



A³ ADVOCACY ACTIVITIES IN ACTION THE DATA ISSUE

EVIDENCE-BASED ADVOCACY CASES FROM
THOSE USING AND GENERATING DATA

TIPS AND RESOURCES TO ACCELERATE
YOUR USE OF EVIDENCE



Global Oncology Advocacy Leaders
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DATA & ADVOCACY

THE TIME IS NOW

Evidence-based decision making is at the center of health policy. Decision makers need the evidence to make changes to the status quo and advocates are often leading the way in developing, using and presenting this evidence

The days of patients being thought of as passive participants in research are largely over. Increasingly advocacy groups are taking center stage in co-developing approaches that generate the evidence needed to support decision making. This is bringing a new dimension to the evidence regulators, payers, industry and local healthcare systems consider when making their choices.

In this A³ resource, we take a look at six very different case studies that explore the way advocates are leading the way. These cases have been written to uncover the thought processes behind the decision to follow a particular route and, where possible, details of some of the overall steps have been included to give you a guide to the process they have followed.

Some of these cases are about using data that already exists. This is an important consideration. Too often, resources have been spent generating data that is hidden or lost behind complicated websites. Advocates can often see the value in this data and have the skills to be able to present it in a way that is usable by others. So, having an impact does not always mean generating your own data. Thinking clearly about your objectives, however, and how data and evidence can support your goals is critical.

We have two very different cases that discuss the role of patient organizations in larger consortia. Increasingly,



TOP TIPS

1. Each example shown in this A³ resource will have a range of tips to consider when thinking about a similar approach

the challenges of solving today's healthcare problems requires many different stakeholders to work together. That is why these consortia approaches are becoming more common. Being involved in a larger consortium can help to up-skill an organization in a new area, or it can help achieve policy objectives with the support of many other disciplines.

We also have several cases that discuss the use of patient registries to generate or use the data that is needed. In one case, we explore the use of a patient registry to impact a decision on access to treatments. In this case, the registry already existed and the case details how the patient organization used the information in a focused way. In the second registry case, we hear how advocacy groups are well placed to spot the challenges and unanswered questions that a rare cancer poses. Here, the organization has embarked on an ambitious project to develop a completely new patient registry.

There are also cases that explore health policy change using evidence where we hear from those that have used the evidence they have been gathering to impact laws.

Six cases are not nearly enough to describe the breadth and range of advocacy involvement with data and evidence. Consider these as 'tasters' that demonstrate just some of the possibilities.

Towards the end of this A³ resource there are a couple of quick 'how to get started' pages. These have been written for those of you that are just starting this journey and contain tips on how to write a survey and some guidance on how to become involved in a consortia.

Finally, the resource section at the end of this book provides links to background information or initiatives that you may find useful. Special thanks go to all the members of GOAL who have made this book possible.



Each of the experiences in this A³ resource will point to some independent tools, guidance or explanations that can help other advocates get started or learn more about the use of data. Look out for this icon to see relevant tools at the back of this resource.

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CREATING A GLOBAL **ATLAS OF DATA** AT THE GLOBAL LUNG CANCER COALITION

Jesme Fox of the Global Lung Cancer Coalition (GLCC) and the Roy Castle Lung Cancer Foundation explains how making existing data accessible can create a resource that advocates and healthcare professionals can use to advocate for better care and policy change

Evidence is needed to shape policies, and perhaps one of the most useful forms of evidence is that which shows the differences between outcomes patients can expect between one country to another. This kind of evidence can be used to advocate for more resources, changes in care models or implementation of new policies. That is the idea behind the Global Lung Cancer Coalition's 'Global Lung Cancer e-Atlas'. Jesme Fox, Medical Director at the Roy Castle Lung Cancer Foundation and Secretary of the GLCC explains the process of developing the atlas.

"We knew that this kind of comparison data can be extremely powerful as it enables us to ask important questions about the differences we see in survival rates, outcomes and other measures from place to place," Jesme explains.

This was not the first time Jesme had been involved in a project like this. In the UK, the Roy Castle Lung Cancer Foundation had used publicly available audit information to develop a similar resource that mapped the outcomes and measures that the UK's National Lung Cancer Audit had been collecting. "One of the key lessons we learned through this process is that there is a lot of good data out there," Jesme explains. "It is not always necessary to generate your own data."

In the UK, the National Lung Cancer Audit collects a variety of data on the care of people with lung cancer and the 'Smart Map' pulls that data into a map so that people can see the figures for their own region of the

UK. The challenge with this audit data was that not many people knew that it existed, and it is not presented in a format that people can easily use and share. "One of the goals we had with the UK project was to make that data more visible and easier to use so people could actually compare between regions in the UK," says Jesme.

It is not just patients and advocates that benefit from this approach. "A lot of healthcare professionals use the Smart Map and e-Atlas because it is a lot easier to extract the data from our resource than from the original sources," Jesme says. "The e-Atlas has been designed to make comparison between countries very easy and it even exports these directly into slides, so it is simple for people to use the data in their presentations at conferences and other events."

When the GLCC started to think about the need for a global resource to enable comparisons, the team suspected that the data would already be available in the public domain. "We knew there would be differences in survival rates from country to country but we also wanted it to show other measures that would help advocacy such as the implementation of policies and registries that show commitment from governments and healthcare systems. So, we knew that our first step would be to see what data was out there," says Jesme.

So how does an advocacy organization start a project like this? "The first thing is to realize that we do not have the capacity and resources to do this ourselves," explains



Jesme. "The GLCC e-Atlas is huge. There is a wealth of data out there, but we needed someone to really audit that data and find the common measures in the data that would allow us to compare country to country."

Recognizing that outside help was needed, the GLCC commissioned a specialist agency to project manage the development and to conduct the audit of available data. "The agency conducted a massive search to see what data was available, and then we at the GLCC could decide what would actually go into the e-Atlas," Jesme says. For example, mortality data was included for all countries and where available survival data was included too.

The GLCC was looking for more than just clinical outcomes data. They also wanted to include information on policy-related issues and resources. Information on national cancer plans and registries as well as whether a country had implemented the WHO Framework Convention on Tobacco Control was also included. "This kind of data is a lever that the advocates can use by asking their

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TOP TIPS

1. Look for existing data before deciding to generate your own
2. Use external expert agencies to perform the audits under your direction
3. Spend time up-front locking down the comparisons wanted and the functionality needed
4. Don't forget to plan for regular updates to the data and the functionality of the tool

1 Global Lung Cancer Coalition - e-Atlas

2 Roy Castle Lung Cancer Foundation - Smart Map

governments why there is no cancer registry or why there is no implemented cancer plan in the country," explains Jesme. So the GLCC members began collecting this kind of information to add to the e-Atlas.

Once the data had been gathered, work could start on building the interactive map. This was a big task and once again, the GLCC realized that they would need expert help. "We found web designers who could build this and then an agency who could import all the data into a format that would work," Jesme explains.

This process was not without its difficulties. "The idea of being able to compare countries in the e-Atlas seemed like an easy thing to do and was an idea that we had during the development of the project," Jesme says, "but it was difficult to implement and because we had not thought of this from the beginning, it meant that the data had to be reformatted to make this work."

