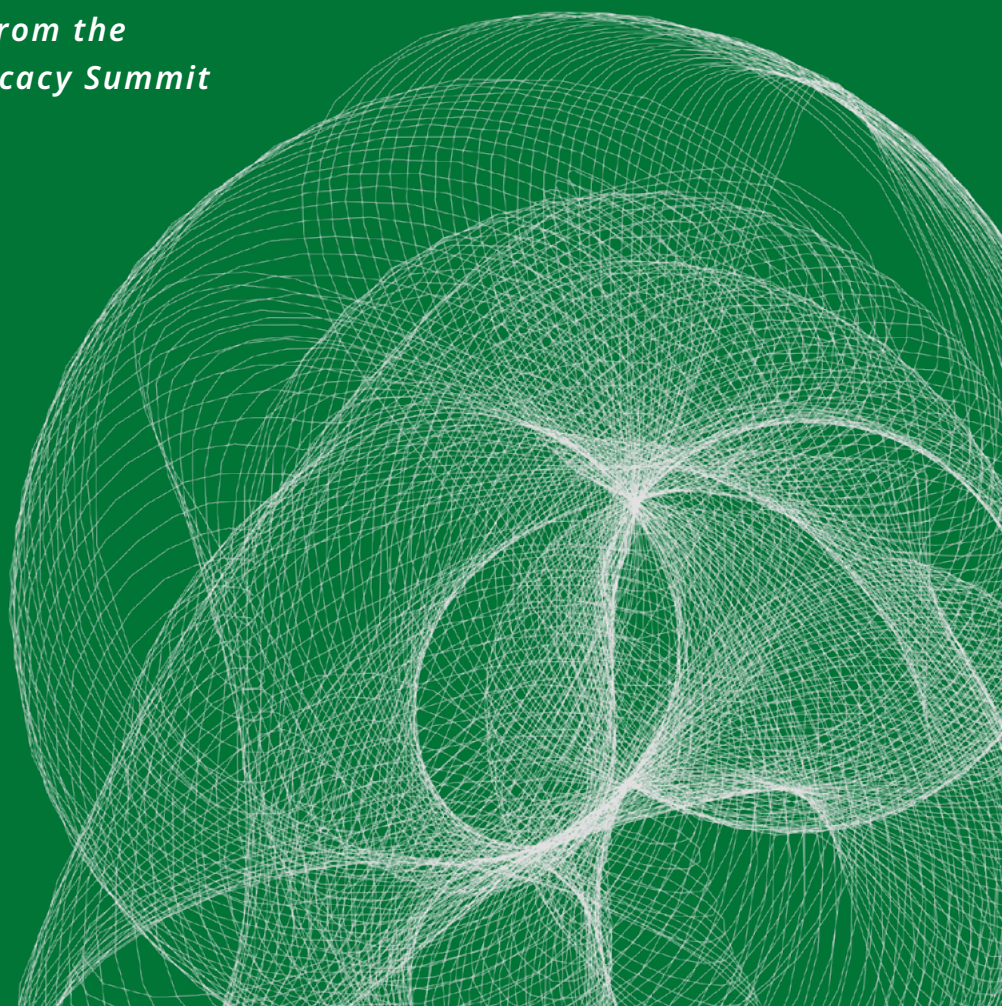
IQVIA INSTITUTE
FOR HUMAN DATA SCIENCE

Empowering Patient-Driven Research to Improve Patient Outcomes

THE ESSENTIAL ROLE OF PATIENT ORGANIZATIONS

Key themes and takeaways from the IQVIA Institute Patient Advocacy Summit held on December 1, 2021



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Introduction

The landscape for patient advocacy organizations is changing radically as these groups increasingly become the critical connectors and conveners of healthcare transformation and the development of innovative, breakthrough therapies. This is reflected in the fundamental shift from developing drugs and treatments *for* patients to developing drugs and treatments *with* patients.

Today, patient advocacy organizations are helping to close the gap between the rapid advances in science and human biology understanding and the slower progress seen in most other parts of the health system. The vital role of patient organizations is manifested in multiple ways, ranging from the translation of scientific breakthroughs to improve available therapies through patient-centered drug development to getting those therapies to the patients who will benefit from them. The involvement of patient groups is also critical in the development and use of diagnostics, the embrace of digital technologies, and the development of meaningful datahubs or registries to securely accumulate data. Furthermore, patient organizations have important roles to play in the development and application of artificial intelligence (AI) and machine learning (ML), the increased focus on tackling the fundamental issues of disparities and racism, the redesign of care delivery and flexibility in site of care and shaping the regulatory environment in which all of this takes place.

To further advance the understanding of the changing role of patient advocacy organizations in healthcare transformation, the IQVIA Institute for Human Data Science, in conjunction with IQVIA Healthcare Solutions, convened an invitation-only summit for patient organization leaders held on December 1, 2021, and titled *Patient Advocacy Summit - Helping Nonprofit Organizations Support Patients through Advocacy-Led Research and Data Initiatives.* The event brought together more than 100 participants along with 28 speakers from patient organizations and foundations to participate in thought-provoking conversations, to explore ideas and best practices around relevant and timely patient driven research and health data topics, and to consider ways to empower patient advocacy organizations. In addition to invited speakers from these organizations, the summit also included experts from IQVIA who drew upon their experience in applying unparalleled data, technologies, and capabilities to advance the success of patient advocacy organizations in meeting the needs of their members.

The following summary provides an overview of the major themes and key takeaways from the sessions at the summit. A list of the event speakers is included at the end of the document.

