A3 ADVOCACY: ACCESS IN ACTION

PRACTICAL EXPERIENCES AND GUIDANCE FROM ACCESS ADVOCATES

A RESOURCE DIRECTORY OF INDEPENDENT TOOLS

TOP TIPS FROM THOSE ON THE FRONT-LINE OF ACCESS ADVOCACY

Global Oncology Advocacy Leaders

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ACCESS ADVOCACY
WHAT IS IT?

Advocates across the world are working with those who make decisions about healthcare to ensure that the patients they serve have access to appropriate care.

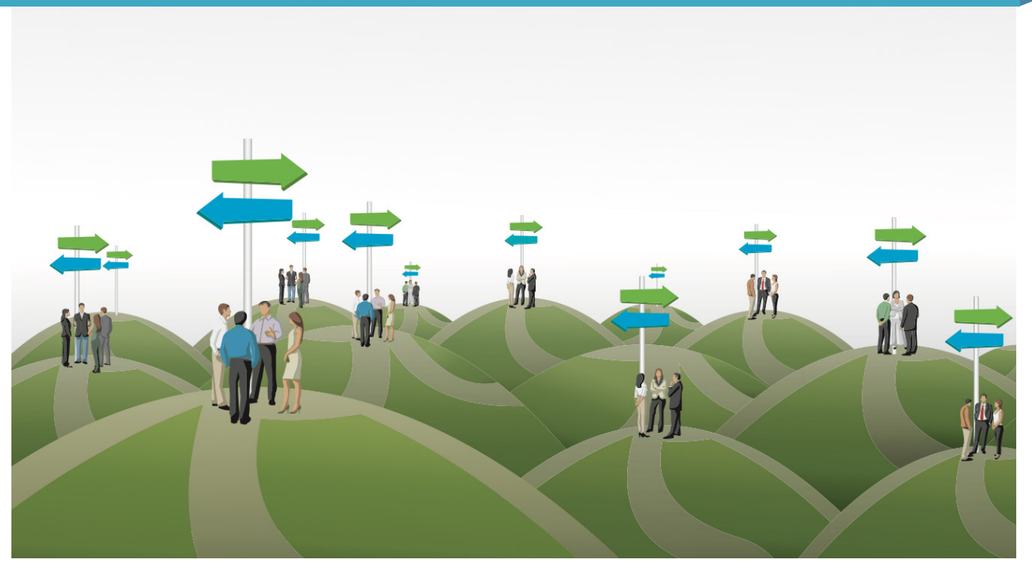
Access advocacy has many faces, and there is not one 'gold-standard' approach to it. The fundamental aim of access advocacy is to work with healthcare systems and those who make decisions to ensure that patients have access to a level of care, treatment and management that improves their condition.

Different healthcare systems have different approaches to granting access to care, and each country or local area will have its own challenges and resource needs.

So, in one country, access advocates may work with a decision-making body such as a Health Technology Assessment (HTA) agency to ensure that the patient experiences are included in the body of evidence that this agency reviews. In another country, there may be no HTA agency, and the advocates may be working with health policy makers to ensure that a particular condition has the appropriate resources devoted to it.

In countries with a lack of healthcare resources, access advocacy may take the form of working within broad coalitions of healthcare stakeholders to ensure that basic diagnostic tests are provided and minimum guidelines to care are adopted. There is no right and wrong, but there are some fundamental principles behind successful access advocacy, including:

- Partnering with decision makers
- Opening dialogues with policy makers
- Understanding the difficulty of making healthcare decisions
- Building networks of members, supporters and other advocates who can work together on the issues



ACCESS ADVOCACY
WHY NOW?

Patient advocates have been working with decision makers for many years. They have built up a wealth of experience in what works and what does not in a variety of access situations.

In this booklet, you will read about the experiences of access advocates in a variety of healthcare systems. There is a single thread that runs through all of these examples: Bringing the patient experience and perspective to decision makers improves the quality of access decisions.

Only people living with a condition can truly describe the day to day challenges they face. These experiences cannot be captured completely in a clinical trial and clinical data cannot fully reflect the priorities that patients put on different aspects of their health and wellbeing.

This has started to be recognized by those that make healthcare access decisions. Formal and informal processes have been developed that allow patient advocates to submit their perspectives to decision makers. We feature one example where the advocate has been working within formal HTA processes for more than ten years, and another where the advocacy group has worked on its own set of data to highlight the access challenges that patients face and the impact that these have on their lives. Throughout these examples, you will see that advocates engage with the decision makers and the science with confidence, knowing that their viewpoint is valued and respected by the other expert disciplines. Access challenges are not going to go away, but these examples show that access advocacy is rising to that challenge, and the time to engage is now.

TOP TIPS

1. Each example shown in this short booklet will have a range of tips to consider when thinking about a similar approach

Each of the experiences in this booklet will point to some independent tools, guidance or explanations that can help other advocates get started in access advocacy. Look out for this icon to see relevant tools at the back of this booklet.

Participation of the advocates in this document is non-binding, voluntary and non-remunerated. Bristol Myers Squibb (BMS) provided financial support for the writing, editing and printing of this publication. BMS did not provide any fees to any of the advocates or their representative organizations for their involvement in this document. The content of the final document reflects interviews conducted with the advocates who had full editorial control over the final articles.

INVOLVEMENT IN A HEALTH TECHNOLOGY ASSESSMENT

ROY CASTLE LUNG CANCER FOUNDATION

Jesme Fox from the Roy Castle Lung Cancer Foundation has been working with England's health technology assessment body, NICE, for many years. For her, the important aspect is bringing the patient experience to decision-makers so that they can make better informed decisions.

Last year was a very busy year for Jesme Fox. A total of 16 products affecting lung cancer patients were assessed by the National Institute for Health and Care Excellence (NICE) in 2016, and Jesme had a part to play in bringing the patient perspective to each one of these assessments.

For Jesme, access advocacy is about making access better by bringing the perspectives of people with lung cancer to the decision-makers. "It is not about saying 'yes' to everything," Jesme explains. "It is about ensuring the best access wherever appropriate, by bringing the insights from the people living with lung cancer to those who are assessing the treatments."

This is not an adversarial role, where the patient organizations sit on one side, fighting the decision-makers - it is a partnership role where the patient organization brings a valuable perspective that is respected by the other disciplines making a decision. "I have a lot of respect for the people at NICE and the difficult decisions that they have to make," says Jesme. "There are specific challenges in understanding the data that exists when a treatment is very new, and by bringing the patient perspective into those decisions, we can highlight specific aspects of a treatment or its effects that have the most impact on patients," she says.