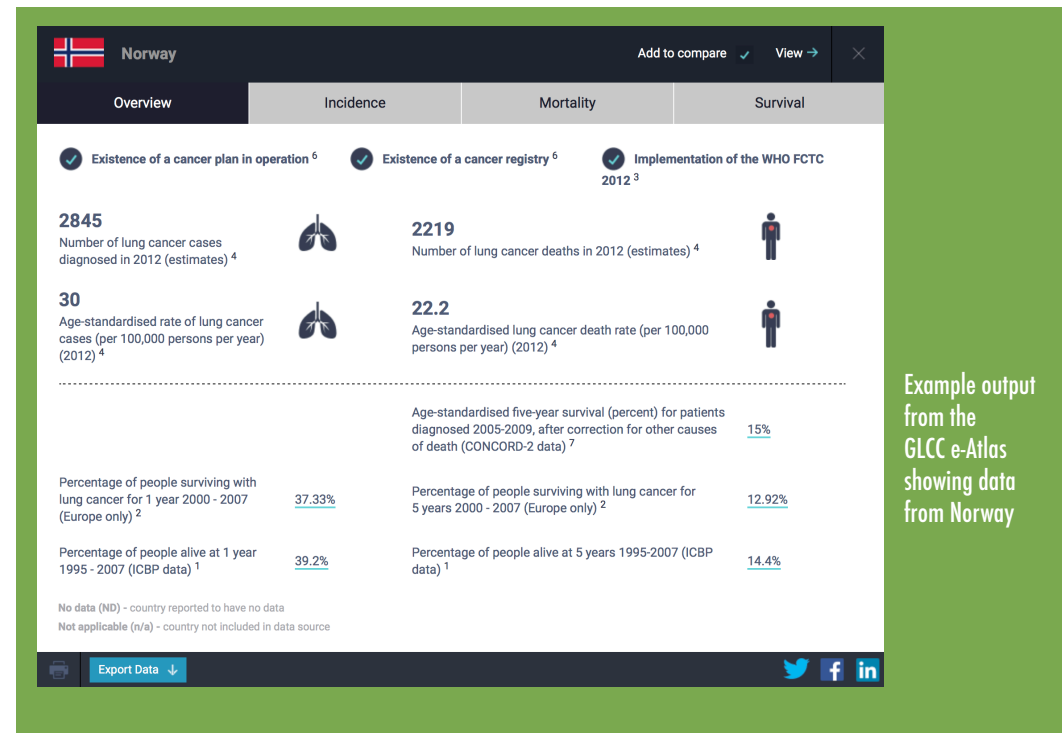
Another function that proved difficult to implement was exporting the data into slides. "We found that it was relatively easy to output graphs from the data, but much more complex to automatically turn these into slides, and yet we knew this was a function people wanted."

With the data and functionality developed, the final task before launching the resource would be to host it on the GLCC website. "The e-Atlas is huge in terms of the resources that sit behind it, and we actually had to rebuild the GLCC website so that it could cope with it."

By working closely with the experts and agencies who were building the resource, the GLCC did manage to implement all of the functionality that they wanted, but it was a lesson from which Jesme thinks others should learn from. "If we were doing this again, I think that we would spend more time up-front being very clear on the comparisons that we would want to see in the final resource and the functionality that we would want it have. In that way we would have avoided some of the changes that were needed during the actual build of the resource."

The final consideration that Jesme thinks it important to keep in mind is that these kinds of resources are only useful if they are updated with the latest data. "This is a big piece of work and must be accounted for in our work-plans," she says. "For the e-Atlas, these are based on big international data sets and we know that some of these are being updated this year. So we must plan and allocate resources."

By working together across the GLCC member organizations the e-Atlas was developed and launched within one year. "This was a real achievement for us," Jesme says. "And it was critical to have the right partners in terms of the agencies that could audit the data and build the website tool, we could not have done this without them. We have our day jobs and simply couldn't have managed this ourselves." Jesme estimates that the cost to create the e-atlas was around €90,000 (\$100,000).



STANDARDIZING ANALYSIS OF PATIENT REPORTED OUTCOMES RESEARCH

Patient Reported Outcomes (PROs) have become a standard part of clinical trials, but the way the data from PROs is analyzed can affect trial results. To address this problem, the European Organization for Research and Treatment of Cancer (EORTC) built a multi-stakeholder coalition to make recommendations on standardizing these processes. Kathy Oliver of the International Brain Tumour Alliance (IBTA) and Andrew Bottomley and Madeline Pe from the EORTC explain why a standard approach is needed and how advocacy involvement has helped.

Patient Reported Outcomes (PROs) have become a major tool in understanding the impact of disease and its treatments on patients. Through the use of PROs, patients, physicians and healthcare systems can gauge the relative impact of different care approaches on a patient's functioning, symptoms and quality of life. Many standard PRO tools are available to collect this data, but at the end of a trial, it is the job of the statisticians to analyze and interpret this raw data. It is this process that those at the EORTC started to worry about, because, as yet, no international standard method exists for analyzing the results from these different PRO tools.

are requested by the journal to change the way we have analyzed the results from the PROs. This was making our statisticians nervous as they know that this can affect the results. But, it was when we saw two very similar studies for a particular cancer that had the same population, the same PRO measures, over the same time-frame, but with different results that we realized that the only major difference between those studies was the analysis methods being used."

"We have conducted hundreds of clinical trials over the years," says Andrew Bottomley, Assistant Director and Head of the Quality of Life Department at EORTC. "When we publish the results of these trials, we often

"That's why the idea of standardizing the analysis methods became such a hot topic within our organization," the EORTC's Madeline Pe explains. "But what we knew from the start was that we would need multiple disciplines and stakeholders to come together to work on this, and we also knew that this must include those from the cancer patient advocacy community. That's why we reached

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PROlearn information resource on PROs



Peer reviewed paper: Analysing data from PRO and QoL endpoints for cancer clinical trials: a start in setting international standards





out to Kathy Oliver of the IBTA as well as several other groups," Madeline says.

"The project is called 'Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints Data for Cancer Clinical Trials' (SISAQOL)," says Kathy Oliver of the IBTA. "In 2016, when I was first approached by the EORTC, I was intrigued to learn more."

Instinctively, Kathy realized that this was an important project partly because of the high caliber of partners already involved. "PROs are a crucial part of the evidence that we, as advocates, support very strongly, and I could tell from the partners in this consortium that this would be a group that could only be assembled for something of high importance, and so I jumped in, knowing that I was about to embark on a steep learning curve, as I am not a statistician."

"After the first meeting, it really brought home to me why we, as advocates must always be involved in these kinds of projects," Kathy adds. "When you are looking at quality of life evidence and patient reported outcomes, and in order to get the most accurate, robust answers from clinical trials, it's vital to be able to compare the results from one clinical trial with those from another. Yet the lack of standards in analyzing this type of data makes this very difficult to do."

Kathy began to reflect on all the ways that quality of life and PRO data can affect the way that clinical data is interpreted and used. "PRO and quality of life evidence affects our ability to compare different approaches. It can affect the labeling of particular treatments. It can

certainly have an impact on clinical guidelines and health policy" says Kathy, "and so I started to realized just how important it is that we work together on creating standard approaches."

Surely working on a project that is basically about complex statistical methods would be daunting for a patient advocate? "Although I am in awe of statisticians, the experience has not been intimidating," Kathy explains. "The people at EORTC such as Madeline and Andrew have given me so much time and attention to help me understand some of these very complex issues."

Being involved in the consortium has helped illustrate to Kathy where she was adding value. "I would be asking questions such as 'how does this affect the patient community?', or 'can you explain that in lay language so that patients can understand what you mean?'" These questions helped to focus the outputs of the consortium and encouraged the group to think hard about how it communicates the issue and the deliverables of the consortium to the wider community.

From the EORTC side, the involvement of patient advocates such as Kathy has provided real value. "We knew from the beginning that we needed to capture the patient voice in what we were doing, and we reached out to Kathy as well as other groups to do this. Kathy immediately understood the value of the project and the need to bring in the patient community. In particular, we always have to be very clear in the question we are testing, the hypothesis, of our research. Kathy was able to bring a clear perspective of this for us," says Andrew.

"This starts by asking really good and clear questions, and challenging each other on what our objectives should be," says Madeline. "This is an area where Kathy has been really helpful. For example we developed a classification of concepts for our research objectives. We call this a taxonomy. We cannot do this without first understanding what the patient perspective is. For example, how would a patient define an improvement in their condition? It is important that we understood this within the project."

Kathy also spotted insights from the project that would be very good to take back into the patient community. "Through involvement with the SISAQOL initiative, I understood how important it is to reduce missing data in PRO and quality of life questionnaires. The more that I learn about how this missing data can affect trial results, the more I realize that the patient community should be aware of this and take action to minimize this," Kathy says.