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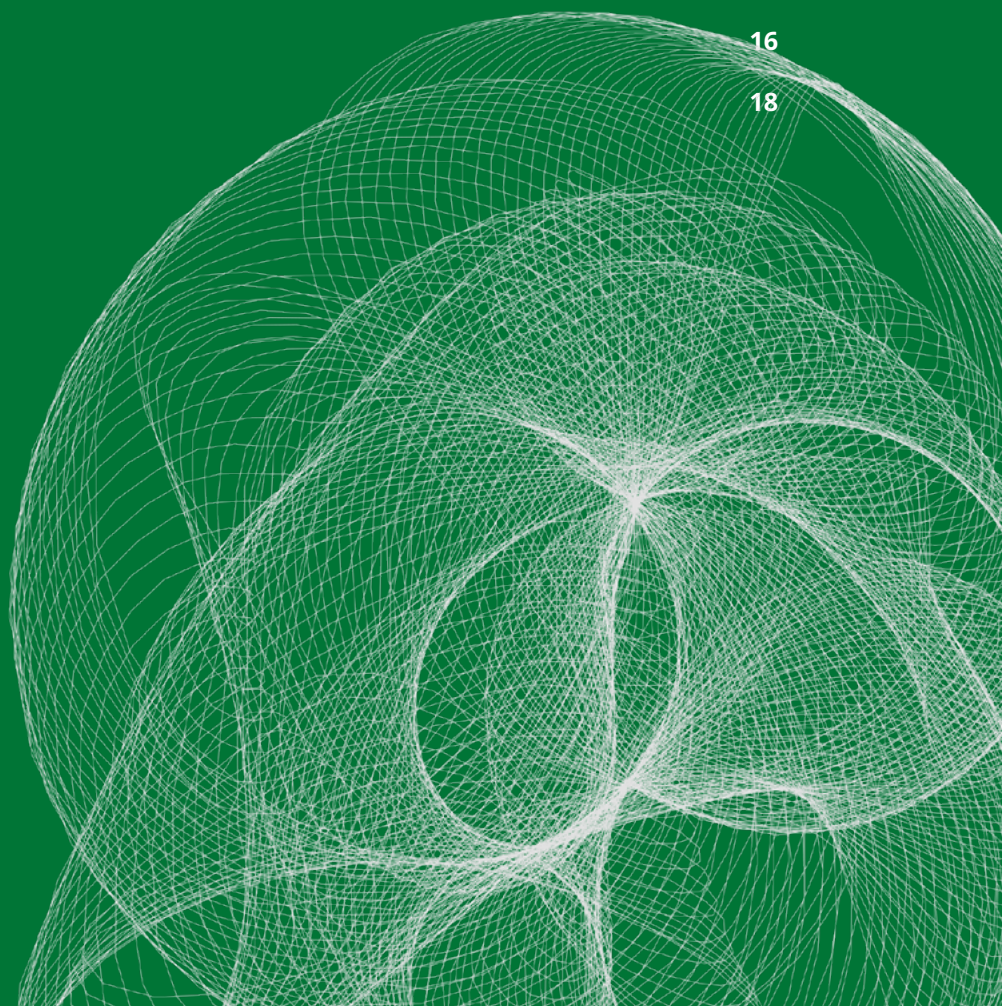


1. Policy and regulatory landscape

There is an opportunity and need for a shared infrastructure for patient organizations to fulfill the trusted convener role and maximize the combined influence that patient organizations can exert on regulatory and policy changes by working in unison. Similarly, there is an opportunity for coalition management to overcome the fragmentation of efforts and enhance the influence of patient organizations.

This session included presentations about current policy regulations relevant to clinical research and patient advocacy organizations to set the stage for discussing the role that patient groups can play in shaping and advancing policy and regulatory changes. This included an overview of the 21st Century Cures Act and the efforts to accelerate medical product development, advance new innovations, and modernize clinical trial design, as well as the proposed "21st Century Cures Act 2.0" that builds on the current legislation to further accelerate the development of new treatments, expand patient access to innovative treatment, enhance the diversity in clinical trials, and make patients a more integral part of the decision-making process around clinical trials and product development.

In addition to the 21st Century Cures Act, the Prescription Drug User Fee Act (PDUFA) VII also includes policy initiatives to enhance the patient voice in drug development and support innovative approaches to product development, including the enhanced use of real-world evidence in decision-making and the continued development of breakthrough therapies and rare disease programs.

Key discussion points in the session were:

- The role of patient organizations as trusted conveners of patient voices, in particular as a consequence of all the elements in the 21st Century Cures Act that continue to ripple across and impact all stakeholders and every part of the healthcare system. This includes the FDA requirement for patient experience statements to be incorporated in applications, patient access to health records, as well as broader patient-focused drug development initiatives. All of this is tied together in the role the patient organizations can play as the trusted convener of patient voices.
- Enhancing the diversity and inclusion of underrepresented patient populations in clinical trials. Importantly, this includes diversity defined beyond race and ethnicity and income level to include age and potentially other characteristics. As part of this discussion, the question emerged whether we are nearing some type of mandatory requirement for clinical trials to represent specific population segments proportionately, and whether we will see action on this in the coming legislative cycle.
- The many evolving elements in the policy and regulatory landscape create urgency for patient organizations to stay on top of potential opportunities to shape and influence policies. The role of umbrella groups or coalitions of organizations is important as they can help overcome the fragmented landscape of patient organizations that make it difficult for any single organization to be as impactful as they can by working together.
- The question of transparency around and access to data was discussed with a particular reference to how some registries appear to be locking up data, in particular registries controlled by specialty societies.

2. Patient-centered research and drug development

Patient advocacy organizations have driven new treatment in their therapeutic space through funding different types of interventional and non-interventional research. They also input into pharma and biotech programs which can be more efficient for their organization. However, the biggest challenge appears to be how to authentically listen to and translate the patient voice into meaningful action and change.

The session heard examples from panel members and audience members of patient organizations who are playing a key role in driving drug development, perhaps best where they are providing funding for research at some point during the drug development cycle. Speakers from two different advocacy organizations talked about their experience driving drug development with funding provided at different stages of drug development in their indication of interest.