"To be involved as an access advocate you need to have rounded experience. That does not mean that you need to be a patient yourself, but you do need to know how to call on a body of patient experience that is relevant to the assessment itself," Jesme explains. In lung cancer it can be hard for patients themselves to take part in this process. "The new treatments tend to arrive for late-stage disease, where the median survival is around eight months. We need to draw on the experience of people with this stage of lung cancer, but it is unrealistic to expect these people to devote the time and energy into access advocacy itself," she says.

NICE has a very specific and well defined process for assessing new medicines, and for Jesme, the first important step in access advocacy is to understand that process. "The process can take a long time and it is a bureaucracy of paperwork," she explains. There are many steps to this process and we get involved in most of these steps at some point in the process.

Outlining the process, Jesme says that it can start well before a new treatment comes for review: "The first step that NICE undertakes is 'Horizon Scanning' - where NICE look at the announcements from congresses such as ASCO or ESMO and try to get a sense of what treatments will be coming in front of them in future. Depending on the situation and the treatment, we may be asked at this point to give our opinion at this very early stage," Jesme says.

Then, once NICE are more certain that a treatment has gone to regulators for a marketing license decision, they will begin the 'Scoping Phase'. "It is here that NICE often works with patient organizations such as ours to determine the fundamental questions that the drug appraisal should consider," explains Jesme. "For example, we may be asked to comment on what people with this stage of disease usually receive for their treatment - leading NICE to understand what would be an appropriate comparator for the new drug in their assessment."

Fortunately, NICE offers a lot of support to patient groups, with on-line guidance, training and dedicated officers who look after the needs of the patient organizations taking part and help them understand what their role is. Jesme explains that: "As an access advocate, there are three key areas you have to get across to decision makers. The first is 'where does your organization get its experience from', i.e. why do I have the credibility to speak on behalf of the lung cancer community? The second is 'what is the experience of the patients relevant to this particular appraisal, i.e. what can I tell them about patients at this stage of disease that match the profile of the treatment under review? The third is sharing experience about the treatment being reviewed, which can be hard to answer."

TOP TIPS

1. If you have a HTA process that allows public observers, go and see one before you get involved
2. Know the process that is required in your system
3. Take advantage of any training or support offered by the HTA body
4. Understand your role as an advocate in the process, where you can add value to the decisions and where your insights fit with those of other experts

One of the areas where Jesme believes advocates add value is outlining where this new treatment may fit in with current approaches. What does the target patient population look like, and what other options do they have?

At NICE, the decisions are taken by an appraisal committee who invites expert members to bring perspectives and analysis to the committee. "Besides myself, representing the patient perspective, we often have expert nurses and always an expert clinician, preferably one that has had experience of the treatment under review," says Jesme. "This is a very formal meeting. You are taken through the data and the committee asking me at various points questions that will allow them to better understand the patient context of the data," she adds.

As treatments are approved quicker for their marketing license, Jesme sees that this role becomes even more important. "Decisions are made much faster now, the process is quicker," Jesme explains. "But that means that NICE are looking at small sets of data that have a lot of uncertainty in them. What we bring is the patient perspective that helps the committee focus on the areas that make the most impact for patients."

It is more than ten years since Jesme first attended an appraisal committee meeting, and in that time the role of access advocacy has changed considerably. "We have evolved," says Jesme. "We are part of the process now, not campaigning from the outside. In the early days NICE would say 'no' to something and our only option was to campaign to overturn that decision. Now we are partners and understand the difficulties in making these decisions. It does not stop us from campaigning if we disagree with them, but there is a new-found respect on both sides."

1 NICE Public Involvement Factsheet

2 NICE Patient/Carer Submission Template

NICE Hints and Tips for Patient Experts 3

INTERNATIONAL ACTION

Kathy Barnard of Save Your Skin in Canada knows first-hand how access can transform a patient's chances. With Canada's fragmented healthcare system, Kathy has been spearheading submissions to the assessment authorities and believes the next step is to work internationally to share learnings, approaches and evidence globally.

Melanoma is a cancer that has seen much recent progress with treatments. While this is very good news for patients, the only way that they will benefit from these scientific advances is if local healthcare systems grant access to these new approaches. Kathy Barnard has been on the front-line of these access debates for more than ten years.

For Kathy, the impetus for taking on the challenge of working in access advocacy came from her own experience after being diagnosed with stage 4 malignant melanoma. Her search for treatments, for clinical trials and for answers made her realize that there was much work to do to ensure that patients had access to the care that they needed. "Ten years ago I would not have known how to be an advocate," Kathy says. "But I realized from my own experience that there was a need to get involved, not just sit on the sidelines. So I made it my mission to learn about the decision making process, to engage with those that decide what is available, and to see where we could make a positive change in access."

Kathy explains that we should not confuse advocacy with lobbying. "I think that in those early days of access advocacy, the decision makers saw us lobbyists who were trying to subvert the system," she says. "Actually, I always saw myself as representing the patient perspective as a true advocate. I don't want to be telling the government that it is wrong, I want to be working with them hand-in-hand developing the solutions."

It is this approach of partnership working that led Save Your Skin to devote resources to work with the formal submission processes of the Health Technology Assessment agencies pCODR and CADTH. These agencies have a process of advocate input into the decision making process through a submission process. This takes the form of a written submission form, populated by data that the advocates collect from their members and networks.

"What we are aiming towards is timely treatment and fair treatment for melanoma patients in Canada," Kathy explains. Although the submission forms that Save Your Skin complete for this process can seem restrictive, the team have learned how to gather the right evidence to bring to life the patient experience of melanoma and its treatment.

Through this process, Kathy and her team have realized just how much duplication is involved in preparing these submissions. "The first time you do this, you have nothing, you are starting from scratch," says Kathy. "But, then you have the second submission to complete and then the third, and so on." Kathy explains that much basic knowledge on how patients experience their melanoma is already contained in previous submissions, allowing Save Your Skin to concentrate on gathering the information needed for this particular submission.

"But what this process also taught me is that we should not be doing this in isolation. I know that groups across the world are similarly collecting evidence, for very similar submissions," she says. "Surely there must be a chance to combine our efforts, learn from each other, and share those access advocacy approaches that work."

Equitable, timely, sustainable treatment is something that applies to all of us, regardless of which country we are in," Kathy says. "We have an opportunity now to bring the great work that we are all doing together to make it stronger and to speak with one voice."