"In response to this challenge, Madeline Pe from EORTC and Professor of Biostatistics and Oncology, Jeff Sloan, (Mayo Clinic) created a workshop for the IBTA biennial World Summit of Brain Tumour Patient Advocates last October in London," says Kathy.

Running a mock clinical trial for the Summit participants enabled the advocates to 'experience' participation in a trial and allowed Madeline and Jeff to highlight the challenges of missing data in PRO tools by simulating a very rushed completion of a quality of life questionnaire. This allowed participants to explore the issue of missing data and led to discussions about how advocates can help by getting involved in the design and implementation of clinical trials.

"Patient involvement in our work is here to stay," says Andrew. "It is high time that patients have a voice. People like Kathy can say 'you are wrong' and we have had that discussion and some wisdom in the discussion. If any of this is going to stick then we need as many people

TOP TIPS

1. Before joining a consortium look at who else is involved to get a sense of who you will be working with
2. Don't be afraid to ask basic questions; if you do not know what something means then most patients will not either
3. This is not a one-way dialogue; bring the knowledge you are gaining back to your patient community

involved as possible. We need to show that we have patients involved from the start."

So far, the project is about three-quarters of the way through and the work has revealed that much more needs to be done. "We aim to finish the key work by September this year where we will ratify our recommendations," says Andrew, "but we want to go much further."

The plan is to gain funding for the next phase of the project where Andrew says that patient participation will be strengthened further. "We would definitely look to have more patient groups involved and have at least one work package led by a patient organization.

For Kathy, the experience so far has been very rewarding and not overly time consuming. "The patient perspective has been listened to and respected throughout the SISAQOL process, with many of our inputs acted upon," she says. In terms of the commitment, EORTC covered expenses for attending meetings and Kathy estimates that her time spent on inputting into the published papers and conference calls would add up to a couple of days of her time per quarter plus any face-to-face meetings. "But for me, the time has been well spent," she adds. "This is not just talking about science, this is touching on a whole range of issues that can greatly affect people with cancer."

SECURING CHOICE FOR PATIENTS USING DATA FROM A REGISTRY

Deborah Maskens of the International Kidney Cancer Coalition explains how Kidney Cancer Canada was able to present evidence to a Health Technology Assessment body that led to the removal of a restriction on access to a medicine for kidney cancer

Often, when a new therapy arrives, limited, or no evidence exists on how it compares to other, recently available therapies. This can cause problems when Health Technology Assessment bodies make decisions about which treatment to grant access to. In Canada, Deborah Maskens, co-founder of Kidney Cancer Canada explains that this caused particular problems for patients trying to access a new therapy option for kidney cancer.

“The initial problem we had was that one therapy had already been granted reimbursement for second line treatment of kidney cancer, then a new drug came along with a different mode of action but no head to head data comparing it against that first therapy. So there was this uncertainty in how the two drugs stacked up against each other,” explains Deborah.

Without this head to head data, the health technology agency, CADTH, decided to stick with its original reimbursement recommendation for the first therapy and to restrict access to the newer therapy unless patients had shown an intolerance to the first one.

“This was a recommendation that we, as advocates were against, and the clinical community were against,” says Deborah. “To us, this was not an evidence-based decision, it was based purely on the timing and sequence of the launch of these two drugs.”

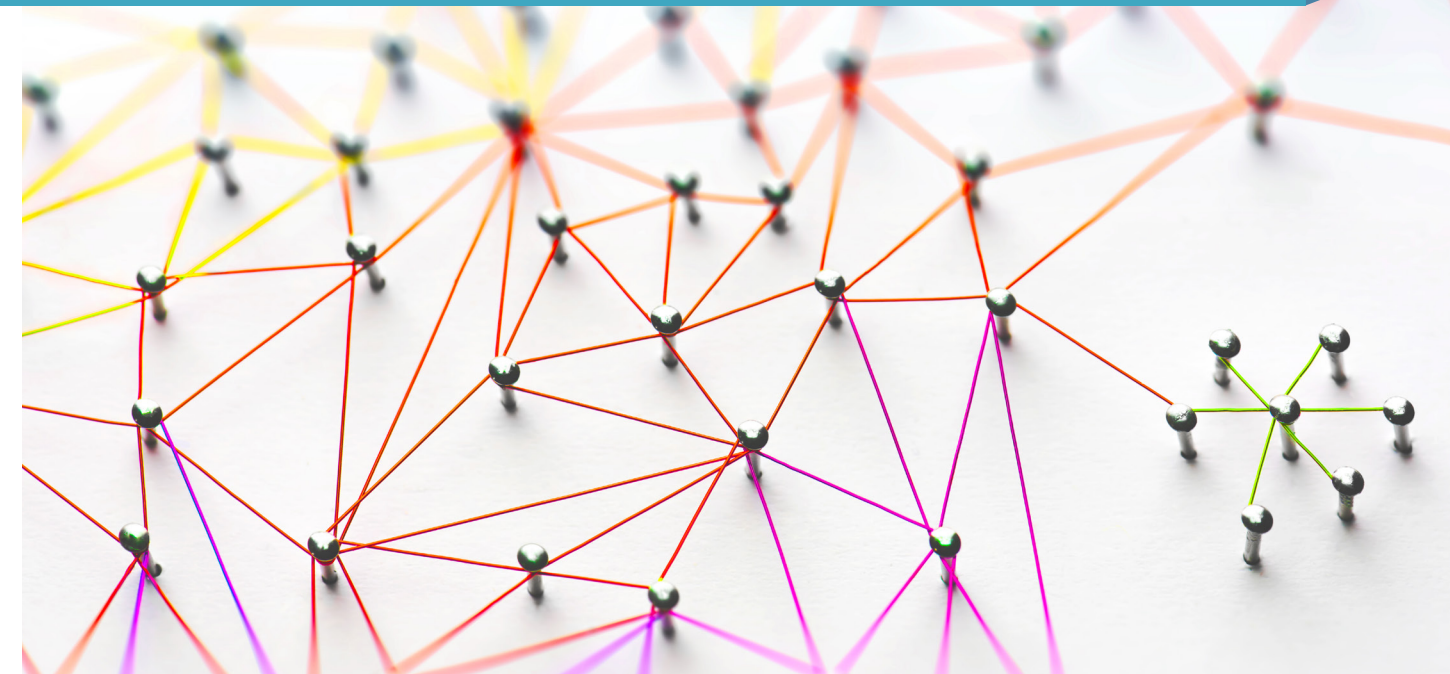
Of particular concern to Deborah and the advocacy community was that this decision, if implemented fully, would force some patients to go through an extra line

of therapy unnecessarily. “These two drugs had different modes of action,” Deborah explains. “From a patient perspective, you want to choose a second line therapy that makes most sense based upon your experience with first line of therapy. You and your physician may decide to stick with a certain mode of action if you did well in first-line, but may decide to switch to an alternative mode of action if your first-line experience was not so good.”

The other challenge with forcing patients to go through an extra round of therapy is that it could have implications for a patient’s eligibility to take part in future clinical trials. “For us, this was completely unnecessary and we needed to see how the provinces in Canada would interpret this decision from the HTA body.

“The provinces read this decision very differently from each other. Some allowed this as a true choice, so that patients and their physicians could select one or other of these two options. Others followed this recommendation to the letter, needing to see medical evidence that a patient cannot tolerate the reimbursed drug before they were allowed to try the newer one. In one province they were even demanding that patients see a second specialist to prove this intolerance,” Deborah says.

Hope for a change came when the team at Kidney Cancer Canada received notice that a process called ‘Request For Advice’ had been initiated to look into this issue. “Request For Advice is a specific process that can be initiated when drug plans in the provinces have difficulty implementing the recommendations of the pan-Canadian HTA agency.



Those stakeholders that were involved in the initial review of these two drugs were alerted by the agency that this Request For Advice was underway and could submit a response,” Deborah explains. “But, we only had ten days to prepare our arguments and input into this process.”

So, Kidney Cancer Canada went through an intense period of deliberation on how they could make their arguments clear. “We could have gone with the option of writing a strongly worded letter that gave the narrative of why the evidence did not support the current restrictions on access to this medicine. This is an approach that has been used many times before, but we know that these kinds of narratives are not seen as strong evidence by those making the decisions.”

