were carried out during the same period and engaged 7 Members of the European Parliament and health experts on the European level.

B. Results from the Access Survey

Based on the breakdown of the criteria in the questionnaire, which is detailed in Annex I, the following weight was assigned for the evaluation:

- Prevalence (20%)
- Cause of Mortality (18%)
- 5 years survival rate (14%)
- Pain caused to the patients (14%)
- Disability caused to the patients (12%)
- Burden caused to the healthcare system (11%)
- There is no treatment available (11%)

The respondents considered the criteria that should be taken into account to determine if a European intervention would be needed, exploring three situations:

- When a suitable treatment does not exist;
- A suitable therapeutic option exists, but it is not financed by some healthcare systems; and
- There is a suitable treatment option, financed by healthcare system but patients have no access to it.

Each of these situations involved different causes of the access problem. The respondents were asked to evaluate separately the value of a European intervention in each case. The similar results obtained in all cases suggest a bias in the responses, indicating a middle-high range of the scale. Thus while the questionnaire results suggest little differences between the situations, some conclusions or tendencies can still be identified.

The results suggest that in instances when there is no existing treatment, a European intervention would be of more value. When there is a suitable therapeutic option, the national budget limitations, lack of infrastructure, and lack of knowledge among physicians and patients were considered to be situations in which a European intervention should be considered.

Furthermore, the lack of suitable treatment was attributed to the complexity, risk and cost of research or limited economic appeal of investing in developing a new treatment option. A smaller subset of the respondents thought that the lack of scientific knowledge on the disease was responsible. On the other hand, when a suitable therapeutic option exists but is not financed by some healthcare systems, respondents justified this according to:

- Health budget limitations;
- Health budget limitations in the short-term (“cash-flow problem”);
- Uncertainty regarding the actual expenses the product will have in real-life;
- Budget is prioritized to other disease areas / treatment options (“Nationality priority”);
- There is no clear value of the treatment, regarding its price/costs (“cost-efficiency uncertainty”); and
- There is no clear value of the new treatment in real life (“real-life clinical uncertainty”).

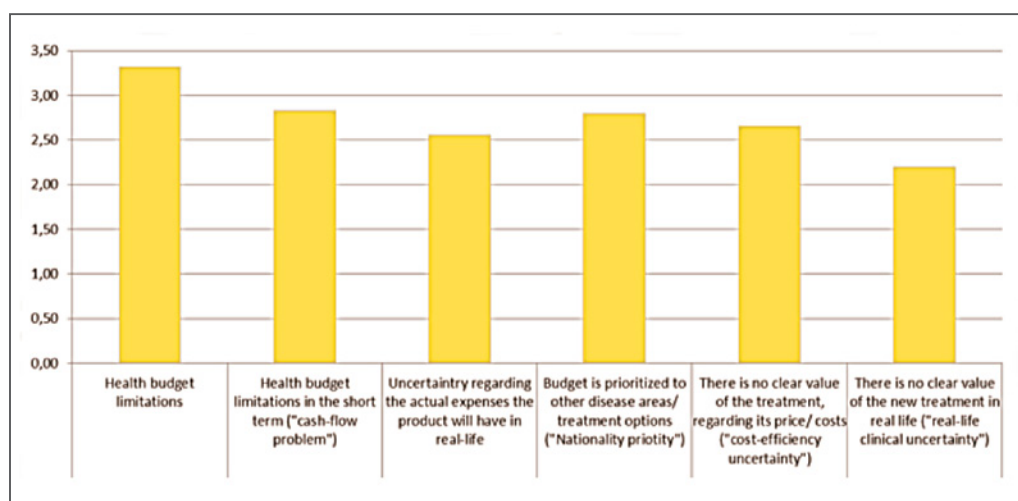


Table 2: A suitable therapeutic option exists, but is not financed by all healthcare systems

When there is a suitable treatment option that is financed by healthcare system, but patients still do not have, respondents indicated that the following criteria is responsible:

- a. Lack of appropriate/enough infrastructures, leading to inefficiencies and poor patient management
- b. Under-diagnosis of the patients (medical education issue)
- c. Long waiting time before patients can actually see a doctor
- d. Patients have to undergo several tests / alternative therapeutic options before being eligible for the therapeutic innovation (restriction in reimbursement criteria)
- e. Physician prefers existing therapeutic options for clinical reasons
- f. Physician prefers existing therapeutic options for cost reasons
- g. Patient or physician is not aware of the new treatment option