Kathy believes that those who engage with the decision makers have won the right to sit at the same table, to debate the options and to work with the system to make it better. "We have learned so much, just in Canada by being at the table," she says. "What we need now is to be able to share evidence, share databases and share good practices across the world. Already, I look to advocates in other countries to ask them what evidence they are generating, and it is time to formalize this into a resource that we are all plugged into."

That is why Save Your Skin is developing a Global Network that will focus on creating a platform to exchange ideas, share best practices and share the evidence needed to put forward the patient perspective to decision makers. But Kathy is keen to stress, that this kind of network will only be successful if it opens its arms to decision makers too. "We have already been approached to host an international meeting made up of patients, advocates, health economists, oncologists, health care professionals, HTA experts and policy makers."

She notes that even a few years ago, it would have seemed unthinkable that such a diverse set of healthcare stakeholders would be willing to work together on the solutions for tomorrow's access landscape. "The outputs from this meeting will flow directly into our network's think tank which will be looking at systemic changes needed to improve access to care." For Kathy, this is an important step because it moves the advocacy community to the next level - not just involved in the submission for a single therapy that is being assessed at one moment in time - but to a chance to think about the whole system and how this could be made better for all future access decisions.

"I am excited by this, because now is the time for action, not talk. We have working groups in this Global Network which will be responsible for specific deliverables and timelines," Kathy says. "This is where we have got to as access advocates, genuine partners in a global solution to improve the access for all."

TOP TIPS

1. Get to know your local access system, pick up the phone, look for training on their website and find out how you can be involved
2. If you can, position yourself as part of the solution. You have unique knowledge of the patient experience that decision makers need to know
3. Don't reinvent what has been done before. Reach out to other groups locally and internationally to see what evidence they have already created

4 CADTH guidance for patient organizations

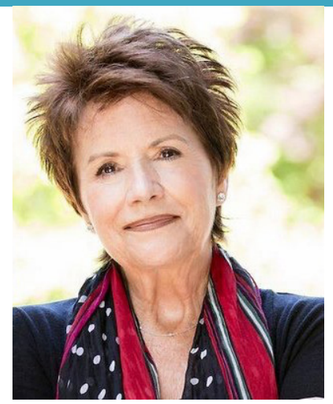
5 The drug evaluation process: Patient Input Submission Webinar

Save Your Skin library of submissions to CADTH 6

The Pharmaco-economics of Cancer Drugs Webinar 7

EVERYTHING IS AN ACCESS ISSUE

BONNIE J ADDARIO LUNG CANCER FOUNDATION



In 2006, when Bonnie Addario started the Lung Cancer Foundation, her hope was to transform lung cancer into a disease that can be managed chronically. However, as soon as she started, she noticed that the cards were stacked against those with lung cancer, affecting their access to care all along their journey.

For Bonnie Addario, access issues happen all across a patient's journey, not just at the moment when a decision is made to reimburse a treatment. "When we started this foundation, our intent was to start fundraisers, donate the proceeds to research organizations and let them get on with research," explains Bonnie. "But then we began noticing access barriers for patients everywhere we looked."

"The stigma of having lung cancer is incredible," says Bonnie, "and it can affect the access that people with lung cancer are offered." She realized the importance of this stigma when she was diagnosed with lung cancer in 2004 and undergoing extensive treatment and surgery before becoming a lung cancer survivor. "When we set up the foundation, our initial focus was on early detection. I am living proof that lung cancer is survivable, if it is caught early, and yet there was no formal screening program."

So, the foundation made early detection its first access fight, raising awareness both with the public and with healthcare decision makers. "There were studies showing the benefits of early detection, but no one was doing anything about them," Bonnie remembers. "We campaigned relentlessly on this issue. We could see the positive effects the breast cancer advocates were having in improving services and care for their community and we wanted the same kinds of improvements for people with lung cancer."

Change doesn't happen instantly, but last year the Centers for Medicare and Medicaid Services (CMS) in the USA approved CT screening for lung cancer, and although this is still restricted to certain risk groups, it is a start.

"We look at the whole experience of lung cancer to see where the barriers are," Bonnie says. For example, she and her team started looking at the standards of care in community centers and comparing it to best practices. "Around 80% of all cancer patients are treated in community centers in the US," she explains. "We found that most were not equipped to deliver best practices."

Working with GE Healthcare, Bonnie and her team designed a 'Lung Cancer Community Centers of Excellence' program which has 15 centers signed up. These centers must provide a standard of care that lives up to the latest medical science. "We collect metrics from these so that we can publish data on the importance of having these high standards of care," Bonnie adds.

An element of the Community Centers of Excellence project is the right for patients to have access to clinical trials. "This is an often-overlooked access issue," explains Bonnie. "Often trials happen in only 10 states in the country and so we need to work as a community to find ways of making these accessible to people across the country."

Much clinical data comes from industry trials, but Bonnie explains that advocates are well on the way to generating their own insightful data. "When we first started, we could see that there needed to be so much done to collect the evidence needed to improve lung cancer care," Bonnie says. "But we were not being invited to the 'big table' where all the decision on what data to collect and how to use it were being decided. We believe that the solution to better care lies with the patient and our experiences."

The Lung Cancer Registry is sponsored by:

AMERICAN LUNG ASSOCIATION. LUNG FORCE

So, partnering with others, the foundation developed a global patient registry project. "This was our way of ensuring that we had a seat at the table on data generation, by basically creating our own table that everyone would be invited to," Bonnie explains. The Lung Cancer Registry has been designed for anyone affected by lung cancer to help researchers understand the disease and also help identify geographical areas or patient demographics where access is a particular issue.

"Patients enter their own data and the registry can connect to electronic medical records to validate the data and provide more information such as scans," Bonnie explains. "The data is open to all, and we want people to be able to use it in many ways to help improve the care offered to patients and to look for patterns in the data that may lead to new breakthroughs."

As an example of how this kind of registry data can be used for access advocacy, Bonnie points to the fact that this registry will collect ethnicity data, gender, where people were born, the city they get their care in, as well as the clinical data. "We will start to see trends that we have never seen before," Bonnie says. "This could be such a door-opener on the access discussions, because we will be able to see if people in a certain area, or from a certain background are regularly receiving sub-optimal options compared to others."