So, the team at Kidney Cancer Canada looked for other ways that they could generate new evidence that would demonstrate that these two drugs should be given equal status in terms of reimbursement and access. Six years ago, the Canadian Kidney Cancer Information System (CKCis) was set up as a web-based national registry to support the development of clinical and basic research in kidney cancer. “We have been part of the Canadian Kidney Cancer Research Network (kcrnc.

ca) and this project since the beginning. To secure a government grant, the patient organization committed 50,000 Canadian dollars of funding at the very start and continues to contribute annually,” Deborah says. “CKCis is more than a database, it includes tumor and blood banking from many of the participating centers, along with data variables specifically of interest to the patient organization.”

This database continues to grow, currently including rich data from over 9,000 patients treated at major cancer centers across Canada. The information is imported directly from the patient’s EMR (Electronic Medical Record), and supplemented with data entry of additional fields, tracking patients’ entire care pathway from diagnosis to death, so there is a gold mine of information of significant interest to researchers and patient organizations alike.”

“The challenge was that this database has a very long list of research projects that were queued up, and an unexpected request for HTA purposes was not one of them, and so we needed to work closely with the research teams to elevate this issue. This is not something that we could do on a regular basis, but we had a good and close

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TOP TIPS

1. Do not rely solely on narrative patient stories to input into reimbursement and access decisions
2. Forge strong relationships with the research community, you will need each other
3. Find out what data infrastructure is available in your country and how it can be tapped



relationship with the team, who could see the benefits of lifting restrictions in second line therapy," Deborah says.

The team at Kidney Cancer Canada explained to the researchers that they were looking for evidence that could show the equivalence of these two second line therapies. "We knew that there would be people in the database that had one of these two drugs, and what we hoped was that there would be enough data to show the decision makers that in the real world context, these two drugs demonstrated similar or equivalent outcomes," she says.

Fortunately, the researchers recognized the importance of this question and were able to pull the data that Kidney Cancer Canada needed. "This took an enormous amount of work from the researchers in a very short space of time," Deborah explains. "That shows how important it is to build and maintain a really close working relationship with the research community."

Reflecting on the amount of work involved, Deborah stresses that these kinds of opportunities to input into a decision making process such as this 'Request For Advice' come with no warning. "The HTA agency doesn't pay for us to do this work, it is all expected to be voluntary. So we have no resources assigned to this," she says.

Besides the time of several researchers, one person from Kidney Cancer Canada gave an enormous amount of his personal volunteer time as a Board Director to make this happen within the 10 day time-frame. "This became priority number one and a mammoth task for a small group of people who decided to do this because it had the possibility of changing the reimbursement criteria for the better."

The result was successful. The evidence presented from the database convinced the decision-makers to change their position. "The recommendation now is that these two drugs could be considered equivalent in terms of access and reimbursement. This was a real victory," Deborah says. "And, the experience is leading us to think about how we can use this kind of real world evidence more often."

"I think this is where we all need to head towards," says Deborah. "Evidence-based advocacy is what is increasingly needed. As advocates, we need to go beyond making statements about what patients want and need from an emotional, moral, or ethical standpoint. We must be prepared to bring forward stronger, evidence-based arguments that address increasing uncertainties in HTA decision making."

EVIDENCE TO DRIVE POLICY CHANGE

Kristen Santiago of the Cancer Support Community explains how making policy change is enhanced when you have the evidence to support the need for change.

The Cancer Support Community (CSC) has been collecting evidence since 2009 on a range of issues that affect people living with cancer in the United States. In 2017, the group released their second index report detailing the impact that cancer is having on quality of life, risk of depression and financial impact among many other issues. Kristen Santiago, Senior Director of Policy and Advocacy uses this kind of data to convince policy makers to take action to best support patients, survivors, and caregivers.

"One of the key issues that we have been looking at is the need to provide more support to people with cancer in terms of psychosocial services," explains Kristen. "Many patients, from those who are newly diagnosed to patients who have been living with cancer for years can experience distress. This has been documented in peer-reviewed literature as well as in our Cancer Experience Registry. We know that high levels of distress can negatively impact patient outcomes."

The National Academies of Medicine, formerly the Institute of Medicine, stated that 50% of people with cancer experience levels of distress. Research has demonstrated that asking people about how they are coping and assessing their needs is an important first step in identifying important concerns, which can lead to improved quality of life, the ability to stay on treatment and overall patient satisfaction.

The Cancer Experience Registry includes a validated tool called the CancerSupportSource® to screen for distress that examines 25 different concepts related to psychosocial, practical and physical needs. This tool can help identify concerns that need to be addressed early on in a diagnosis before they become a barrier to care. This screening tool is also administered through CSC's Cancer Support Helpline and at their Affiliates nationwide."

CSC was interested in having a process where distress screening and appropriate support was provided within

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clinical trial protocols, and to have more emphasis on collecting psychosocial and patient experience data in clinical research.

“We know that the social and emotional impact of cancer is undeniable, but what is perhaps more compelling to the research community is that distress related to cancer can also negatively impact a patient’s adherence to a clinical trial and to their medications,” Kristen says. “So we embarked on two parallel strategies to change the policy landscape to encourage more support for patients and the collection of more evidence so that we can understand the impact on patients.”

Kristen outlines the problem that the CSC is trying to solve. “If clinical trials and other forms of research are not looking at the psychosocial impact of disease and treatment, then how do we know that they are measuring the outcomes that really matter to patients?”, Kristen says. “We want to work with legislators, regulators, researchers, and the industry to make sure that we are both capturing this evidence and supporting patients through the process.”

To make this change, the CSC embarked on two separate initiatives. The first of these was to change the definition

of ‘patient experience data’ used by the Food and Drug Administration (FDA) so that it encompassed the psychosocial impact of a condition, therapy or clinical investigation.

“The FDA had previously focused on just the physical impact on patients, and we knew that this was not enough. The legislation that governs the U.S. Food and Drug Administration (otherwise known as The Food and Drug Administration Reauthorization Act of 2017 (FDARA) gave us an opportunity to redefine this definition,” says Kristen.

“We know that to comprehensively evaluate the real-world impact of cancer therapies, it will be essential to capture the full extent of patients’ concerns such as disruptions to family and work life and the logistical and financial issues they experience,” Kristen adds.

The inclusion of language in the FDA’s definition of patient experience data would facilitate this kind of evidence being generated. “So, in 2017, after assembling the evidence needed to demonstrate this need, we were successful in advocating for this change in FDARA,” says Kristen. “We are now working with the FDA to ensure they use this expanded definition in all their guidance, documents and processes.”

The hope is that now that this definition is in place, researchers will begin to collect patient experience data that can inform future decision making. “That will help us build the body of evidence we need to better support people with cancer in the future,” says Kristen.

The second element of the CSC’s policy efforts has been directed at supporting legislation called ‘The Patient Experience in Research Act’ which would create an environment that would require the pharmaceutical industry to evaluate, document and follow-up on the social and emotional factors experienced by patients during clinical trials. The act calls for a pilot project that would invite three manufacturers to collect patient experience data to further understand how patients are living with their disease and are impacted by the treatment.

“Evidence was a major part of our argumentation for drafting this legislation,” Kristen says. “Studies have shown, for example that in breast cancer, those patients who engage in social and emotional interventions have significant benefits from those who do not such as increased survival and a reduced risk of recurrence.”

“There is a huge difference for patients when they have to travel very frequently for treatment. The impact of this time out of their life is not being captured, but it would be beneficial for patients to know what kind of impact this is having on quality of life and psychosocial measures,” adds Kristen. “Ultimately, we want the evidence to guide patients so that they have an opportunity to make decisions based on the full range of treatment and related impacts.”

The process of enacting legislation such as this is a long one. “It starts with building awareness with lawmakers. We have built strong relationships over many years and have champions within the House and the Senate that we work closely with,” says Kristen. “Part of this is sharing the data we have with them so that they are equipped with the facts that enable them to garner support among their colleagues.”