In one model, the advocacy organization started up a trial for personalized medicine in rare cancers, built the infrastructure and were running the trial within their organization, and partnered with an external group of experts who comprised a tumor review board. The study provides the opportunity for patients to be matched with appropriate treatment for their tumor mutation status, and also provides the possibility of providing efficacy evidence for new sponsor-funded interventional trials where outcomes are positive.

Another group had funded basic science on the condition over five to six years, identified that a class of drugs being developed in other indications may have efficacy here and played a fundamental role in meeting with pharmaceutical companies to make the case for development in NF-1. In 2020, Koselugo became the first drug approved for NF-1, providing a treatment option to patients where there were previously none.

In the context of patient-centered research, patient advocacy organizations can play a very proactive role as connectors, reaching out to regulators, engaging with pharma, contacting researchers to get patients involved in trials early on and keeping them engaged throughout the trial process.

The discussion centered around several challenges and opportunities:

- When patient advocacy organizations drive drug development, they naturally and uniquely have a patient-centric approach as patients and families are more involved in funding and programs. Patient groups typically have a high number of staff and volunteers who have been personally impacted by the disease of relevance to their organization.
- Patient advocacy organizations can play a key role in working with pharma and biotech to consider developing drugs for their disease of interest based on good preclinical data and a sound rationale. Non-interventional studies can help provide more information on disease progression and quality of life, in addition to helping identify a group of patients to participate in research, which is often attractive to companies when considering pursuing an indication. Finally, treatment recommendation or interventional studies can provide clinical evidence that a more formal drug development program could be pursued.

- The FDA is very keen on listening to patient organizations and has many programs that enable these organizations to have their voices heard. FDA's listening sessions can be extremely helpful to drive awareness of a disease to explore the development of potential candidates for new therapies by incorporating patient insights, which can help drive protocol and program design and delivery. Some patient organizations have also run external listening sessions. Both require significant time and financial investment from patient organizations.
- Truly incorporating the patient voice remains a challenge, especially how to take practical steps to get the patient voice translated into action. In some ways, it is easy to affirm the value of including the patient voice in research but translating this into meaningful change is more complex and challenging.
- Deciding how to utilize funding is challenging for patient organizations as they often need to ensure funding for a variety of other programs for patient and family support. All the activities where patient groups can truly drive drug development are costly. It is less costly to provide feedback to programs that are led by pharma and biotech, and we need a more consistent framework to ensure patient and family input is always being sought by sponsors.
- There are some limitations to the interactions that pharma and biotech can have with patients for commercial reasons, but all stakeholders agreed that patient advocacy organizations can play a role in motivating pharma and biotech companies to be more patient focused at every stage of drug discovery, development, and commercialization.

3. Putting patients first when applying AI to healthcare: Ethical, analytical, and development considerations

Humanizing AI is a major requirement for the successful adoption of AI in healthcare moving forward. Putting patients at the center of AI — from the design of algorithms through to the interpretation and decision-making based on AI-generated insights — is essential for the successful application of AI at scale in healthcare. Patient advocacy organizations can play an important role in bringing patients into the design process of AI — demystifying the "black box" of AI and finding ways to engage patients who are representative of the relevant population in the development of algorithms. Close collaborations and partnerships between patient groups and researchers is critically important for this endeavor.

This session considered the practical considerations when applying AI for medical event prediction:

- Selecting the right data set to build and train algorithms on is key to the success of AI deployment in healthcare . The data must be representative of the data source which will exist in the healthcare settings where the built algorithms will be deployed, otherwise translating the development work — where you may achieve very impressive results — to the clinical setting may not be possible.

- Another important aspect that is frequently overlooked is assessment of bias, both bias that exists in the underlying data as well bias created by the algorithm. Researchers should disclose any detected by bias to the end user of the algorithm, but also attempt to correct it, while carefully assessing the impact of the correction against all protected characteristics, such as age, gender, race, etc.

- It is important to consider the different types of data available when building your model. While most efforts have looked to capitalize on structured data, approaches which make use of both structured and unstructured data can lead to more precise algorithms, or the discovery of novel predictive features. A good example of this could be using indicators of social isolation as predictors for disease progression.

There are many considerations for patient organizations to be aware of when applying AI in healthcare:

ETHICAL CONSIDERATIONS

- Privacy: How are principles and protocols around privacy and safety met? Is informed consent being secured? What are the potential benefits and risks for patients involved? How do you achieve ethical compliance by ensuring that patients and communities really understand what AI is, what it is supposed to accomplish, and the potential risks and benefits?

- Data sharing: How do you achieve the benefits of data sharing while maintaining privacy and integrity of individual patient data?

PURPOSE

- Purpose of application: Is AI being applied for the purpose of discovery or for clinical development of therapeutic options? What are the questions that the algorithms are intended to solve?

- Adoption in clinical workflow: How do you translate insights from AI-driven research into improvements in clinical workflow?

- Maintaining medicine as an art: While AI provides opportunities for generating insights that exceed human capacity due to the volume or complexity of data, how do you maintain the recognition that medicine is also an art?

PATIENT ENGAGEMENT

- How do you engage patients in the application of AI, recognizing all the complexities of AI? How do you engage patients in the development of queries and the design of algorithms from the early development stage and throughout the application? Local and global events with patient groups is one area for engagement, but there are also opportunities to create workshops, hackathons and other strategies for engaging the patient community in algorithm design. The general consensus is that patients want whatever will advance treatments fastest — including AI. However — if the AI only uncovers new rare disease — where there is no treatment — there is also a voice of concern within the patient community — "if there is no treatment — why would I want to know?"

4. A learning health systems perspective on the changing role of registries and data hubs in healthcare

Registries are important tools for patient organizations as they are building dynamic learning health systems. Registries can help organizations empower patients to better advocate for themselves, improve provider treatment protocols, support researchers in asking more relevant questions that lead to improved outcomes that matter, and assist organizations in creating more strategic partnerships.

