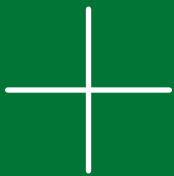IQVIA
INSTITUTE
FOR HUMAN DATA SCIENCE

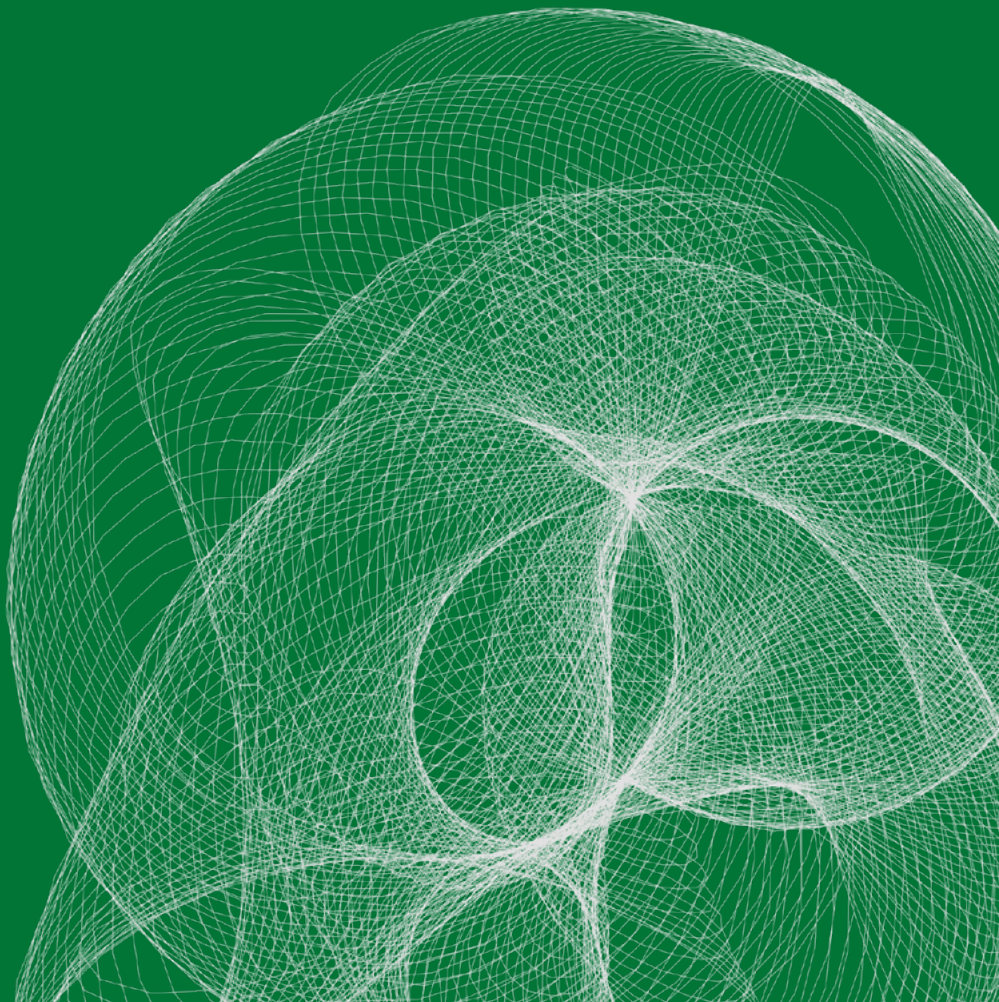# Elevating the Authentic Voice of Patient Advocacy

ACCELERATING ADVOCACY-LED RESEARCH AND DATA INITIATIVES
Key themes and takeways from the 2022
IQVIA Institute Patient Advocacy Summit

Introduction

Patient organizations are increasingly becoming the leaders and authentic voice of patients in healthcare transformation, the advancement of research, and the development of innovative breaktrough therapies. At the fulcrum of change and innovation, patient organizations are evolving as the critical conduits to patient-centered insights, advocacy-led research, and data initiatives in a fragmented healthcare landscape and a complex biomedical ecosystem.

Initially, patient advocacy organizations were focused on providing support for patients and their families and loved ones, as well as raising awareness for the cause and funding research to find cures or better treatments for specific patient populations. These organizations were often started by a few parents meeting at the kitchen table to identify relevant researchers and sponsors of programs to develop therapies for their children facing challenging diseases.