Then the team will have to work on a concrete solution that they can take to the lawmakers for feedback and adaptation.

“We have to build the solution, and that takes time. The lawmakers need to see something that they can work with and react to,” says Kristen. “For this act, we worked closely with Representative Diana DeGette and Representative Leonard Lance to draft the legislative language and are continuing to work with their offices, and their colleagues to build support for the bill. This whole process takes years - to build the arguments, draft the act and work with policy makers.”

“What has become clear to us working in policy is that the continued use of evidence to support the need for psychosocial evidence remains as important as ever,” says Kristen. “I think we, in the U.S., are a little behind the curve here. We hear cases from across the world where patient experience data is becoming part of decision making. We will continue to gather our own evidence on the need to support patients more, and arm our policy makers with this so that they can fight for a future where it is normal to collect this evidence in research and use it to create a better environment and support for people with cancer.”

TOP TIPS

1. Be realistic about the time needed to change policies or enact laws
2. Where possible, use existing processes and legislation to gain the changes you are looking for
3. Lawmakers demand evidence before they act so map the evidence you have and highlight any gaps that you will need to fill
4. Don’t be disappointed by setbacks, this is normal

FILLING THE GAPS IN EVIDENCE THROUGH DEVELOPING A REGISTRY

Rare cancers present researchers and the patient community with so many unanswered questions. In ocular melanoma, the team at CURE OM, an initiative of the Melanoma Research Foundation (MRF), have embarked on the development of a patient-reported registry to hopefully answer some of the mysteries that this form of melanoma exhibits. Here, Dr. Sara Selig of CURE OM and Kyleigh LiPira of the MRF explain why this is so important to them.

Dr. Sara Selig, Co-Founder and Director of CURE Ocular Melanoma (CURE OM) has been on a mission ever since her husband was diagnosed with ocular melanoma (OM) in 2006. "I didn't know anything about this disease, until my husband, Gregg, was diagnosed. Then, I made it my business to learn as much as I could," Sara says.

"What struck us immediately was the need for more research in this area, as well as the need for more support and education for the patient and caregiver communities, and the need for advocacy to support the entire field. So, we teamed up with the Melanoma Research Foundation and established CURE OM (Community United for Research and Education of Ocular Melanoma)," says Sara.

Unfortunately, Gregg didn't survive his battle with the disease, and this spurred Sara on to keep asking those basic questions about the disease. "While there are amazingly dedicated researchers in OM, there is still so much that we simply don't know," Sara explains. "The basic science and the causes of the disease still need to be identified, and there are questions about the patient experience, and how different approaches in managing OM drive outcomes. So, for more than five years, we have been discussing how to fill those gaps."

For Kyleigh LiPira, Chief Executive Officer of the Melanoma Research Foundation, CURE OM is exactly the kind of initiative that the MRF believes should be supported. "This is a type of melanoma that is devastating. Patients and families are looking for answers and in need of support. That is why we are committed to the CURE OM initiative and supporting OM research."

Although many rare disease research is supported by the use of registries, the idea of developing a patient registry for ocular melanoma was not an instant decision. Every year, CURE OM convenes annual conferences to bring together leading experts to look at the science and issues of OM, holding typically two meetings a year.

"At those meetings the same issues kept coming up," says Sara. "We were identifying the need for more information about the demographics of those with ocular melanoma, the need for tissue samples that could perhaps give us clues to its origin and molecular mechanism, and the outcomes from different approaches to managing and treating it - all leading us closer to effective treatments, and ultimately, a cure."

It is through these repeated discussions about the gaps in knowledge that Sara and CURE OM advisors began to realize that a patient registry is needed to start collecting the data to answer these questions. "There were lots of triggers to this decision, but perhaps the one that caught the public's attention was a group of people from North Carolina that developed the disease and all lived very close to one another. That brought up the very same questions on causality that we had all been asking ourselves over the previous years."

Dr. Marlana Orloff, a member of the CURE OM Registry Steering Committee, explains why finding out about the natural history of ocular melanoma has been so difficult. "Ocular melanoma, in addition to being a rare cancer, has a number of other features that make it extraordinarily difficult to track in the traditional state cancer registry



sense," Marlana says. "There are issues in the fact that most patients are diagnosed (and treated) out of their home state so not captured in their home state's registries. Also, there is often no biopsy or pathology diagnosis at primary presentation. Finally, there is not a good and uniform diagnosis code that incorporates pathology and location."

As these challenges were analyzed by the team and advisors, it became clear that the goal should be to have a 'patient-powered and patient-reported registry', one in which patients themselves help develop the registry and can enter their own data. In this way, the registry will help answer questions important to all stakeholders and be able to capture data from patients across state lines and across institutions, therefore overcoming a major obstacle in current registry approaches.

"With a patient-reported registry we would be able to capture information from patients wherever they live, and even across the globe. So, we are thinking big here. We would also be able to capture information that only patients know themselves, such as their experience over time, their preferences, their journey across and between centers," says Sara. "This is going to be inter-institutional

so that we can pool the data from multiple centers where patients may be getting their care. We will be able to look at data in a new, more comprehensive way."

Setting up a registry is a daunting task, and the CURE OM team are in the middle of the process. They secured funding to develop the concepts and conduct the planning, and that phase is almost over. Sara outlines the main steps she and the team have followed to get to this point. "We started by learning more about registries themselves. We hadn't appreciated the breadth and depth of registries at first. As we began to learn more, we had a real 'light bulb' moment where we realized we could achieve so much with this approach."

"Next, we found people who knew about registries and started asking a lot of questions, to really educate ourselves in the possibilities and trade-offs of various options. Through this, we were able to connect with someone with expertise in registries who could guide us through this, Jacqueline Kraska. We are delighted to welcome Jacqui onto our team," Sara adds.

The next step was to engage the community in fundraising efforts so that CURE OM would have the

Continued on next page...

financial support needed for the planning stage. "Our community fund-raisers have been vital," says Kyleigh. "The community have been the driving force behind this initiative. This is so important when developing something like a registry that can seem so abstract." Marlana agrees: "The ocular melanoma community is like no other! They are truly the most informed and engaged patient and caregiver group that you could imagine."

Another member of the registry steering committee, Chad Kimbler, a patient with OM, flags that it is also important that the steering committee is so diverse. "The steering committee represents the community in so many ways. It consists of patients, a research nurse, social workers, caregivers, and of course clinicians. With such diverse representation, we should be able to engage with the community and maintain the momentum needed."

With the steering committee formed, the next steps were developing the goals and objectives, getting additional input from the patient community and working out the components that this registry will have. It was important also to get input from a wide range of other stakeholders, such as academic partners and international collaborators, and investigate different technical partners.

"We are now at the stage of working on what data fields we should have initially. We have to be ambitious but also realistic. So, we are mapping out what we need at the start and what we will build on once the registry is up and running," says Sara. "The interdisciplinary steering committee is vital for this work, as we need to bring in

a variety of perspectives to map out our options." This takes time as Kyleigh notes: "Everyone feels both a sense of urgency as well as a commitment to a thorough process to create the best registry possible. While this has resulted in a time-line longer than we had initially hoped, researchers and staff are working at the fastest pace possible without sacrificing quality."

This is a grass-roots driven project, and so once the team have mapped out the data fields, the next task will be to communicate progress to all of those who have supported the project. "We have spent so much energy on this, and we want people to be excited about where this is going," Sara says.

Part of that excitement is driven by hope that the registry will help solve some of the mysteries around OM. "Of course, I would hope the registry finds a cure for ocular melanoma. Unfortunately, I don't think the cure will be as simple as having a patient registry, however, I believe the registry will certainly help direct and attract research into this cancer," says Chad. "In more broad terms, I hope the registry can help identify the epidemiology of the disease. Being a young adult in my 30's, I would like to understand the onset of when the cancer occurs and outcomes after onset, specifically in young adults like myself."

For Sara, this is a chance to put patients in the center. "I think when you have been impacted by rare disease like OM it can be an isolating experience. The registry allows us to address the gaps that we see and puts power in the hands of patients and family members - that's exciting!"