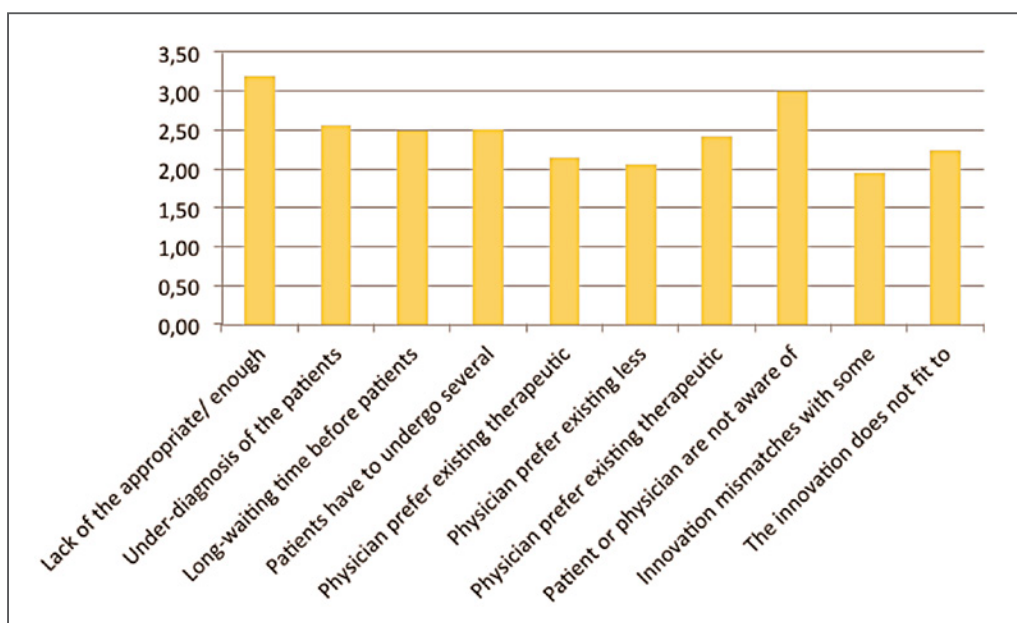


Table 3: A suitable treatment option exists and is financed, but patients still do not have access.

The results also show that when the problem is linked with the organisation of the health system, a European intervention would be valuable, especially when there is a lack of information among the physicians and when the infrastructures are inappropriate. Likewise, the responses suggest that a European intervention would be more valuable when the problem is linked to health budgets. To combat this problem, the majority of respondents were positive about the creation of a European fund to reduce health inequalities between European countries, which would predominantly be focused on the better organisation of infrastructure of the health system.

Considering the results from the survey, each sub-item of the priority equation should integrate a weight on a scale of 10, with a binary evaluation score of 0 or 1 for each criterion.

Some predefined cut-offs were defined to select disease area priorities and assess unmet medical need. The disease will receive a score of 1 if some characteristics are met. There is an important limitation in this method, as deciding only one cut-off point is too simple taking into account the wide range of situations and characteristics among all diseases. For the purpose of this paper the method was simplified as follows:

Prevalence: Affects more than 0.1% of the population.

Cause of mortality: Disease causes more than 50.000 deaths per year across Europe.

5-year survival rate: Less than 75% of patients are alive five years after their diagnosis.

Pain: More than 50% of patients suffer from moderate to severe pain, according to a Brief Pain Index (BPI) equal or more than 3.

Disability: Disease induces more than one million disability-adjusted life years (DALYs) – the sum of life years lost due to premature mortality and years lived with disability, as adjusted for severity - across the EU Member States.

Economic burden (healthcare cost + indirect cost): Total cost per patient per year is higher than 43.000€.

This objective and simple assessment will allow the matrix to qualify the urgency related to the unmet medical need and provide the first key item of the priority score (Annex II). In the same binary assessment, a review of each access issue criteria should be done to define the access issue score and complete the matrix below:

ACCESS ISSUE REVIEW			
Criteria	Eval. (Yes=1; No=0)	Weight	Score
Lack of scienceknowledge for the disease		0,63	
Complexity, risk and the cost of the research		0,72	
Limited economic appeal of investing in R&D		0,68	
Health budget limitations		0,64	
Cash-flow problem issue		0,56	
Uncertainty regarding expenses in real-life		0,49	
Budget is prioritized to other disease areas		0,54	
Cost-efficiency uncertainty		0,50	
Real-life clinical efficacy uncertainty		0,41	
Inappropriate infrastructures		0,63	
restriction criteria before being eligible to the innovation		0,49	
Time to physician acces		0,49	
Under-diagnosis of the patients		0,50	
Physician prefer existing therapeutic options for clinical reasons		0,40	
Physician prefer existing therapeutic cost reasons		0,41	
Patient or physician are not aware of the new treatment option		0,59	
Physician prefer existing therapeutic options for vested interest		0,48	
Innovation mismatches with some cultural concerns (eg. Religion)		0,39	
Innovation does not fit to country specific atypical situation		0,46	
Acces Issue score			