Registries are important tools for patient organizations as they collate data and generate evidence about a broad range of matters, including disease definition, disease progression, treatment protocols, treatment response, clinical trials, real-world data, patient reported experiences, and other content. This session discussed how patient advocacy organizations can leverage data through registry programs to accelerate access to interoperable data, resulting in new means to engage patients and generate evidence.

Key themes discussed included:

- Considerations about priorities for new registry programs: How can patient organizations become educated about the best way to build registries, for example by seeking advice from other patient groups? How do patient groups invest in community building and partner networks? How can patient groups invest in organizational alignment on data governance models, meta-data alignment, and standardization?
- The transformation of patient-centricity from a general direction to an immediate operational priority: What are the new approaches to involve patients in every element of planning, research, and the return of data to patients in a timely fashion? How does family and community involvement enable patients and families to have better hope for the future for their children?
- Emphasizing the "trusted convener" role of patient groups: How will patient-centricity be shaped in the future revolve around patient groups as the fulcrum of change?
- The changing role of registries from data hubs to dynamic learning health systems: How are patients empowered to better advocate for themselves? How are provider treatment protocols being improved? How can registries help researchers ask more relevant questions and develop better hypotheses that lead to improved outcomes that matter? How can registries help organizations create more strategic partnerships?
- Linking legacy datasets: When linking legacy datasets, what are the interactions between regulatory and legal constraints, incentive pressures, and technical capabilities? How does one distinguish between what is technically possible and what is contractually or legally allowed when it comes to linking data?

5. Operational challenges: Understanding and navigating the evolving challenges affecting patient data initiatives

Today's patient advocacy organizations are more sophisticated and knowledgeable than ever, which leads to opportunities as well as new challenges. They are not only providing patient-friendly disease awareness, education and support, but they are participating at a higher level in the healthcare process, engaging in drug development and health technology assessments. Patient advocacy organizations are also increasingly playing a critical role as a neutral convener that helps patients, industry regulators, and clinicians interface in a safe environment where there is trust and allows the use of data to make advances in science for the benefit of patients.

As patient organizations build registries and health data platforms, they inevitably experience operational challenges. This session engaged a panel of experts from a diverse set of patient organizations on their experiences and guidance related to navigating operational challenges ranging from technology to regulations to partnership and sustainability. Participating experts represented organizations with focus areas ranging from chronic and rare diseases to very large patient populations.

The session discussed commonly known and new challenges for navigating patient data initiatives.

Major challenges at the center of discussion were:

- Understanding the role of the 21st Century Cures Act in terms of changing patient access to data and what that means for a patient advocacy organization.
- Understanding that the siloed data capture and operational approaches that patient organizations have been optimizing around in the past are no longer scaling. Today, these organizations need a core backbone that ensures they understand who in the community has contributed information, what they are allowed to do with it, and what is appropriate when reaching out to leverage those relationships.
- Tying all the data and information together to ensure sustainability from a revenue and engagement perspective is also a challenge. In other words, clarify how to pay for the data. Operational excellence is driving value in the community right now.
- Building governance models that provide value back to patients is a challenge. Patient organizations continue to work to address data silos and data fragmentation, privacy issues, and data linkages to ensure that patients who have consented to their data being used can get value back in the form of the knowledge that has been gained.
- Involving more and more public private partnerships is necessary to address challenges, but these collaborations also create new obstacles when accessing and integrating multiple and diverse data sources.

6. Diversity in real-world research

Plenary session

The increase of diversity and inclusion in clinical trials is absolutely necessary to ensure the safety and efficacy of new products in the patients they are designed to treat. Patient advocacy organizations and boards will need to continue to champion critical work that directly impacts this goal of health equity.

The plenary session began with a discussion of the history of exclusion, biased care, and mistreatment of Black, Indigenous and People of Color (BIPOC) patients in in the U.S. healthcare landscape. This highlighted many of the poor health outcomes and related consequences of this many decades-long disparity.

The COVID-19 pandemic threw these disparities into sharp relief and has shown a bright light on the needs for diversity, inclusion, and health equity. Equality and equity are not the same, and health equity is what is now required.

A collective and collaborative change is required across the healthcare spectrum, and there is broad consensus about the urgency to address health disparities and this session discussed how that change will require knowledge, common sense, science, faith and both critical and realistic measures of success.

This change necessitates a framework that enables cultural sensitivity and multicultural outreach if it is to capture diverse patient voices, drive equity in clinical trial participation, and achieve positive health outcomes for underrepresented and underserved patient populations.

Patient advocacy organizations and their boards will play a crucial role in implementing, measuring, and sustaining these important changes.

Breakout session

Patient advocacy organizations are at the front line of addressing the complex issues of inclusion and diversity. The challenges lay in reaching patients of very different backgrounds with very different experiences. Yet collaboration, vulnerability, and humility, alongside best practices, are driving next-level solutions being developed that keep the patients as the focus. There is an opportunity for an ongoing consortium around diversity and inclusion for patient advocacy organizations since there is no current leadership around this among specialty societies and advocacy organizations. Building modular approaches around this is a real opportunity.

The session began with the sharing of diversity and inclusion initiatives that organizations are pursuing, including those that are — and those that are not — showing positive results.

Some well-intended organizations had rolled out initiatives for diversity and inclusion initiatives and found that they fell absolutely flat. They had planned what they thought would be great outreach, but they were not listening to the real needs of the minority audiences. So the theme of listening was very current throughout the discussion, along with being active listeners and knowing your community. Also, listening first before putting a lot of energy behind deploying tactics to engage underrepresented patients because it is hard to know if those patients are being reached.

Two organizations noted that they had developed several outreach programs but learned they were not working as they expected. These organizations are still searching for a good solution but continue in their commitment to reach as many patients as possible.