TOP TIPS - REGISTRY DEVELOPMENT

1. Educate yourself on the types of registries
2. Work with someone who has done this before
3. Work with your steering committee to identify questions you want to answer and develop the goals and objectives based on this input
4. Survey the patient and carer community as well as other stakeholders
5. Don't underestimate that this can be a costly endeavor ensure that you have funding
6. Engage the community often so that they understand what you are doing and can input
7. Have multiple disciplines and backgrounds feeding into the planning stage
8. Spend time talking with, and learning from, technical advisors and potential partners
9. You can't always include everything, be realistic about your start, and ambitious about where you want this to go

MAKING BETTER USE OF REAL WORLD EVIDENCE

Kawaldip Sehmi, Chief Executive Office of the International Alliance of Patients' Organizations (IAPO), explains the role of an advocacy group in a big international consortium to look at how real-world evidence can be used to enhance healthcare decision making and speed up the development of new medicines.

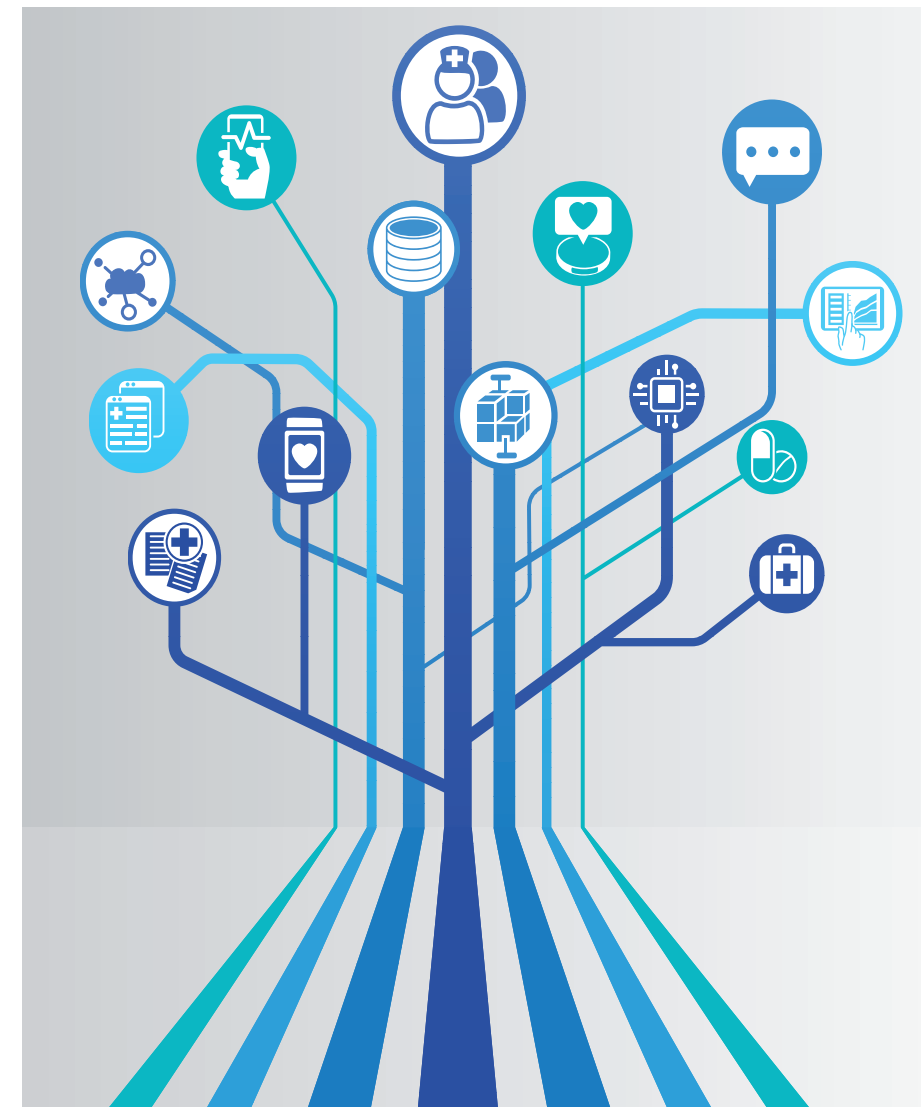
Real world evidence has become a pillar of evidence that has grown dramatically in recent years. However, significant questions remain on how real world evidence could be used earlier in research and medicines development, and how it could help inform more healthcare decision making. To answer some of these questions a large international consortium, called GetReal, was launched in 2013 and the International Alliance of Patients' Organizations (IAPO) were invited to join.

"We were invited to bring the patient perspective into the heart of the project," says Kawaldip. "Real world evidence is ultimately about understanding what really happens to patients outside of a controlled clinical trial, and those are the kinds of perspectives that we were keen to bring to the various parts of this three-year project."

Ultimately, GetReal was looking to generate a consensus on the best practice use of real world evidence in regulatory and reimbursement decision-making, as well as drug development. The consortium developed a range of tools and papers over its three-year duration that help stakeholders make better use of real world evidence.

"This was a big undertaking," says Kawaldip, "and part of the reason we got involved was

Continued on next page...



Step 1: Clarify the issues

This section helps to **gain a greater understanding of the potential issues** (or 'effectiveness issues') in demonstrating relative effectiveness for a medicine, including **actions** that can be used to explore the potential issues.

CLARIFY THE ISSUES

Step 2: Find RWE options

This section function provides **different study designs or analytical techniques** that could be considered **to address the issues** (or 'effectiveness challenges'), depending on the development stage of a medicine and the relevant PICO category.

FIND RWE OPTIONS

Directory of resources

Access to all webpages, such as background information on real-world evidence (RWE), sources and study designs providing RWE, analytical methods using RWE, and find related GetReal work and other authoritative sources.

READ MORE

The GetReal RWE Navigator

TOP TIPS

1. Understand the commitment needed and the skills required to input into a consortium
2. Seek input from your members on the topic areas to focus on
3. Link into policy initiatives that you may already be undertaking
4. Even if there is funding to cover this, know that you sometimes have to put more time in than expected

that, back in 2013, we were already thinking about how research and development could be improved to more closely meet the needs of the patients."

Like most big consortia, work was split into 'work packages' and IAPO became involved with two main packages. "The first work package was all about estimating the effectiveness of new medicines using real world evidence. The second area we focused on was looking at ways of generating and using real world evidence much earlier in the development of medicines," says Kawaldip.

"At IAPO, we had been really thinking about ways that the time to develop medicines could be reduced. The hope is that with shorter development times, new medicines can get to patients quicker and the cost of development will be lower," adds Kawaldip.

Another opportunity for real world evidence, is that it can be used to better understand the patient perspective, and ultimately demonstrate the patient-relevant value of a particular medicine or medical intervention. "What we did was bring that patient dimension into the discussions.

Patient preferences were missing and it was these kinds of concepts that we added to the consortium," recalls Kawaldip.

As part of this work, the consortium published a set of policy recommendations that includes a section on the broader involvement of patients in real world evidence and decision making. This called on patients to be involved at all stages of decision making process; that patients are considered as proper research partners; and that appropriate information is provided before, throughout and following the conclusion of the research process so that patients are informed.

"It is these kinds of policies that will ensure that patients understand and take part in real world evidence projects," says Kawaldip. "We also identified that there is more work to do to explain real world evidence studies in clear language so that people know how they differ from traditional kinds of research."

At the heart of the outputs from the consortium is the 'RWE Navigator' - an on-line tool that brings together the work across GetReal into a three main steps. This

tool makes it easy for others to find out more about real world evidence and its application to healthcare decision making. "You can play around with this tool to navigate to various issues that we, and the other consortium members worked on. You can see the published work of the consortium and you can even sign up to a course on real world evidence," says Kawaldip.

In terms of the commitment from IAPO, GetReal was co-funded by the European Union and members of the industry and so IAPO did not have to find additional funding to join. "We had a lot of people on this project at various times. There are meetings to attend and lots of conference calls," recalls Kawaldip. "Sometimes the project needed policy input, and so our policy officer would get involved, sometimes we were asking for input from our members and so we would pull in our membership officer. The list goes on."