TOP TIPS

1. Look at access issues from various angles, not just the reimbursement decision
2. Don't think that you have to do everything on your own. Partnership working is the way forward
3. Think about the information and data that you need to make your arguments
4. The patient experience is important information that no other stakeholders can provide

As a global registry, Bonnie hopes that other advocates will access the data to answer some of their own access needs. "We can customize the data output from the registry to look at particular factors. So, for example, if an advocacy group in the UK needs some data to input into an access decision, then we can work with them to get them the data they need," she says.

It is not just the data that is open to others, the whole platform itself is open as well. "This means we can partner with different members of the lung cancer community to build customized projects," Bonnie explains. Examples include working with researchers from Moffitt and Thomas Jefferson on an Immunotherapy Patient Reported Outcome study as well as using the Registry as part of the 'Beat Lung Cancer in Ohio' study. "This flexibility is thanks to the many ways we can collect data on the platform."

"Personally, working in this area has enriched my life enormously," Bonnie says. "I would not have the knowledge that I have today without being on this journey. I survived lung cancer and that felt like a gift that I needed to do something with. The foundation has partnered with some amazing people and organizations and, as a team, we have learned how to take those access issues one at a time and target them with programs and evidence."

MEASURING VALUE
THE ESMO MCBS

Melanoma Patient Network Europe

Gilly Spurrier and Bettina Ryll from MPNE became interested in the ESMO Magnitude of Clinical Benefit Scale (MCBS) when they noted that some Melanoma therapies, which in their opinion were promising, received comparatively low ratings. While they believe that consistent value frameworks benefit patients, they see it as their responsibility as patient advocates to ensure that these frameworks adequately capture what matters to patients.

The past years have seen a veritable explosion of value frameworks, in particular in the US, after similar concepts have existed for years in Australia and Europe. The phenomenon is mainly driven by decision-makers grappling to come to terms with judging the benefit of costly new therapies, in particular in the field of oncology. More recently, also a cancer center and medical societies have produced their own value frameworks (the Drugabacus by the Memorial Sloan Kettering Cancer Center, the ESMO MCBS and the ASCO value framework, all 2015).

"While one can wonder whether anyone needs such a number of independent value frameworks, this trend clearly shows that different stakeholders have a need for consistent approaches to the assessment of value, in particular in times of limited resources," says Bettina Ryll, the founder of MPNE. "In our opinion, consistent value frameworks are valuable tools to ensure broad and fair access to effective therapies for patients," explains Gilly Spurrier.

Indeed, one of the original motivations for the ESMO MCBS had been oncologists' desire for a tool to identify those drugs with the greatest clinical benefit to ensure fast and wide-spread access for patients in Europe. "As the international organization committed to the interest of the oncology community at large, we are concerned about some anti-cancer medicines approved by the European Medicines Agency (EMA) not being available or affordable to patients when prescribed," says Rolf A. Stahel, ESMO President. "With the ESMO-MCBS, we aim to signal the drugs with a large magnitude of clinical benefit which should be endorsed across Europe for rapid patient access, especially when these medicines are recommended through evidence-based standards set forth in the internationally recognized ESMO Clinical Practice Guidelines."

MPNE widely uses the ESMO Melanoma guideline to educate European Melanoma patients about standards in Melanoma treatments, in particular in the advanced setting. Bettina and Gilly became concerned when some of the ESMO MCBS recommendations for recent but, in their opinion, highly promising treatments were relatively low.

"Ratings like this, in particular when they come from a trusted and respected source like ESMO, carry enormous weight," says Gilly whose husband is living with Stage 4 Melanoma. "My concern is that low ratings directly influence treatment and access decisions and a patient with advanced Melanoma simply does not have the time to wait for more evidence to accumulate but needs to access treatment immediately."

"When one looks into the methodology of the ESMO MCBS, the emphasis on a solid evidentiary basis is clear. This is also what we have seen: With new evidence available, MCBS ratings shot up considerably. So our concern is the space 'in between'. Melanoma is a desperate cancer where delay is paid for with lives. In such a situation, we would like to see a way to capture 'promise' while fully acknowledging the lack of sufficient data," explains Bettina.

"We have seen in our work that Stage 4 Melanoma patients are way more risk-accepting than healthy individuals. Telling these people they cannot access a drug 'for lack of evidence' while fully knowing that the alternative is death is unacceptable from our point of view," adds Bettina whose husband died of Melanoma in 2012.

Gilly and Bettina are further concerned that the current version of the ESMO MCBS only incorporates Grade 3 and 4 toxicities and is not sensitive to patient subgroups with particularly good or bad prognosis. "With patients living longer and longer, low grade toxicity begins to gain in importance as people want to live well, not just survive, just as we have seen with our colleagues from the CML community," says Gilly. "So we need to start paying attention."

Bettina adds: "The MCBS relies on clinical trial data, so unfortunately, inadequate trial design directly affects the scale. If trials are insufficiently powered or subgroups not pre-specified, there will simply no way to arrive at a high value for a subgroup of patients who might be doing particularly well. However, that's not really a problem specific to this scale. Generating high levels of evidence for increasingly smaller patient populations is one of the central challenges of personalized medicine."

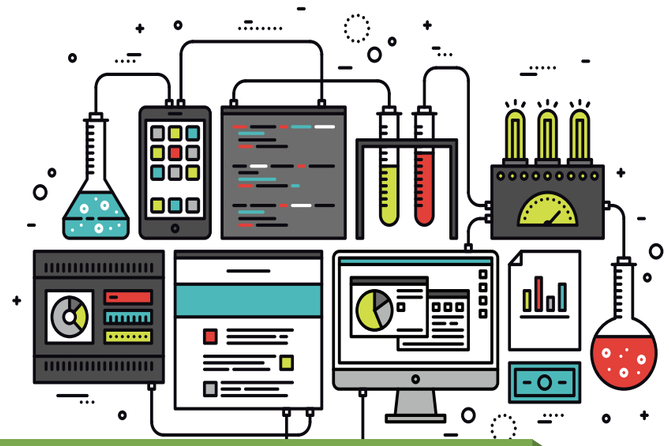
The two have now reached out to the MCBS working group to for the detailed calculations behind the ratings for Melanoma therapies and how these changed with new data becoming available, and are looking forward to an in-depth discussion with the chair of the working group. "We already know that they are thinking about incorporating low-grade toxicity and we hope to also arrive at something constructive on the other issues. Any tool has strengths and limitations, so the MCBS might just not be right in the 'high unmet-need, promising but immature data' setting. But a conclusion like 'access under evidence-generation' would very much be in patients' interest," says Bettina.