The conversation continued to cover several main points:

- Diversity and inclusion, outcomes, and health equity are all intertwined and necessary for achieving organizational goals. The various patient populations are not monolithic. Black, Hispanic, and Asian populations are not homogenous. There are many diasporas within each ethnic group.
- Each community must be assessed for its specific needs, putting organizations in listening mode — and those needs will change regardless of each community being Black or Hispanic, as other factors, such as geography, play important roles in health. This focus on specific community needs creates a challenge in scaling programs.
- Listening and intentionality are cornerstones to doing the work when reaching patients, adapting, and progressing.

There was agreement about the value of creating a toolkit for patient advocacy organizations to achieve their goals.

A kit might include tools to undertake the following:

- Assess how well you know your community - how to know what you know and what you don’t know about your defined patient community.
- Profile the board and staff of your organization to determine how representative they are of the people you are trying to reach.
- Develop objective measures your organization is using to define success.
- Evaluate the outcome of initial outreach and engagement to know where it worked, where it did not work, and where it can be adapted.
- Establish goals for expanding or scaling initial measures and defining success.

7. Building and mobilizing your community: Finding, connecting, and engaging patients and caregivers

Truly listening to patients and constituents is critical for success. Deeply understanding the emotional needs of patients and the barriers for behavior change is critically important to move people from a passive to an active role in dealing with their personal burden of disease. This is a particular challenge as patient organizations grow in size. Maintaining a culture of being warm and welcoming and staying attuned to the individual needs of patients is critical, as is personalizing and individualizing the approach to engagement and outreach, especially when using digital engagement tools.

Utilizing health data technology is a key strategy for all patient organizations to engage with their communities.

This requires robust research to understand the community through the use of technologies, registries, and AI, doing rigorous and scientific research as well as good market research on what matters most to patients, and listening to patients to understand their needs. This also includes utilizing technology to advance relationship building, enhance patient support programs, and mobilize local patient communities.

Through the session there were discussions about how to balance the use of digital high tech with high touch personal engagements. The question was raised around how to avoid losing the feel of the mission and the emotional attachment to the patient membership when using high tech, digital solutions.

All organizations are working through how to scale and grow while continuing to stay close to patient communities. An important challenge is how to serve more people while not losing sight of the individual patient, which includes the resounding importance of listening to the patient. And when using technology to listen to patients, it is important to find ways to make the interaction personal.

Other key points raised in the discussion included:

- A big moment was focusing on the notion of listening, and discussion of how technology companies can be useful partners to assist with understanding, listening, and truly knowing the broader patient communities of the different disease areas.
- Patient populations are heterogenous with highly individual values and beliefs — even if they are impacted by the similar condition.
- Identifying and connecting with people does not automatically lead to engagement and activation.
- Sharing the experiences and resources among patient advocacy communities would be most useful, including those relating to connecting patients to clinic trial search engines and registries.

8. Where's the money in data: Sustainability and data monetization in action

A dynamic monetization strategy and roadmap is critical for patient organizations to ensure they develop datasets and capabilities that can accelerate their mission while also supporting financial sustainability and adjust to the changing landscape and shifting needs of their own constituents.

Patient advocacy organizations are increasingly building data assets and developing research capabilities that represent significant value with the potential for monetization that can turn these assets into financial value to help fund operations and innovations in pursuit of the organization's mission.

The session unveiled the shroud of sensitivity around the topic, and there were some lively discussions of key themes:

- The topic of registry monetization with industry is moving from being viewed as a conflict of interest to a critical enabler and accelerator of the mission and overall sustainability strategy of advocacy organizations. This trend is growing — with many successful examples of collaboration in the industry being seen.
- It is not a one size fits all. Instead, there are different monetization paths that organizations are taking — each with a different mix and evolution of data and service offerings, capabilities being built around the data, policies guiding data governance and industry engagement, and the ecosystem that encompasses what is being done in-house and through external partnerships.
- There are some commonalities around pain points: a lot of challenges for advocacy organizations to think about how to value, price, and effectively deliver their data and service offerings. There is a common view that organizations are consistently leaving money on the table and not fully reflecting the value of what they have invested in.
- Organizations that are building or looking to enhance registry programs are benefiting from developing a robust sustainability / monetization strategy and road-map — in order to have a dynamic plan to support more accurate investment planning and resourcing and evolve as their organization matures and the needs of the community and industry changes.

9. Making all the right moves: Determining the next right move when resources are limited

Learning from others and collaborating with external stakeholders are critical elements in successful organizational journeys for patient advocacy organizations. Thinking ahead and keeping a low center of gravity is equally important to ensure balance, stability, and options. Successful patient organizations have extraordinary assets in their experiences and knowledge that other patient groups can learn from and build on.

The session showcased different experiences and journeys of patient organizations as they evolved in maturity from being small grassroots organizations, typically staffed with parents, family members and friends, to larger, more professional organizations. The journeys also displayed the arc of development from small clinical trials, through the use of translational tools for testing and screening and sophisticated biomarkers and imaging data, to partnerships with NIH and FDA around the creation of trial site networks, protocol designs, and industry partnerships.

Interspersed with the scientific and organizational learnings were deeply personal stories about fighting to combat rare diseases and losing children to the diseases they worked on.

The key learnings from their organizational journeys included:

- It is important not to be intimidated by the challenges you are facing.
- Don't start action before thinking through the details.
- Don't go at it alone. Try to determine who else is out there that you can leverage.
- There is a lot to be learned from more established patient advocacy organizations.
- Collaboration between external stakeholders, the government and the patient organization is important.
- Plan ahead to build tools and assets that will be of value to industry and other stakeholders to ensure funding and sustainability.
- Be curious and do your homework.
- Keep a low center of gravity to make sure you have balance, stability, and options when faced with challenges.