"But the commitment has been worth it," says Kawaldip. "This is such an important topic that will shape the future of how research is done. They would have lost the most important insights, the patient insights, without our involvement. So I am proud of what we achieved."



GETTING STARTED SURVEYS

For those advocates and patient groups that are just starting to generate their own evidence through the use of surveys, lots of guidance exists on the best way to construct a survey. Most universities publish guidance for researchers that can be very useful, and some healthcare systems also provide guidance for those collecting healthcare surveys. Below are some of the most common areas to consider.

Objective (Purpose of the survey)

- Be clear on the objective before you write any questions. This may focus on a particular issue or target a particular group of patients
- It is always worth searching to see if a similar survey has already been conducted recently, this may mean that your survey is not needed

Drafting questions

- Involve patients, caregivers in the design and review of the questions
- Try to keep the questionnaire short - around 25 questions should be the maximum
- Concentrate on asking only one question at a time. For example, "Was our information leaflet clear and useful" is two questions, and so split them up
- Think about the order of the questions. People can be influenced by earlier questions in the survey. So try to put your general questions first and then lead onto any specific areas you want to cover
- Demographic questions are usually best left towards the end of a survey

Types of questions (open versus closed)

- Open ended questions allow respondents to answer in their own way. You'll get a greater variety of responses but it will require more work to analyze
- Closed ended questions require people to choose from a fixed list of responses and are easier to analyze

Testing your survey

- Test it with a few people who match the target group. Did they understand the questions?.
- Did the answers received match your expectations? If not, the questions may need to be clearer

Sending out your survey

- On-line and email surveys may work for most, but some vulnerable and hard to reach groups may need a postal survey or a telephone survey

Confidentiality and privacy

- If collecting personal data then be aware of local privacy laws that may apply, and be clear to your participants about how confidentiality is managed

Examples of guidance for further reading:



Harvard University
Tip-sheet on
question wording



Scottish Health
Council guide
to surveys and
questionnaires



Virginia University
Survey Design:
Getting the results
you need

GETTING STARTED GETTING INVOLVED IN A CONSORTIUM

Several of the case studies in this book include projects in which different organizations come together into a consortium. There are many types of consortia and many reasons for being a part of one. Perhaps the biggest opportunity for multi-stakeholder consortia is the Innovative Medicines Initiative (IMI), a project of the European Union. Below is a summary of their guidance which will be applicable to multiple other consortia opportunities.

Decide at what level you want to be involved

- In many consortia, there is an opportunity to be a full project partner or an advisory board partner
- A full project partner will be an equal member of the consortia with the other partners, and will carry more responsibility and commitment
- An advisory board partner will allow you to input the patient experience into a project but you will not be part of the project delivery teams

Letting others know you want to be involved

- Organizations such as the IMI publish a list of topics that are under consideration. Monitor those channels and when you see a topic that is of interest reach out to your network of other advocates and researchers to let them know that you are interested
- Start discussions with others that are also interested to define the role that you can play within the consortium
- Some organizations such as the IMI have a partner search tool to let potential partners find each other
- Social networking tools are also used to find consortium partners. The IMI has a dedicated LinkedIn group where information is distributed

Be clear on the commitment

- Being a full project partner can be a large commitment. Speak to others who have been involved in similar consortia to understand how they have experienced and managed this
- There will be contractual obligations to fulfill as a full project partner, so be prepared to handle the review and signing of a consortium agreement
- As a full project partner, there is often a role for you on the governance of the project, including being part of the steering group for the project

What can you achieve?

- Consortia are often formed to address issues that one stakeholder group cannot solve alone. These are often covering big topic areas that need the skills and knowledge of multiple partners
- Look at the outputs from consortia projects that have already finished their work to determine the kind of impact that these projects can deliver (see link to EUPATI below for an example)
- Identify patient group partners that have been involved in these projects and reach out to them to get a clear assessment of their role in the project and the impact that they believe their contribution made

Examples of guidance for further reading:



The EUPATI project



IMI portal on future
topics and open
calls for proposals



Innovative
Medicines Initiative
Patient Brochure

1 GLOBAL LUNG CANCER COALITION E-ATLAS

WHAT IS IT?

An interactive map that allows you to explore and compare the lung cancer data from countries across the world. The data includes mortality, and in many cases survival data, as well as policy related issues such as the existence and implementation of cancer plans.

WHY IS IT RELEVANT?

This is a good example of how existing data can be pulled together in a format that advocates can use to inform their health policy activities.

HOW CAN I USE IT?

If you are active in the lung cancer area then the tool can be used to help frame some of the policy initiatives that you may be planning. For those outside of the lung cancer area, the tool provides a good example of what can be achieved with existing data.

WHERE CAN I FIND IT?



<http://www.lungcancercoalition.org/e-atlas/>

2 RCLCF SMART MAP

WHAT IS IT?

This is a national map of lung cancer services for the UK. Like the GLCC e-atlas, this is a tool that pulls together existing data. However, because the data set is more comprehensive it contains more detailed statistics.

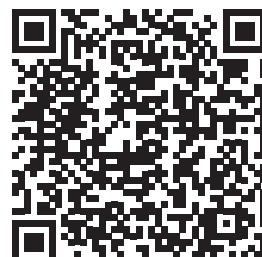
WHY IS IT RELEVANT?

Many healthcare systems conduct national audits of the care provided for particular diseases. This data is often hard to find or difficult to interpret. The Smart Map shows how this data can be pulled together and presented in a way that patients, advocates, clinicians and health policy makers can use.

HOW CAN I USE IT?

Use this smart map as an example to show your colleagues if similar data sets are available in your country.

WHERE CAN I FIND IT?



<https://www.roycastle.org/how-we-help/our-campaigns/improving-treatment-and-care/interactive-map>

UNIVERSITY OF BIRMINGHAM: PROLEARN 3

WHAT IS IT?

Freely accessible information about Patient Reported Outcomes measures (PROs) designed to explain PROs to those that are new to the area.

WHY IS IT RELEVANT?

Patient Reported Outcomes are now a standard tool used in clinical studies and other forms of healthcare research. Because patients are expected to complete these tools, it is important that advocates understand them and the main differences between different kinds of PROs.

HOW CAN I USE IT?

A section of the site is dedicated to PROs for patient advocates. This contains videos and other links that have been designed to help patient organizations get up to speed on the use of PROs.

WHERE CAN I FIND IT?



<https://www.birmingham.ac.uk/research/activity/applied-health/research/prolearn/patient-advocates.aspx>

PEER REVIEWED PAPER FROM SISAQOL 4

WHAT IS IT?

The first publication from the SISAQOL case study that outlines the rationale for standardizing the analysis of PRO and health related quality of life tools

Note: This is a paid-for publication

WHY IS IT RELEVANT?

For those wishing to understand more about the need for a standard approach to PRO analysis, this opinion piece gives a good grounding.

HOW CAN I USE IT?

Use this article if you are heavily involved in the development or use of patient reported outcomes measures

WHERE CAN I FIND IT?



[https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045\(16\)30510-1/abstract](https://www.thelancet.com/journals/lanonc/article/PIIS1470-2045(16)30510-1/abstract)

5 THE ROLE OF PATIENT-POWERED REGISTRIES

WHAT IS IT?

This is a white paper from the Agency for Healthcare Research and Quality in the U.S. that discusses the role of patient powered registries (those developed by advocacy organizations) and patient powered research networks.

WHY IS IT RELEVANT?

Increasingly patient advocacy organizations are spotting unanswered questions that the research community is not addressing. Many decide to develop their own registry to collect data to answer these questions.

HOW CAN I USE IT?

This is good background reading to understand how these advocacy driven initiatives are perceived and used.

WHERE CAN I FIND IT?



https://www.ncbi.nlm.nih.gov/books/NBK164513/pdf/Bookshelf_NBK164513.pdf

6 2017 CANCER EXPERIENCE REGISTRY REPORT

WHAT IS IT?