"Of course it would have been great to be involved in such an initiative from the beginning. However, this work started in 2014, if not earlier and we only started our network back then!' says Gilly. They see it as their responsibility as patient advocates to ensure that processes affecting Melanoma patients are in patients' best interest. Being constructive is one of the four principles of their network, next to absolute patient-focus, evidence-based advocacy and proactivity.

"We've only ever have had good experiences with that approach. It is a matter of investing the necessary effort to really understand the problem, the context and the motivation of the people involved. Other stakeholders are not always used to that approach coming from a patient group but ultimately, what are they going to say against rock-solid argumentation?" say Bettina and Gilly. "After some initial irritation, most are actually grateful for constructive input and we have built a number of mutually beneficial working relationships that way. No one has all the knowledge, but very few people have actually bad intentions," they conclude.

TOP TIPS

1. Any process can be criticized and improved if done properly
2. Do your homework. One cannot solve a problem one doesn't understand
3. Focus on the problem, not on people



BE THE DRIVER OF CHANGE

Andrew Spiegel is an access advocate with both national and international experience. This has led him to consider how the access experience in the US can be used to help other countries build the arguments for better access. Being open to opportunities and mindful of what you do not know is part of his recipe for success.

The Global Colon Cancer Association advocates for patient centered policies that build increased awareness of colon cancer, the need for screening and access to quality medical treatments. Andrew Spiegel, Executive Director of the Global Colon Cancer Association likes the challenge of access advocacy. "I have been a patient advocate since 1998, co-founding the Colon Cancer Alliance in the USA, and I have seen how hard it is to get colon cancer on the agenda, and even basic steps such as screening programs have been an uphill struggle," he says. "But for me, access advocacy is worth the struggle. I see it as trying to get the best possible care for the greatest number of people that you can."

It is a challenge that is getting increasingly more difficult as health systems around the world find it difficult to cope with aging populations. "It is our job to make compelling arguments about treating and preventing diseases like colon cancer so that people do not suffer unnecessarily."

For Andrew, the whole prevention argument is a very interesting one to tackle from an access perspective. "In a way, we are fortunate in colon cancer to have a range of screening options that have been shown to spot polyps, which are often the precursor to cancerous growths. These can be removed before they turn malignant."

Yet, although the science is clear on the benefits of screening, it is something that the healthcare systems have been reluctant to invest in. "Payers have a very short window of time that they look to make a return on their

healthcare investment. With screening programs, you are preventing a cancer that may not appear for another decade, if at all," he says. "That payer may have moved on before any savings of a screening program come to fruition. So it can seem like they are paying the cost of the screening now with no idea when they will reap the rewards of the savings for the healthcare system."

In the USA, it took many years to win this argument, with just a few insurers willing to fund screening programs. The turning point came when the evidence started to mount on the cost-benefits of screening. "Suddenly we had multiple insurers offering screening. They began posting out screening kits to their members and even the US Military began a screening program," Andrew remembers. "It was the advocate community along with physicians and economists that worked together to bring this change."

In 2011, Andrew joined forces with Jola Gore-Booth of EuropaColon to launch the Global Colon Cancer Association to create a truly global community devoted to battling the disease with one unified voice. One of the items at the top of their agenda was raising the access to screening for colon cancer across the world.

"Five percent of the population worldwide are thought to be at risk from colon cancer. Think about that when you are in a meeting of 20 or so people, on average one of the people in that room could be at risk," Andrew says. "And so, not only is screening an economically sound approach, it is the moral thing to do for all those people."

People are quick to point out that the US healthcare system is not representative of many systems across the world, and so some people will challenge transferring experience from the US environment to other countries, but Andrew insists that it is about taking the right lessons forward and applying them to these different environments. "For me, I think of the challenges we had in the US as two-fold. Firstly, we had to convince decision makers that access to screening made economic sense. But we also had a second challenge to overcome, the stigma associated with any discussions of bowel disease. People just wouldn't talk about it and so raising awareness was a difficult issue."

Many parts of the world have relatively young and poorly financed public healthcare systems and for Andrew, these provide a real opportunity to engage with access issues on screening as the potential benefits are clear for a relatively modest upfront cost. "In some of the emerging healthcare systems, I see really clear parallels with the screening situation we had 20 years ago," he explains. "The stigma is there and the lack of a coordinated voice from the healthcare stakeholders is there too."

Thinking back across the successes in the US, Andrew realized that to make access advocacy work in these countries, he needed to build a coalition of expertise that spanned clinical and health economic expertise. "I know that the last thing that would convince a payer in Latin America would be an American turning up and telling them how to do this," he says. "So, I attended a conference where all these experts in health economics attend and I started to ask them if they would be willing to donate their time and expertise for a good cause." They said yes, and Andrew now has nine specialists in Latin America who are knowledgeable about the local access environment to work together on a package of arguments and evidence generation, designed for each individual country across the region. "They already talk to ministers about access issues, they know how to navigate the decision making process," he says. "I don't have all the answers, I don't have exclusive knowledge, but I do know how to get people together and rally around a cause. Together we are designing the access arguments fit for the local environment and this September will be putting our access strategies in front of the decision makers and policy makers. We'll also be raising the profile of colon cancer in a way that will start tearing down the stigma that still exists in these countries."

TOP TIPS

1. Be confident in your ability to rally people around the access cause, don't try to be the expert in everything
2. Look for tools that help you navigate decision makers such as IAPO's stakeholder engagement toolkit*
3. Ask for help from the experts, you may be surprised when they offer it for free
4. Don't think that your system is so unique that others cannot learn from it

**Andrew is a board member of IAPO*

BUILDING EVIDENCE ON ACCESS ISSUES

The USA's Cancer Support Community (CSC) embarked on a multi-year project to quantify and bring to life the access issues faced by people with cancer in the US. **Linda House**, President of the CSC explains how the combination of evidence and patient stories is a powerful tool for working with access policy makers

The Cancer Support Community were already well aware of the difficulties that patients with cancer were facing in accessing appropriate care and treatment. The advent of the Affordable Care Act promised to remove many of the barriers that patients had flagged as critical issues, including the lack of affordable insurance for those with pre-existing conditions and lifetime caps on coverage.

"It was exactly one year since the implementation of the Affordable Care Act and we knew that it would be important to continue the work with access policy makers to ensure that access to cancer care remained at the top of the agenda," explains Linda. "The kinds of access issues we were still hearing about shouldn't happen in any country, and we were determined to highlight them to the US policy makers."