10. Patient advocacy leadership discussion

Patient organizations are looking for common frameworks, tools, and platforms to overcome their own fragmentation, enhance their learning, elevate their influence, and accelerate impact. Rather than each organization building its own data infrastructure and reinventing themselves individually, there is a mutual desire and an opportunity for creating and building a joint interconnected infrastructure for data and evidence-generation, application of advanced technologies and analytics to standardize and automate processes, reduce redundancies, and accelerate innovation and progress. Particularly urgent is the realm of rare diseases and the emergence of cell and gene therapies, where many organizations are struggling with the development of value and burden of disease models.

The session started with questions being posed to the attending patient advocacy leaders regarding the new trend of patient advocacy organizations moving from advocacy to evidence-generation. Specifically, the attending patient leaders were asked what they saw as potential accelerants of the future.

Some pointed to the major priority of advancing therapies by removing barriers to access, accelerating research, and reducing the burden of disease. This includes the need to reimagine, recognizing we can all do better. Some of the key problems and opportunities to tackle are:

- Breaking down the data silos by sharing data and insights and achieving a future state of openness.
- Overcoming the lack of usable data by having patients collect data, which raises the question about how patients are enabled to share the right data at the right time.
- Building an interconnected ecosystem of data by leveraging extraordinary technology to generate more data and better insights.

An essential opportunity is how to automate the process of data collection and standardize evidence-generation to make it easier for patients to collect and deliver their own data.

Another leadership opportunity was presented in pioneering work to develop burden of disease models that can be applied across different rare diseases and can be utilized effectively to demonstrate the value of therapeutic interventions in discussions with payers, health technology assessments (HTAs), and other relevant bodies, such as the Institute for Clinical and Economic Review (ICER).

Key considerations in the discussion:

- There are strong commonalities across the many patient organizations despite the diversity and unique character of the disease and patient populations that each group serves. There is a palpable hunger for sharing experiences and learning from each other given the common nature of the challenges and opportunities that each organization is facing, whether it is sharing learnings from clinical trial development, enhancing the understanding of natural history of disease studies, building burden of disease models, advancing collaborations with NIH, FDA, and other relevant bodies, or making hard decisions about priorities given limited resources.

- Every organization has unique, specific needs, but they also have very similar issues and needs; for example, getting informed consent, figuring out how to do a data dictionary, understanding how to visualize and bring value back to patients, and how to engage over the arc of the journey of the disease.

- Every foundation is creating their own technical infrastructure and the data don't talk to each other. This begs the question how patient organizations can accelerate their success by working more closely together, not just learning from each other, but ultimately building joint infrastructures around data generation, creating common frameworks, shared tools and platforms, developing applications for advanced technologies such as AI, and advancing burden of disease models to promote their value story. Every organization does not need to reinvent itself, if they can access third parties that can provide a plug and play approach.

- Every organization at some point when they get close to having an angle on a cure will never have that cure sitting in a pharmacy unless somebody will pay for it. So, there is a need — particularly in rare diseases, gene and cell therapies, and curative products — to figure out what the value is in terms of health economic impact and disease burden. Therefore, the idea that we should be doing something collectively rather than each organization trying to do this on their own was a key point of discussion.

General key takeaways

1. **There is an urgency for patient advocacy organizations to understand and take advantage of the evolving policy and regulatory landscape** that provide a plethora of opportunities to lean in and leverage new initiatives and guidance in the 21st Century Cures Act 1.0 and 2.0, PDUFA IV and PDUFA VII around the vital role of articulating the patient experience in drug applications, advancing access to health records, addressing diversity and inclusion in clinical trials, as well as broader patient-focused drug development programs. The policy environment is highly conducive to patient advocacy organizations playing an active role as conveners of the patient perspective and voice.

2. **There is a fundamental shift in the role of patient advocacy organizations** from being advocates for specific patient interests to becoming critical agents in accelerating the healthcare transformation based on patient-centered evidence-generation. Patient organizations are becoming the fulcrum for change in the healthcare transformation toward patient-centered science. Patient organizations are increasingly becoming the connectors and convenors of a multistakeholder, data-driven ecosystem as they uniquely and authentically represent the key interests of the stakeholders that ultimately benefit from improved health outcomes, i.e., the patients and their families.

3. Despite the clarion call for elevating and amplifying the patient voice across multiple dimensions, listening to patients, and translating the patient voice into meaningful action, is a general challenge for patient organizations and the stakeholders they work alongside. **More work is needed to truly understand the underlying issues underpinning health disparities and lack of diversity in drug development**, and how to move from identifying the right patients for the right interventions to mobilizing patients to take an active role in their own health and care.

4. **Many patient organizations are struggling as they mature**, grow, and elevate the scale of their activities and influence. The warm and nurturing culture of smaller patient organizations, which typically are led by people who are patients themselves, or parents to patients, is the soul and power of these organizations, but it can be difficult to maintain as the organizations grow in size. Finding the right balance between authenticity and emotional attachment on the one side and professionalism and organizational efficiency on the other side can be a challenge. Both strategic and operational challenges must be confronted as these organizations seek to fulfill their missions in advancing research and bringing cures to their members.

5. **Engaging with other stakeholders, whether life sciences companies or data vendors, appears to be tricky** as it often involves challenges around ownership to and monetization of data and assets. Furthermore, there can be issues relating to differences in culture, style, and values. More efforts are required to building trusting and sustainable partnerships for the mutual benefits of all partners.

6. Patient advocacy organizations have unique and diverse needs based on the specific patient populations and the diseases they service, but they also have strikingly similar needs and challenges. Patient organizations are eager to learn from each other, avoid redundancies in learning, and share experiences and insights. They recognize that it is not meaningful that every organization tries to reinvent itself in isolation. Therefore, **there is general desire among patient organizations to generate common frameworks, tools, and platforms for sharing insights and learnings, build connected data infrastructures, and advance common burden of disease and value models to collectively accelerate innovation and breakthrough therapies.** There is also a joint interest in advancing collective action and building a shared consortium of patient advocacy organizations to exert their influence and impact on policy and regulatory changes that will benefit science, innovation and improve patient outcomes.