C. Validation of matrix by experts

Following the expert interviews conducted by the Sub-Committee, the responses were analysed based on overarching themes in terms of barriers and possible opportunities. While the stakeholders interviewed had a variety of backgrounds – representing industry, patients, Members of the European Parliament, healthcare professionals, and academia – they consistently perceived access as a long-term investment that cannot be stifled by short-term budget cuts and austerity measures. Furthermore, the experts agreed that the varieties that exist within the EU, and even within Member States, make it inherently difficult to agree on a unified approach to access. More importantly, while fragmentation between Member States poses significant problems, the on-the-ground result for patients is a significant concern when one country's citizens have access to a therapy that their neighbours across the border may not have at their disposal.

Patients have a right to access healthcare at all stages – from preventive care to current treatments to innovative therapies. However, they must also be informed at all stages to ensure that the decisions they are making are well-informed and the best option for them. Where therapies are under development, healthcare professionals and patients both reinforce the message that research and clinical trials must be conducted in such a way that patients are self-determined and involved in the process.

In line with the results of the quantitative survey, the experts identified key areas of EU cooperation as possibilities for future development. Cross-border collaboration on pricing and reimbursement, for instance, was a common theme addressed by several of the stakeholders. Investment in infrastructure, as indicated by the qualitative survey's emphasis for the allocation of a European health fund, was a reoccurring theme within the scope of the expert interviews. Integrated healthcare systems with smart spending were perceived as the foremost way of moving forward, albeit with the experts acknowledging that an access policy cannot be dictated by the EU, but requires a top-down and a bottom-up approach to be truly successful.

D. Application – two case studies

The "matrix" approach aims at defining the level of EU intervention required to enhance the access of innovative therapies across Europe. By assessing the medical unmet need and the access issues, the matrix identifies where national and where European integration on a specific disease area may be most appropriate. Through the following two case studies – on Hepatitis C and diabetes – the Sub-Committee has assessed the relevance of the approach and highlighted its strengths and weaknesses.

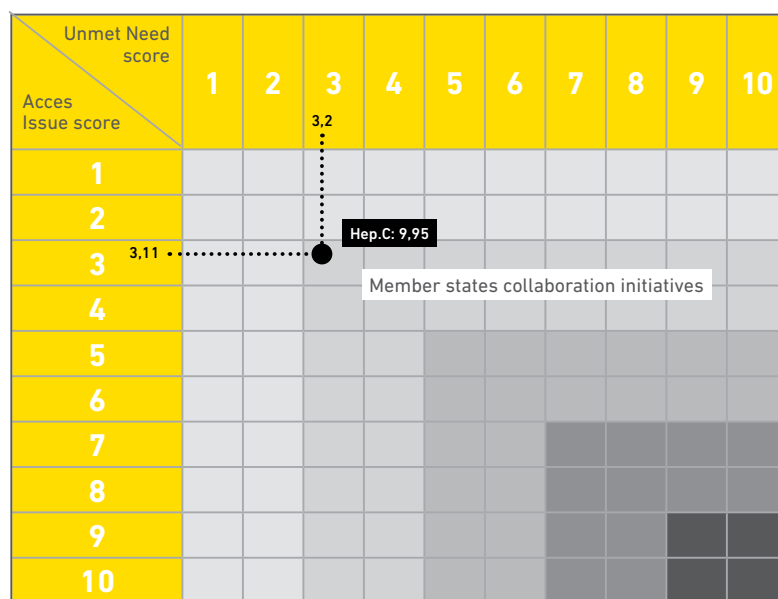
HEPATITIS C

The Hepatitis C (HVC) landscape has radically changed over the past five years. As is the case with numerous infectious diseases, prevalence and progression is associated with poverty and a difficult socio-economic situation, which in turn leads to significant discrepancies between EU Member States. Nevertheless, new innovative therapies have demonstrated a strong ability to change the future of the disease, including the possibility of eradication, but several access issues and on the ground patient access are not resolved across the EU. The Sub-Committee aims to examine whether the matrix is a relevant tool to identify appropriate policies to sustain access of HVC drugs in 2015.