The challenge for the Cancer Support Community team was to cut through the inertia and the complacency of the policy makers. "What was clear is that patient stories in themselves were not going to be enough to get the policy makers attention," says Linda. "In fact, we have heard in public meetings policy makers in Capitol Hill say that they are 'tired of having patients march in front of us'"

The Cancer Support Community took a two-pronged approach: Generate the evidence that showed what was really happening across the US; and contextualize that evidence with patient stories that highlighted the physical, mental and emotional impact of the access barriers that the evidence showed. Few good ideas come to fruition in one single stroke and this project was no exception. The team had to think hard about the kinds of data that they would need to collect.

"When we started thinking of this, we actually had something very specific in mind. We wanted to take a look particularly at the new 'market places' that were set up to offer insurance through the new system," Linda explains. "However as we started looking at the issues that patients were coming to us with, we realized this was far too narrow and what was needed was a piece of research that looked much broader across the community."

The first report, released in March 2015, focused on:

- Access to and satisfaction with health insurance
- Access to providers, including availability, time and discussions with providers
- Access to services
- Concerns about the direct cost of cancer care

The team also realized that in this digital age this project should deliver much more than a printed report. To back up the report, the team developed a series of patient videos and a documentary which could be used in multiple environments and with a much larger audience.

The team developed a cross-sectional survey of adults affected by cancer which went live in October 2014. Linda emphasizes that the strength of the Cancer Support Community's networks and registry were vital to ensure that enough people completed the survey. "We used several avenues open to us," explains Linda. "We have built up an extensive on-line network over the years as well as our Cancer Experience Registry." The team used these networks as well as reaching out to advocacy partners and social and traditional media outlets. Almost 700 people started the survey, with 511 complete responses, giving enough data for the team to conduct their analysis.

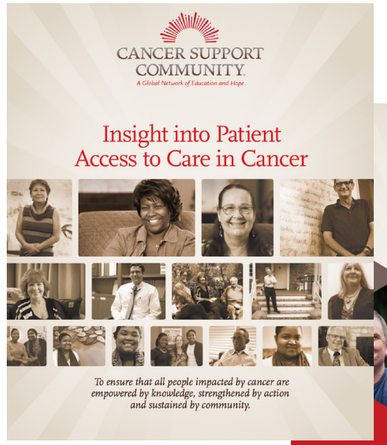
The evidence was compelling, showing that almost a quarter of patients were experiencing delays in accessing care, and a similar amount felt that they did not have enough time with their health care team. Almost three quarters reported that they were not receiving social and/or emotional support services. In terms of costs, over a third of the patients reported being seriously or very concerned about bankrupting the family.

Using simple graphs, and clear descriptions, the report was structured in a way that policy makers could instantly see the impact of access issues for patients. Pictures of patients were shown alongside the evidence to remind the policy makers that behind these numbers are real people. "We passionately believe in evidence to support the points we need to make, but we also need those patient stories to bring that evidence to life," says Linda.

That is why the team developed a documentary to accompany the report as well as a series of videos. These allowed the team to develop a holistic story to discuss with policy makers, so that the impact on individuals was just as compelling as the report. "We needed the policy makers to know that we were bringing them messages from patients, not from Linda," she explains. The report was launched and used to ensure that policy makers understood the positive impact that the Affordable Care Act was delivering, but also the remaining challenges with accessing cancer care. "But, we didn't stop there," says Linda. "We knew that we would have to keep looking at this issue."

And so in November 2016, the team launched their second report in this area. "Although the Affordable Care Act had removed some of the most basic barriers to accessing health insurance, we were hearing that new practices were being put in place that were adding new restrictions." This second report built on the findings of the first but also highlighted how new cost-containment strategies were impacting patients including prior-authorization for prescribed treatments, step-therapy requirements and difficulties finding specialists in their insurer's network.

Leveraging the strength of the community is at the heart of the approach that the Cancer Support Community has developed. "For us, it is about delivering against our mission to ensure that all people impacted by cancer are empowered by knowledge, strengthened by action and sustained by community. Bringing together this evidence for the community ensures their voice is heard by those making the decisions, backing up their stories with real data," Linda says.



TOP TIPS

1. Evidence and patient stories work well together for policy makers
2. Advocate's existing networks of members are a valuable source of the evidence
3. Be focused on what you are trying to achieve and collect the appropriate data
4. Present the data clearly with graphs and charts and consider backing up with video

1 NICE PUBLIC INVOLVEMENT FACTSHEET

WHAT IS IT?

From NICE, the HTA body in England: A guide to public involvement in the NICE health technology appraisal process, detailing an introduction to HTA, and how patient organizations can get involved.

WHY IS IT RELEVANT?

NICE is one of the HTA bodies that is globally recognized for its public involvement processes. This 8-page guide gives a very good overview into the role of patient organizations in the decision-making process. This is not a complex document and so is useful for those wanting to get an overview of the process.

HOW CAN I USE IT?

Whatever the process in your country, the NICE factsheet can give an overview of the steps that are often taken in a HTA process and the relevant roles of patient organizations. Use this document with yourself or colleagues that are trying to understand HTA.

WHERE CAN I FIND IT?

https://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Developing-NICE-guidance/Overview-TA-Patient-Carers.pdf

2 NICE PATIENT/CARER ORGANIZATION SUBMISSION TEMPLATE

WHAT IS IT?

From NICE, the HTA body in England: This is the submission template that patient organizations use to submit their perspective to NICE. It lays out all the pieces of information that NICE are looking for from patient groups.

WHY IS IT RELEVANT?

Many HTA bodies have submission templates, and these can vary in terms of their content. However the basic principle that patient organizations can add value by bringing to life the patient perspective is central to all of them. Studying this template will allow you to understand the main areas that a body such as NICE is looking at from the patient organization perspective.

HOW CAN I USE IT?

This template can be used to organize your own thoughts on a health technology, even if your decision making body does not use submission templates. Just looking at the headline questions in this template can help you think of areas you can focus on in your response to a HTA. *If you have a HTA body that does take submission templates, always use the template provided by that body.*

WHERE CAN I FIND IT?

https://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Developing-NICE-guidance/Patient-Organisation-STA-Template.docm

NICE HINTS AND TIPS FOR PATIENT EXPERTS 3

WHAT IS IT?

From NICE, the HTA body in England: This guidance is for people who are attending a technology appraisal committee meeting as a patient or carer expert.