Speakers

The following individuals generously contributed their time and perspectives to the IQVIA Institute Patient Advocacy Summit. The views and opinions expressed in this summary are drawn from the entirety of the summit and represent those of individual participants but do not necessarily reflect the views of everyone or those of specific organizations.

GUEST SPEAKERS

Alliance for Aging Research
Sue Peschin, President & CEO
Policy & Regulatory

Arthritis Foundation
Maria Vassileva, Ph.D., Senior VP Science Strategy
Operational Challenges

Canadian Organization for Rare Disorders
Durhane Wong-Rieger, President & CEO
Putting Patients First When Applying AI to Healthcare

Children's Tumor Foundation
Traceann Rose, Patient Engagement Director
Patient-Centered Drug Development

Crohn's and Colitis Foundation
Angela Dobes, VP
Operational Challenges

Cystic Fibrosis Foundation
Christopher Dowd, Business Director of Scientific Affairs
Where's the Money in Data

debra
Brett Kopelan, Executive Director
Leadership Panel

DLA Piper
Geoffrey Levitt, JD, Co-chair Life Sciences Policy and Regulatory Group
Policy and Regulatory

Foundation Fighting Blindness
Steven Ringel, VP & GM Rare Diseases
Operational Challenges

Friedreich's Ataxia Research Alliance
Ron Bartek, President & Founder
Making All the Right Moves

Global Parents of Eczema
Korey Capozzo, MPH, Founder & Executive Director
Building & Mobilizing Your Community

Lupus Foundation
Jason Harris, Director, Public Policy
Where's the Money in Data

Lupus Research Alliance
Diane Gross, National Director of Advocacy and Programs
Diversity & Inclusion

Lustgarten Foundation for Pancreatic Research
Linda McNeil Tantawi, CEO
Putting Patients First When Applying AI to Healthcare

Multiple Myeloma Research Foundation
Eva Lepisto, VP of Informatics
The Changing Role of Registries in Healthcare

National Brain Tumor Society
Danielle Leach, MPA, Chief Communications & Government Relations
Leadership Panel

National Medical Association
Doris Browne, MD, MPH, 118th President
Diversity & Inclusion

Pancreatic Cancer Action Network
Donna Manross, VP Science and Medical Affairs
Building & Mobilizing Your Community

Parent Project Muscular Dystrophy
Kaylan Moitoso, Chief Business Officer
Operational Challenges

Parent Project Muscular Dystrophy
Ryan Fischer, Chief Advocacy Officer
The Changing Role of Registries in Healthcare

PCD Foundation
Carey Kauffman, Patient Registry Director
Making All the Right Moves

PKD Foundation
Elise Hoover, Senior Director of Research
The Changing Role of Registries in Healthcare

RARE-X
Nicole Boice, Executive Director
Leadership Panel

Stoke Therapeutics
Dawn Kalmar, Chief Communications Officer
Patient-Centered Drug Development

T1DExchange
David Walton, CEO
Where's the Money in Data

TargetCancer
Jim Palma, Executive Director
Patient-Centered Drug Development

UsAgainstAlzheimer's
Russ Paulsen, COO
Building & Mobilizing Your Community

IQVIA SPEAKERS

Murray Aitken, Executive Director, IQVIA Institute
Summit Executive Sponsor, Opening Remarks, Policy & Regulatory

Barbara Arone, MS, VP, Real World Solutions
The Changing Role of Registries in Healthcare

Ian Bonzani, Ph.D., Senior Principal, Healthcare Solutions
Where's the Money in Data

Leslie Clapp, MD, FAAP, VP Medical, IQVIA Biotech
Diversity & Inclusion

Gregory Dennis, SVP, Global Therapeutic Science & Strategy, Research & Development
Diversity & Inclusion

Sarah Johnson, Head of Patient Advocacy, EMEA Strategic Operation
Building & Mobilizing Your Community

Jeffrey Keefer, MD, VP Medical Therapeutic Center of Excellence
Patient-Centered Drug Development

William Lawrence, Senior Director, Registry Center of Excellence, Healthcare Solutions
Making the Right Moves

Nadea Leavitt, Senior Director, AI for Healthcare & MedTech, Real World Solutions
Putting Patients First When Applying AI to Healthcare

Adam Mariano, Esq., MPSH, Senior Director, Strategic Operations, Integrated Health Practice
Diversity & Inclusion

Adrian McKemey, Ph.D., SVP & Managing Director, Global R&D Strategy Solutions
Leadership Panel

Jon Morris, MD, VP & CMIO, GM, Healthcare Solutions
Summit Executive Sponsor, Closing Remarks

Leon Rozenblit, JD, Ph.D., Head of Registry Center of Excellence, Senior Director Product & Strategy
Registries & Learning Healthcare System

Jamie Skipper, Ph.D., Director, Healthcare Registry Technology Consulting Services
Policy and Regulatory

Ali Smyth, Ph.D., Strategy Director, Pediatric and Rare Disease Center of Excellence
Patient-Centered Drug Development

David Voccola, Senior Director, Global Strategic Planning, Integrated Health Practice
Operational Challenges

Alexandra Weiss, Director, Strategic Operations, Patient Advocacy, Healthcare Solutions
Building & Mobilizing Your Community

Calum Yacoubian, MD, Associate Director, NLP Healthcare Strategy
Putting Patients First When Applying AI to Healthcare

About the Institute

The IQVIA Institute for Human Data Science contributes to the advancement of human health globally through timely research, insightful analysis and scientific expertise applied to granular non-identified patient-level data.

Fulfilling an essential need within healthcare, the Institute delivers objective, relevant insights and research that accelerate understanding and innovation critical to sound decision making and improved human outcomes. With access to IQVIA's institutional knowledge, advanced analytics, technology and unparalleled data the Institute works in tandem with a broad set of healthcare stakeholders to drive a research agenda focused on Human Data Science including government agencies, academic institutions, the life sciences industry, and payers.