By integrating the evaluation results and considering criteria weight for unmet need, the unmet need is evaluated at 3.2 (Table 3).

Unmet Medical Need review - Hep. C in 2015		
Criteria	Eval. (Yes=1 ; No=0)	Criteria Weight
Prevalence	1	2,0
Death/y	0	1,8
5 y / survival rate	0	1,4
Pain	0	1,4
Disability	1	1,2
Economic burden	0	1,1
Therapeutic alternative	0	1,1
Unmet medical Need score	3,2	

Unmet medical need review would suggest that Hepatitis C medical need in 2015 is low to moderate.



In the Hepatitis C case study, the disease has been considered as a uniform disease, whereas the long duration of the disease and the different phases a patient has to endure could justify it being considered as different medical needs. The disease progresses slowly over years in most patients, from a state in which the liver does not show fibrosis (F0) through different fibrosis grades (F1-F4), which can lead to cirrhosis or liver cancer. The urgency of need to treat for each patient is different as pain, disability and mortality increase through the phases.

If this assessment had been made taking into account the most severe patients F3-F4, the results might have been different as the unmet need would have been higher and the score would also have increased with more probably 1 scores in pain, disability, mortality and economic burden. If the assessment is made for a disease with such heterogeneous patient groups, the tool would be of more value used separately for each patient group or disease phase.

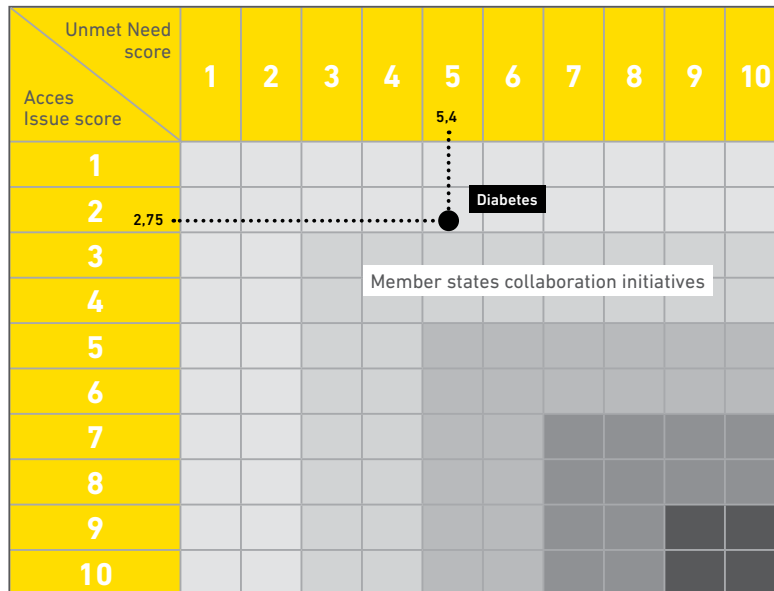
DIABETES

While type 2 diabetes is a condition that has been part of the healthcare landscape for a long time, the significant rise in prevalence and incidence in recent years has added a new dimension and placed a significant strain on healthcare budgets. Yet despite more and more cases arising every year, the policy framework on diabetes is not changing as rapidly as its landscape, nor are patients gaining access to innovation that would help them to best manage their disease. Often, this is the result of under-diagnosis, but other factors – such as national policies, prioritisation and prevention initiatives – also play a role. Is there a role for the EU to play in this environment?

Though national statistics vary, diabetes is an EU-wide problem, one that has a broad impact on healthcare systems. The nature of diabetes also implies that the appropriate care for diabetes patients is widespread, encompassing lifestyle aspects alongside continued treatment, management, and monitoring. As such it has a profound impact on healthcare systems and requires an integrated approach, particularly considering that diabetes is strongly linked to other complications and comorbidities, including cardiovascular disease – the leading cause of death in Europe.

Research into the condition is high on the agenda, with national projects as well as EU initiatives, such as those under the scope of Horizon 2020, considering diabetes care. Nevertheless, innovation in the field is not as rapid as it could be, particularly when considering aspects like interoperability of devices, which would help patients to better monitor their insulin intake and blood glucose levels in an easier way.

According to the matrix assessment characterising unmet need and access surrounding diabetes care, the condition received a score indicating ad hoc collaboration between Member States and at the EU level.



DISCUSSION

Another limitation of the matrix is that health systems across the EU are different and linked to a wide range of problems that lead to reductions in access to treatments. The different causes of the access problems can vary among member states, making more difficult the assessment in the second dimension of the matrix.