Today, many patient organizations have matured and become impressive, sophisticated powerhouses of knowledge, patient-driven insights, and data without which researchers, life sciences companies, and regulators will not be able to advance R&D and new innovations. In doing so, patient organizations have been the purveyors of the authentic, engaged and resilient voice of patients and caregivers in the transformation healthcare and accelerating therapeutic breakthroughs.

To further accelerate the knowledge of advocacy-led research and data initiatives, the IQVIA Institute for Human Data Science, in conjunction with IQVIA Healthcare Solutions, convened the second invitation only annual summit for patient organization leaders and partners held on November 16, 2022.

The event brought together 175 participants from patient organizations, foundations, life sciences and technology companies to share their experience and explore ideas and best practices around patient-driven research and data programs, prioritize how to make best bets on investments to maximize impact, and consider

how to utilize real-world evidence to elevate the patient and caregiver voice, put patients at the center of clinical development, apply technologies, and foster better partnerships between patient organizations and life sciences companies.

The summit also included experts from IQVIA who drew upon their experience in applying real-world data, technology-enabled solutions and capabilities to advance the success of patient organizations in meeting the needs of their constituents and stakeholders.

Throughout the summit, the leaders of the many represented patient organizations shared their experiences and insights by delivering thought-provoking insights and engaging in lively interactions and making connections to inspire future collaborations and initiatives.

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

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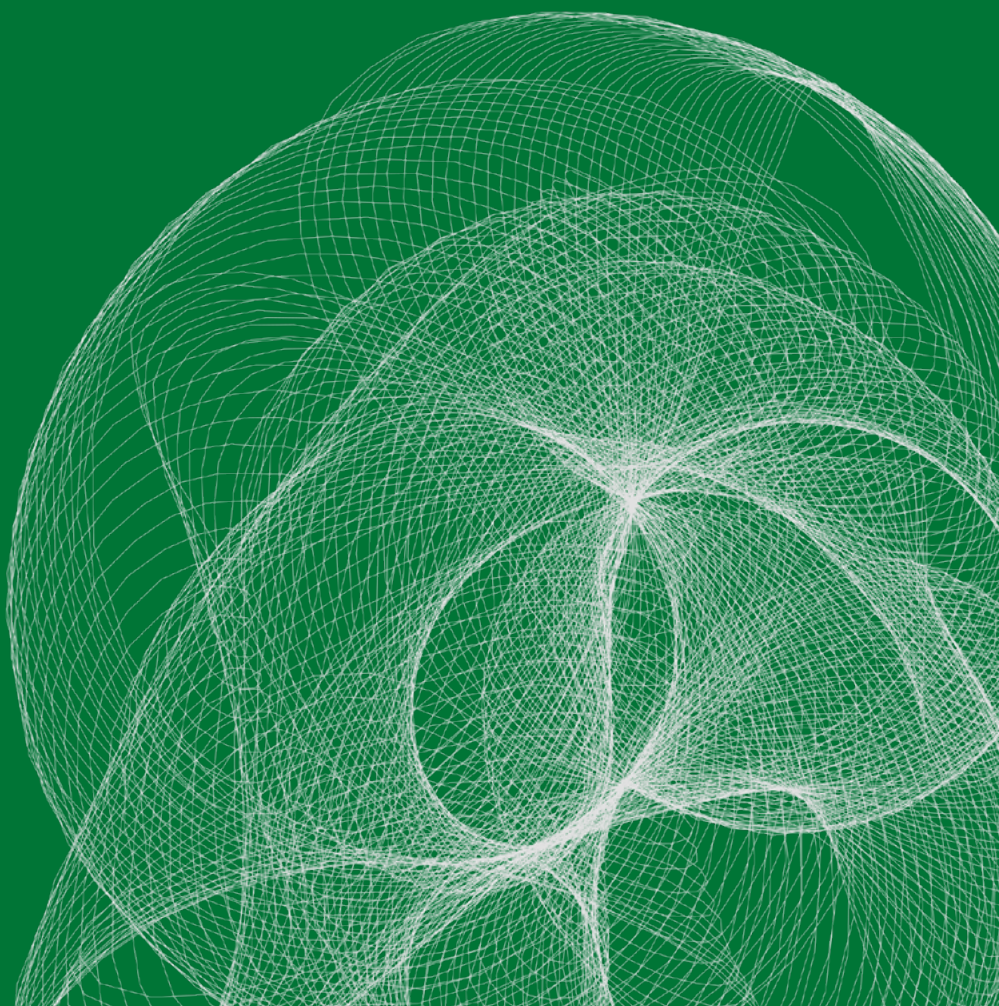
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1. A patient and caregiver listening session on what matters most to those we are serving

Engaging with authenticity, understanding diverse cultures, providing resources, and meeting patients where they are constitute critically-important approaches when interacting meaningfully with patients.