The Cancer Support Community has been running its experience registry for many years. This 2017 report details the latest results from the experience that people with cancer in the US are reporting.

WHY IS IT RELEVANT?

For those in the U.S., this report gives a very thorough overview of the issues facing people with cancer in the country. This report focuses on cancer-related distress, quality of life, treatment decision making, side-effect and symptom management, financial toxicity and work-related experiences.

HOW CAN I USE IT?

For those in the U.S., this information can be used to help support advocacy initiatives. For those outside the U.S., this is a good example of the kinds of experience data that can be generated from an experience registry.

WHERE CAN I FIND IT?



https://www.cancersupportcommunity.org/sites/default/files/uploads/our-research/2017_Report/registry_report_final.pdf?v=1

RARE DISEASE REGISTRIES GUIDE

WHAT IS IT?

From the Global Genes organization, this is an on-line guide to rare disease registries.

WHY IS IT RELEVANT?

This is a well structured guide with top-level detail. It contains sections on understanding registries, designing and developing registries as well as tips on ensuring a successful registry. There is also a useful resource guide for people to learn more.

HOW CAN I USE IT?

If you are thinking of starting a registry, this guide will explain some of the basics that you need to know.

WHERE CAN I FIND IT?



<https://globalgenes.org/toolkits/understanding-rare-disease-registries-2/resource-guide/>

NIH LIST OF REGISTRIES

WHAT IS IT?

A list of frequently asked questions about registries along with an A-Z index of some of the registries that have already been set up.

WHY IS IT RELEVANT?

Before deciding to start a new registry, it is important to check to see what is already out there. There are several lists of registries around the globe, this one is mainly U.S. based. So use this list as a starting point to see what exists already, but do search more widely than this list to be sure you have a complete picture.

HOW CAN I USE IT?

Use this list to identify registries that could be a useful resource for you, or as part of your due diligence before you commit to setting up a new registry.

WHERE CAN I FIND IT?



<https://www.nih.gov/health-information/nih-clinical-research-trials-you/list-registries>

9 REAL WORLD EVIDENCE NAVIGATOR

WHAT IS IT?

An educational on-line resource helping users find out more about the potential of real world evidence. Some guidance is provided and a directory of resources is included

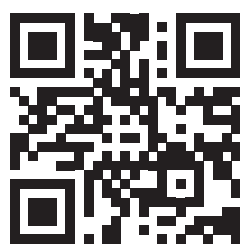
WHY IS IT RELEVANT?

A lot of the information in the navigator is technical, designed for those constructing real world evidence studies. However, sections of the Navigator do explore the role of patients and advocacy groups.

HOW CAN I USE IT?

The site is clearly laid out in steps and so it is worth taking a look at the content if you are starting to get involved with real world evidence projects.

WHERE CAN I FIND IT?



<https://rwe-navigator.eu>

10 PATIENT PERSPECTIVES ON REAL-WORLD EVIDENCE

WHAT IS IT?

From the National Health Council, this report of a 2017 round-table meeting clarifies the views of patients on real world evidence and explores the skills needed to make best use of it.

WHY IS IT RELEVANT?

For those groups that are getting involved in real world evidence projects, this provides a good summary of the issues to be aware of from a patient perspective. It makes good background reading and identifies some of the challenges.

HOW CAN I USE IT?

Use this as background reading to support any work that you may be doing in the area of real world evidence.

WHERE CAN I FIND IT?



<http://www.national-healthcouncil.org/sites/default/files/Patient%20Perspectives%20on%20Real-World%20Evidence.pdf>

FDA PATIENT FOCUSED DRUG DEVELOPMENT

WHAT IS IT?

A site provided by the FDA that outlines their approach to patient focused drug development, the need for patient experience data and the outcomes of recent FDA and other external meetings on this issue.

WHY IS IT RELEVANT?

Advocates are increasingly being invited to be part of drug development processes and it is useful to see what other organizations are engaging in this space.

HOW CAN I USE IT?

This is a site worth visiting regularly as the FDA are still developing their approach to this topic. The site also has links to external resources beyond the FDA which can direct you to other similar initiatives.

WHERE CAN I FIND IT?



<https://www.fda.gov/drugs/developmentapprovalprocess/ucm579400.htm>

EMA PATIENT AND CONSUMERS OVERVIEW

WHAT IS IT?

A site giving the overview and frameworks used by the European Medicines Agency to involve patients and citizens in their activities.

WHY IS IT RELEVANT?

As advocates and patients become more central to the development and generation of data, it is important to understand how the regulators are also involving the patients. This site gives a broad overview of this.

HOW CAN I USE IT?

There are links on this site which cover a wide range of issues plus a list of different committees and boards where patient representations happens. If you are interested in being involved in the EMA activities, this is a good place to start.

WHERE CAN I FIND IT?



http://www.ema.europa.eu/ema/index.jsp?curl=pages/partners_and_networks/general/general_content_000317.jsp&mid=WC-0b01ac058003500c

EVIDENCE BASED DECISIONS IN RARE DISEASES

WHAT IS IT?

A report that looks at the multi-stakeholder approach to improve the evidence base for decision making by regulators and Health Technology Assessment bodies in the area of rare diseases.

WHY IS IT RELEVANT?

Rare cancers represent a challenge in collecting and using the evidence needed to inform decisions. This report brings together various perspectives that look at this issue as well as suggesting areas for improvement. The report includes several examples of good practice

HOW CAN I USE IT?

If you are involved in rare cancers then this is a report that is worth reading to see how the multiple stakeholders view the need for patient experience data to be part of decision making.

WHERE CAN I FIND IT?



https://www.htai.org/file-admin/HTAi_Files/ISG/PatientInvolvement/v2_files/Resource/PCISG-Resource-HTAi-2016-Panel-Report-final-for-web-Sep2016.pdf

HTAi VORTAL

WHAT IS IT?

A library of peer reviewed articles in the area of patient involvement in Health Technology Assessment (HTA).

WHY IS IT RELEVANT?

This has sections on PROs, patient preferences, patient input and many other related categories. If you are working with HTA bodies and want to know more about the methods and evidence that they use, then many papers within this library that could help.

HOW CAN I USE IT?

Browse the library by category, focusing on the patient-relevant sections to see what is there. A good starting paper to look at would be: Facey K, et al. Patients' perspectives in health technology assessment: a route to robust evidence and fair deliberation.

WHERE CAN I FIND IT?



http://vortal.htai.org/?q=cpil&term_node_tid_depth=217

PATIENT VALUE: PERSPECTIVES FROM THE ADVOCACY COMMUNITY

WHAT IS IT?

A peer reviewed paper written by many members of the Global Oncology Advocacy Leaders (GOAL) group that discusses the concepts of value in oncology care from a patient perspective.

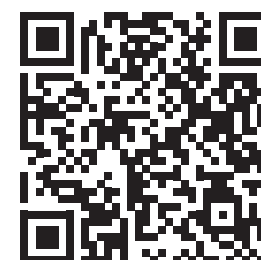
WHY IS IT RELEVANT?

There is a constant debate about how value is perceived in oncology, and a proliferation of value frameworks. This paper discusses the concepts behind value and recognizes that one person's values are not the same as another's.

HOW CAN I USE IT?

Use this paper to further discussions about value within your own organizations and take it to other stakeholders to drive dialogue between stakeholder groups on value.

WHERE CAN I FIND IT?



<https://onlinelibrary.wiley.com/doi/10.1111/hex.12628>

PROJECT TRANSFORM

WHAT IS IT?

A project by LUNGeVity in partnership with the John Hopkins School of Public Health to quantify patient preferences in lung cancer.

WHY IS IT RELEVANT?

This is an example of how patient organizations are partnering with the research community to answer some fundamental questions about the preferences and needs of people with a particular cancer.

HOW CAN I USE IT?

This can be used as an example project as you think about ways that you could generate or use data within your advocacy group's own field.

WHERE CAN I FIND IT?



<https://lungevity.org/research/patient-focused-research-center-patient-force/patient-preferences-and-needs/project>



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.