Research Agenda

The research agenda for the Institute centers on 5 areas considered vital to contributing to the advancement of human health globally:

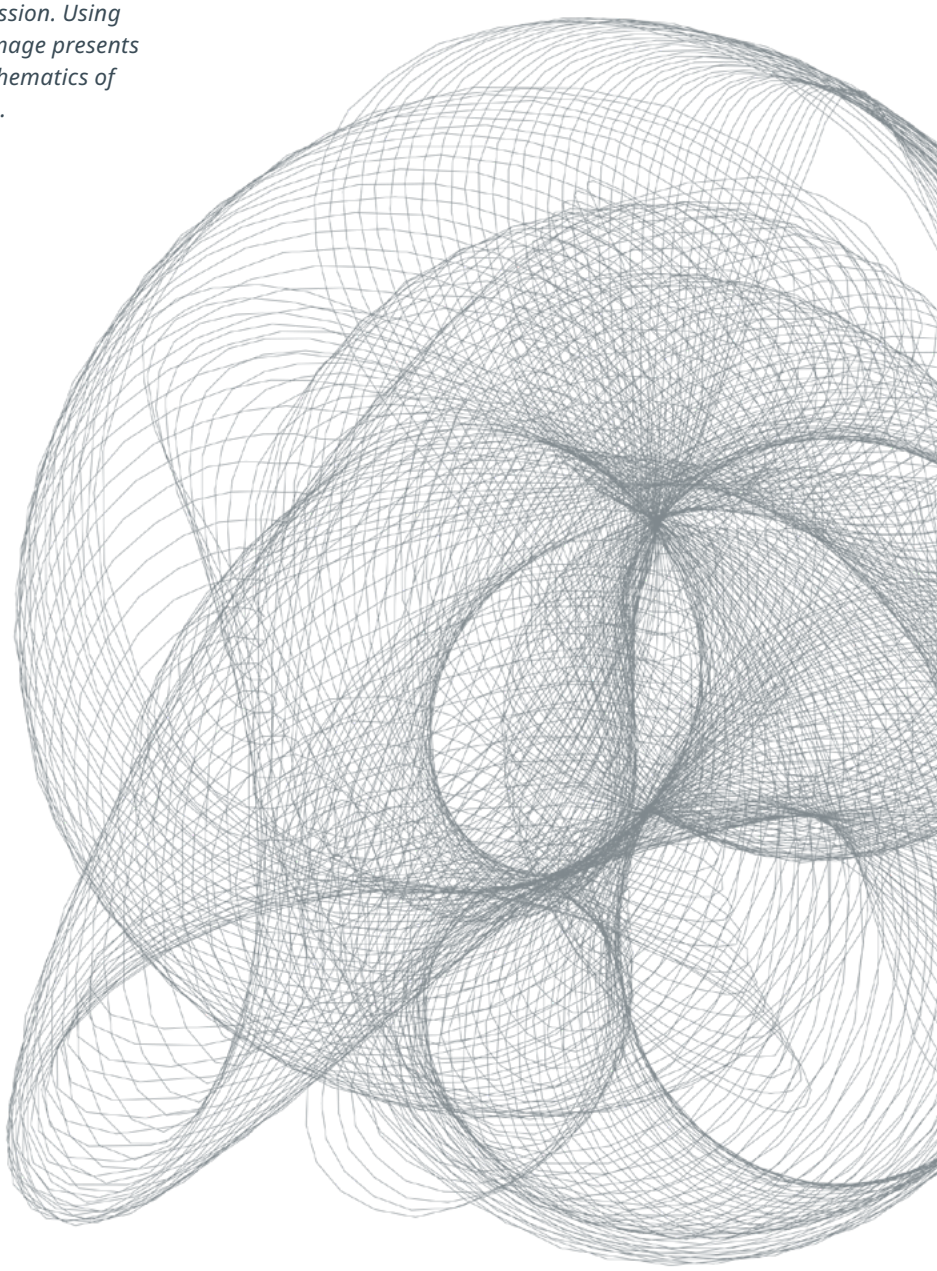
- Improving decision-making across health systems through the effective use of advanced analytics and methodologies applied to timely, relevant data.
- Addressing opportunities to improve clinical development productivity focused on innovative treatments that advance healthcare globally.
- Optimizing the performance of health systems by focusing on patient centricity, precision medicine and better understanding disease causes, treatment consequences and measures to improve quality and cost of healthcare delivered to patients.
- Understanding the future role for biopharmaceuticals in human health, market dynamics, and implications for manufacturers, public and private payers, providers, patients, pharmacists and distributors.
- Researching the role of technology in health system products, processes and delivery systems and the business and policy systems that drive innovation.

Guiding Principles

The Institute operates from a set of guiding principles:

- Healthcare solutions of the future require fact based scientific evidence, expert analysis of information, technology, ingenuity and a focus on individuals.
- Rigorous analysis must be applied to vast amounts of timely, high quality and relevant data to provide value and move healthcare forward.
- Collaboration across all stakeholders in the public and private sectors is critical to advancing healthcare solutions.
- Insights gained from information and analysis should be made widely available to healthcare stakeholders.
- Protecting individual privacy is essential, so research will be based on the use of non-identified patient information and provider information will be aggregated.
- Information will be used responsibly to advance research, inform discourse, achieve better healthcare and improve the health of all people.

The IQVIA Institute for Human Data Science is committed to using human data science to provide timely, fact-based perspectives on the dynamics of health systems and human health around the world. The cover artwork is a visual representation of this mission. Using algorithms and data from the report itself, the final image presents a new perspective on the complexity, beauty and mathematics of human data science and the insights within the pages.



IQVIA INSTITUTE FOR HUMAN DATA SCIENCE

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