To achieve their missions of serving patients and improving outcomes, it is essential for patient advocacy organizations to listen and learn from patients and caregivers to understand personal needs and goals and to foster engagement with their organization, involvement in clinical research, and sharing those experiences and data. The panel discussion about listening to patients and caregivers comprised a diverse group representing the chronic, acute, oncology, and rare disease communities. The session focused on ways that patient advocacy organizations can adjust their approaches and initiatives to best meet patients and caregivers where they are as they tackle ambitious initiatives to improve patient outcomes and support patients and their families. Panelists represented Project Sleep and the Arthritis Foundation, in addition to individual patient advocates and caregivers.

Key themes from the session included:

- **Building trust:** Meeting patients where they are is essential to building trust, keeping them engaged in clinical trials, sharing their data, and being able to get the benefits from the clinical trial results. Ensuring that you are striving for fair and uniform care despite location, socio-economic background, ethnicity, and educational background is foundational for patient engagement.

- **Overcoming barriers to access clinical trials and contributing data:** One of the numerous barriers for patient engagement is access to clinical trials when trial sites are often located far away and patients and

their caregivers are therefore forced to travel at great distance to participate in the trials, have tests done, and report on their progress. The COVID-19 pandemic changed this as primary care practitioners and local hospitals were allowed to perform these activities. The pandemic also stimulated the evolution of remote trials and telehealth services. Maintaining local and virtual access is important for keeping patients and their caregivers involved and engaged.

- **Meaningful engagement and feedback is essential in everything patient organizations do:** Many times there is limited or no feedback to patients who have been involved in clinical trials, so they don't know what the results of the trial have been and whether their involvement brought value to improving outcomes for patients. Reporting back and providing meaningful feedback is important to show people that their contributions are part of something bigger that will do good and make a difference.

- **Addressing language issues, cultural and educational barriers:** Helping patients understand the complexities of clinical trials and healthcare delivery overall is important as patients may not have the capabilities in terms of language because English may not be their first language. In addition, few patients have the scientific training that will enable them to understand the science underpinning a clinical trial. There may also be cultural barriers as some with diverse ethnic backgrounds may be embarrassed to talk about their condition.

- **Sharing stories and experiences:** Encouraging patients to share their stories and experience is an important way for them to give back to other patients who may benefit and get hope from their positive experience.

- **Anticipating the changes that come with life-altering medications:** As chances of living longer increase, there is a greater need for support systems for many life events, such as financial planning or retirement. As health improves for those with chronic diseases, patients may be expected to start working again. Patients may also be disadvantaged by the years spent focusing on their health when they were not able to work or attend college. For patients with lifelong, chronic diseases, it is also important to enhance their understanding of the disease and what they may go through over time as their situation and needs may change.

- **Applying technology as a game changer:** Incorporating technology into patient care can enhance healthy patient interaction and deliver positive impact on patient care. However, generating the benefits from technology requires human support and can't replace the power of human interaction with caregivers and clinicians.

Listening to patients, engaging with them in an authentic way, and incorporating their needs into everything patient organizations do is foundational components for success. This also means providing feedback and reporting back to patients and caregivers so they know that their contributions are making a difference and ultimately benefiting the broader patient community.

2. Making patient advances together and faster: A practical guide to life science partnerships

Building and maintaining a good relationship between a patient advocacy organization and a life sciences company requires navigating differences in language, understanding variations in processes among sponsors, and open communication to ensure alignment around measurements of success. Several practical steps are essential for patient organizations to realize the ultimate vision of driving science forward together and faster with life sciences companies.

In this session, members of advocacy and life science organizations discussed examples of what a good relationship between a patient organization and a life sciences company looks like. By examining practical barriers to collaboration and mitigation considerations — things such as speaking a common language, assessing value of data and assets, data quality expectations, and navigating life science organizations — the goal was to educate and inform teams about areas to prepare and consider when investing in collaborations. The focus was on how to achieve an advocacy organization's mission while working

symbiotically with life sciences organizations whose vision and definition of success may differ. Panelists represented Parent Project Muscular Dystrophy, SMG Consulting, Cytokynetics, Susan G. Komen, and IQVIA Real World Networks.