When determining the causes of the problems, another limitation is that in most cases the cause is not 100% clear. For example some stakeholders would state that there is no health budget limitation but that the value of the treatment is not clear among the physicians, whereas physicians would state that budget cuts are limiting their freedom in prescribing treatments. When assessing this dimension, it would be necessary to make a deep analysis of the real cause of the problem, not only to determine if a European intervention is needed, but also to ensure that the type of intervention will solve or palliate the problem.

One of the challenges of this type of approach is to balance simplicity and complexity. If the tool is too simple the results could lead to a mistaken action, but if the tool is too complex and the analysis is too deep the tool would not be used, and would be only a theoretical approach, with no practical implementation.

Despite the limitations of the tool, the aim of this paper is to open a workflow in the EU that addresses unmet needs related to different diseases and ensure that a European intervention would take place if the value is demonstrated.

IV. CONCLUSIONS

The health environment in the European Union is uncertain. Patients have no guarantees that a therapy proven to be effective and approved for market access will actually reach them at the local level. Initiatives undertaken thus far – across borders, nationally and by the European Union – are making strides forward, but have ultimately not succeeded in overcoming all the existing barriers. What is evidenced by these efforts, however, is that the missing link seems to be a comprehensive strategy that allows for governments, both national and EU, to have a clear view of where their input would have the greatest positive impact for patients.

A significant point of concern in such strategic discussions is the question of competencies and where the EU influence begins and ends in relation to Member States. To address this point specifically, creating a scientific assessment to analyse where cooperation is of benefit and where action locally is most efficient, the EHP matrix tool emphasises that an evaluation is possible and could very well help to inform decision makers at all levels. The experts engaged in the interview process also expressed hope that full advantage would be taken of existing initiatives and legislation, where it is appropriate and presents opportunities for patients and the sustainability of healthcare systems.

With that in mind, the EHP Sub-Committee on Access to Innovative Therapies proposes concrete recommendations that have arisen throughout research, outreach, and review with experts to ensure access becomes more than a discussion point for Europe.

Top recommendations

The EHP Sub-Committee on Access to Innovative Therapies encourages awareness-building through developing knowledge and understanding at the European level of the existing barriers to access in Europe as they appear in national healthcare system environments and in disease areas. This is envisaged through a three-step approach, including concrete actions at both EU and Member State level.

1. Create a European Access Observatory (EAO) to annually assess the infiltration of new products and therapeutic alternatives in Member States. The EAO would:

- Not be a fundamentally new structure, but rather an entity leveraging existing networks;
- Be responsible for defining a common definition and methodology to assess access;
- Carry out an annual access survey for Member States to respond to.

The annual results should be the object of a multi-stakeholder discussion engaging patients, pharmaceutical and medical technology industry representatives, policymakers, public health authorities, and regulatory bodies, as relevant. Such a meeting would be coordinated by the European Commission.

The results of the annual report and subsequent discussions would be public information in an effort to promote transparency, as well as to encourage responsible uptake, investment and accountability.

2. Maximise the value of current EU initiatives to address the existing barriers to access, such as:

- Promote consistency and cross-fertilisation across all on-going discussions at EU level pertaining to access, including open dialogue and mutual leveraging of outcomes and proposals;
- Enhance stakeholder engagement and involvement in all initiatives, in particular those led by the Council, to encourage the implementation of achievable recommendations and monitoring progress;
- Consider the best methods for appropriate follow up on the Corporate Social Responsibility Process initiated by Directorate-General for Internal Market, Industry, Entrepreneurship and SMEs (formally DG Enterprise and Industry);
- Incorporate concrete qualitative (compliance, adherence, health and socio-economic outcomes) and quantitative (increased access) recommendations regarding access to cost-effective innovative therapies as part of the European Semester.

3. Develop, in collaboration with healthcare stakeholders and policymakers, an EU-level analytical tool to support the prioritisation and decision-making specifically focused on addressing access questions in health policies by:

- Conducting a European-wide study on societal preferences regarding unmet medical needs and access to innovation under the auspices of the EU Health Programme 2014-2020 or Horizon 2020;
- Financing academic research dedicated to the development of a “matrix” or similar tool that would serve to define and measure unmet medical need in a consistent manner across Europe; and
- Engagement of all relevant stakeholders in the discussions.

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DISCLAIMER

The views and opinion expressed in this article reflect the perspective of the European Health Parliament Committee on Acces to Therapeutic Innovation collectively. It does not reflect the views of the individual members in the committee, nor the views of their respective employers or other organizations they may be affiliated with.

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