WHY IS IT RELEVANT?

Appraisal committee meetings can be daunting. It is important to know what will be expected of you and to have details about how the meeting will be run. This guidance walks you through the process, explaining the different parts of the meetings and what happens during this formal process.

HOW CAN I USE IT?

Before attending a HTA appraisal committee meeting look for documents like this from your own HTA body. If these documents do not exist, read through the guidance from NICE and draw up a list of questions to ask the HTA body on their own processes. Use these questions to get clarity about your role and the expectations of your input at the meeting.

WHERE CAN I FIND IT?

https://www.nice.org.uk/Media/Default/About/NICE-Communities/Public-involvement/Developing-NICE-guidance/Hints-Tips-Patient-Experts.pdf

CADTH PATIENT ENGAGEMENT GUIDE 4

WHAT IS IT?

From the Canadian HTA body: Guidance for patient and advocate input into the review process in Canada, detailing how advocates can participate and guidance on filling in the submission templates used in the review process

WHY IS IT RELEVANT?

Many countries use a process of Health Technology Assessment to determine if a national or local health service will reimburse a medicine. In several countries, advocate groups are invited to give their perspective via a formal submission process, often using a template that the advocacy group fills in. This is the process that the Canadian national assessor uses, and this guide provides a comprehensive overview of that process. Section 6 in particular gives guidance on filling in this template.

HOW CAN I USE IT?

For advocates in Canada, this guide details what exactly is expected in each part of the form as well as an overview of the general process. For those outside Canada who are starting to fill in these kinds of templates, section 6 of this guidance provides some general guidance on what the decision makers are looking for.

WHERE CAN I FIND IT?

https://www.cadth.ca/sites/default/files/pcodr/pCODR%27s%20Drug%20Review%20Process/pcodr-patient-engagement-guide.pdf

5 PATIENT INPUT SUBMISSION WEBINAR

WHAT IS IT?

From Save Your Skin Foundation: This webinar discusses the process used to evaluate drugs, concluding with recommendations about whether they should be covered by provincial formularies. In particular, this webinar looks at how patients can engage in the process

WHY IS IT RELEVANT?

National decision making is not the only access barrier. Often there are additional decision makers in local districts and provinces that further assess the access to new treatments. In this webinar the speakers discuss how patients can engage with the drug evaluation process, what are the best practices to promote the patient's voice and a discussion on how to improve patient's opportunity to engage in the process. Recorded in 2015.

HOW CAN I USE IT?

This will give the Canadian view on where and how the patient voice can be valuable. It is useful to hear these discussions to spark ideas in your local country on how advocates can engage in the process there. Note that the link below requires you to register your name and email to access the content.

WHERE CAN I FIND IT?

https://attendee.gotowebinar.com/recording/8678172970450867714

6 SAVE YOUR SKIN LIBRARY OF SUBMISSIONS

WHAT IS IT?

From Save Your Skin Foundation: A library of submissions and recommendations from the Canadian HTA body that have had submissions from Save Your Skin Foundation as part of the evidence package that was reviewed.

WHY IS IT RELEVANT?

When engaging in access advocacy, it is useful to look at how decisions are made and how other advocacy groups have submitted information into these decisions. This library contains the submission forms that Save Your Skin Foundation have submitted, showing how they answered the questions in the submission forms. It also contains notices from the provinces across Canada to show where particular treatments have received a funding decision.

HOW CAN I USE IT?

Studying the examples of submissions from other groups can help you refine your approach to a submission process in your country. This library also allows you to see the relevant funding decisions related to a submission. Use these as guides or examples when you are thinking about an approach to input into a formal HTA system.

WHERE CAN I FIND IT?

http://saveyourskin.ca/pcodr-submission-news-surveys/

THE PHARMACO-ECONOMICS OF CANCER DRUGS WEBINAR 7

WHAT IS IT?

From Save Your Skin Foundation: This webinar provides a greater understanding of the role of health economics in reimbursement decision making using the Canadian system as an example.

WHY IS IT RELEVANT?

Although patient advocates are not expected to be experts in health economics, they are often in meetings with health economic experts and it is useful to understand the basics of the science of health economics to take a more active role in discussions. Also, some advocates may see information related to an analysis of health economics and it could be helpful to have a grounding in this subject when reviewing this kind of evidence.

HOW CAN I USE IT?

If you are new to health economics, you can use this webinar to familiarize yourself with the concepts. Anyone attending a HTA committee meeting, who is unsure of how health economics plays a role in decision making could use this webinar to understand the perspective of the economists around the table.

WHERE CAN I FIND IT?

https://attendee.gotowebinar.com/recording/478647471251448580

THE LUNG CANCER REGISTRY 8

WHAT IS IT?

From the Bonnie J Addario Foundation: A global registry of patient entered data looking to build the knowledge base on patient experience with lung cancer, their care and their outcomes.

WHY IS IT RELEVANT?

This registry is an example of how advocates are building the evidence base to secure access for the future as well as identifying patterns in the clinical profiles of patients that could lead to new scientific breakthroughs. The way that this database captures demographic as well as clinical information is an important factor in using these kinds of registries for access advocacy, as it will allow advocates to see if particular locations or demographics are exhibiting poorer access than others.

HOW CAN I USE IT?

For those working in the lung cancer arena, this registry provides a potential source of data for your access discussions. For those working in other areas of oncology, this registry provides an example of how advocacy groups are working with other experts to develop their own data sources.

WHERE CAN I FIND IT?

https://www.lungcancer-registry.org

9 REGISTRY OF PATIENT REGISTRIES

WHAT IS IT?

From the Agency for Healthcare Research and Quality: This is a searchable database of patient registries, designed to help people and researchers find registries that already exist.

WHY IS IT RELEVANT?

Registries can provide important data that is useful to the access debate, showing how people are really treated for their disease. This information can be vital to show decision makers that there are gaps in care or there are particular parts of the patient community who are not served well by current approaches to care.

HOW CAN I USE IT?

You can search this database for the disease you are interested in to see if there is already a registry devoted to collecting data on patients. However, there are many other databases of registries. Some, for example focus on the registries used to collect data on rare diseases including rare cancers. Also remember to ask others in the field about the registries available.

WHERE CAN I FIND IT?

https://patientregistry.ahrq.gov

10 BUILDING SMARTER PATIENT REGISTRIES REPORT

WHAT IS IT?

From Faster Cures: A detailed report looking into the future of patient registries, how they are evolving and how they can lead to meaningful outcomes.

WHY IS IT RELEVANT?

This is a very recent report (2016) looking at the state of patient registries that are led by patient organizations. It takes a snapshot of the situation today but also looks forward to the practices and approaches that would enhance existing registries and inform the creation of new ones. It is a useful report for any group that is thinking of initiating a patient registry or for updating existing registries.

HOW CAN I USE IT?

This report can be thought of as a guide for patient registries for those that are new to the subject. It also contains some guidance, tools and checklists on planning and implementing a patient registry and so forms a useful background for those that are starting discussions on potential patient registries.

WHERE CAN I FIND IT?

http://www.fastercures.org/assets/Uploads/PDF/Patient-Registries.pdf

ESMO BENEFIT SCALE BACKGROUND 11

WHAT IS IT?

From the European Society of Medical Oncology: A range of resources and videos explaining the ESMO Magnitude of Clinical Benefit Scale, including links to published papers on the scale, presentations explaining the scale and questions and answers about the scale.

WHY IS IT RELEVANT?

Value tools are increasingly being used by third parties to assess the relative value of oncology medicines. Every tool has strengths and limitations and as patient advocates it is our responsibility to ensure these tools adequately capture patients' needs.

HOW CAN I USE IT?

Use these resources to educate yourself and your team on the ESMO benefit scale, its methodology and the way that ESMO is positioning the tool and its outputs. It is important to understand the functioning of the tool in order to be able to judge whether they capture patients' needs and preferences.

WHERE CAN I FIND IT?

http://www.esmo.org/Policy/Magnitude-of-Clinical-Benefit-Scale

IAPO WORKING WITH STAKEHOLDERS TOOLKIT 12

WHAT IS IT?

From the International Alliance of Patients' Organizations (IAPO): This toolkit has been designed to support patients organizations to develop effective relationships with key stakeholders. It contains information and tools to help understand and overcome challenges that can be experienced when working with external parties.

WHY IS IT RELEVANT?

This toolkit lays down the basic frameworks for interacting with other stakeholders. It covers working with other patient organizations, medical associations, governments and the industry. It has roadmaps showing different stages of some of the engagement approaches and simple checklists to consider.

HOW CAN I USE IT?

If you are looking for partners to work together on an issue and this is the first time you or your organization have formed these kinds of collaboration, then this toolkit provides you with the basic background you need to plan those partnerships and collaborations.

WHERE CAN I FIND IT?

https://www.iapo.org.uk/sites/default/files/files/IAPO%20toolkit%20-%20Working%20with%20partners%20and%20stakeholders.pdf

13 INSIGHT INTO PATIENT ACCESS TO CARE IN CANCER (2015)

WHAT IS IT?

From the Cancer Support Community: A detailed report looking into the experience of patients in the USA around access issues. It has findings on the experience across different insurance schemes and across multiple dimensions, including access to services, access to care and financial impact.

WHY IS IT RELEVANT?

For advocates in the USA, this report paints a very clear picture of the critical access barriers that patients are facing. For those outside the USA, this report provides a very good example of the kinds of issues that it is useful to quantify. The report also demonstrates the value of converting data into clear messages and graphics so that policy makers can instantly understand the impact of the evidence that you have generated.

HOW CAN I USE IT?

For those in the USA, this document can be used to highlight the access issues that some of your members may be facing, particularly at times when new proposals for healthcare delivery and access are being debated. For those outside the USA, this report can be used to inform your own approach to access advocacy.

WHERE CAN I FIND IT?

http://www.cancersupportcommunity.org/sites/default/files/uploads/our-research/presentations/access-to-care/insights_into_patient_care_march_2015.pdf

14 ACCESS TO CARE IN CANCER 2016

WHAT IS IT?

From the Cancer Support Community: A more recent report that expands on the access issues faced by cancer patients in the USA by including additional data on the impact of cost containment strategies and the lack of discussion to patients on the healthcare costs of treatments being proposed by the providers.

WHY IS IT RELEVANT?

For those advocates in the USA, this report provides a timely reminder of the impact of cost containment and financial burden on cancer patients and their families. For those outside the USA, many of the issues highlighted in this report also occur in other countries, such as the pre-authorization of access to medicines and the requirement that patients may have to experience unsuccessful treatment on a range of therapies before being offered more recent alternatives.

HOW CAN I USE IT?

For those in the USA, this document can be used to highlight the access issues that some of your members may be facing with restrictive practices of the insurance plans. For those outside the USA, this report provides a guide to the kinds of data that could be collected locally as well as an example of the use of infographics to make detailed arguments.

WHERE CAN I FIND IT?

http://www.cancersupportcommunity.org/sites/default/files/uploads/policy-and-advocacy/patient-access/csc-access-to-care-barriers-challenges.pdf

LIBRARY OF ACCESS RELATED VIDEOS 15

WHAT IS IT?

From the Cancer Support Community: A library of interviews and documentaries, all focused on access issues. These include presentations, interviews, patient stories and full documentaries.

WHY IS IT RELEVANT?

In the digital age of advocacy, it is important to have a variety of methods to communicate. Videos can be used in meetings with decision makers and stakeholders and also form an important part of today's social media landscape. Videos also allow you to connect with your patient communities and other stakeholder groups in a way that is more emotive than a written report.

HOW CAN I USE IT?

For those in the USA, these videos are an existing resource, in the public domain, that can be shown to your team, to stakeholders and your members to highlight the current debates and impact of access issues. For those in the rest of the world, these videos are a good example of how complex access issues can be distilled down into video form.

WHERE CAN I FIND IT?

http://www.cancersupportcommunity.org/policy-advocacy/cancer-policy-institute-videos

OTHER RESOURCES TO CONSIDER

HTAI PATIENT & CITIZEN INVOLVEMENT

An international group of patient organizations, researchers, HTA bodies and industry members devoted to improving the quality of HTA decisions by promoting more patient involvement into the decision making.

Contains a library of education resources and tools for both patient organizations and HTA bodies.

http://www.htai.org/interest-groups/patient-and-citizen-involvement.html

EUPATI TOOLBOX

A toolbox of education for patient advocates on the medicines development process and HTA. This online resource starts with the basics of medicines development and then breaks that down into modules of more detailed education that helps advocates gain the knowledge they need to engage with researchers, industry and decision makers. Use this toolbox to look for education for yourself and your colleagues and review the HTA section for an overview of HTA methods and processes.

https://www.eupati.eu

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