Key themes from the session included:

- **Co-creating an opportunity:** One example of a successful collaboration addressed the challenges when patients with a chronic disease are discharged from the hospital. The project started with an open conversation between the industry partner and the patient organization about the issues affecting patients after discharge. This led to a positive solution around a particular chronic disease that could be emulated for other chronic conditions.

- **Engaging patients in the work:** Bringing patients into the work — via brainstorming sessions, for example – delivered great results for the launch of a new therapy for multiple sclerosis. The creation of a patient survey was driven by a patient group, which helped the life sciences company hone in on the symptoms that patients experience with the disease.

- **Creating a common language:** Having a common language is often a challenge for collaborations between patient organizations and life sciences companies. While the goals of a collaboration are often shared, specific terms may have different meanings. Some examples were shared during the session: The term "advocacy" is typically used by patient organizations for public policy engagements, whereas industry tends to view advocacy as the entire engagement with patients. "Therapeutic areas" are commonly used by industry while patient organizations talk about disease areas. It is important to be cognizant of differences in language, including the use of acronyms, when aiming to foster good collaborations. This is particularly challenging in global collaborations where team members on both sides need to be clear and explicit with the use of words and understanding the cultural differences.

- **Understanding differences in process among sponsors:** Life sciences companies may have different processes for collaborating with patient organizations and different protocols for compliance. Companies may also differ in how they want to contribute to a project. Furthermore, most companies prefer to have other sponsors involved to reduce their risk. Some companies focus on donations, others on sponsorships. It is important that patient organizations are proactive and ask direct questions about these issues before developing an application for a sponsorship or a donation, as a misunderstanding of process may lead to delays. Creating a profile and check-list specific to each company may be a helpful tool.

- **Addressing turnover:** One of the biggest challenges in collaborations is consistency with staff as there are often changes on both sides. The arrival of a new compliance officer in a life sciences company may mean a change in compliance protocol. Keeping an open conversation going (about potential changes in staff, for example), conducting regular check-ins, and building authentic relationships are important to address such challenges.

- **Learning from failures:** Co-creation was also applied in another example addressing the problem about disparities in research grants for breast cancer research. Instead of the knee-jerk approach to only share successes, this collaboration was focused on learning from failures that often generate the best insights. The project called out areas that did not work, such as weaknesses in the grants, and co-creation helped generate better materials for research grant applications.

- **Improving measurement:** The ability to monitor and measure outcomes of collaborations is very important to industry sponsors. It is not enough to look at traffic on a website. Evidence of meaningful impact is important; for example, whether an education campaign is motivating patients to go for a screening or to see a doctor. Impact is also very important to patients. Determining and sharing metrics for measurement of success are important prerequisites for driving value of collaborations between patient organizations and industry and metrics help validate whether a return on investment is delivered to mutual satisfaction for both sides in a partnership.

- **Bringing stakeholders together:** Getting stakeholders to align around common goals is critically important for success, especially when there are multiple partners. This also encompasses using technology to break down data silos and share data. This can help reduce the burden for patients and families as it reduces the time they need to spend sharing and accessing data.

- **Building a data warehouse – an example of successful collaboration:** The session discussed different examples of collaboration between patient advocacy organizations and life sciences companies. One successful pilot was focused on the development of a post-marketing surveillance study for a rare disease where the challenge was how to combine disparate data sources. The solution was the creation of a data warehouse where several industry sponsors could share data on the development of new therapies. The pilot was successful as it led to the development of several post-marketing studies that help with the understanding of the natural history of the targeted rare disease.

KEY TAKEWAYS FROM THIS SESSION:

Five practical steps are essential for accelerating the journey between a patient organization and a life sciences company to drive science forward together and faster:

- Mapping stakeholders to ensure knowledge of industry players across disease areas.

- Building authentic relationships looking for the right contacts and not refraining from asking basic questions.

- Networking with other organizations to learn about their experience working with industry.

- Staying up-to-date about important developments that may impact the collaboration.

- Sharing your successes through communication with existing and potential partners.

3. Putting patients at the center of innovation in clinical development and patient care

Incorporating patients and their insights at all stages of therapeutic discovery, research, clinical development, and care can help shape and improve the design of clinical trials, amplify the voice of patients, transform the lived experience of patients into evidence, and change the culture of healthcare research for all involved stakeholders.

In recent years, patient insights have transformed clinical research and therapeutic guidance by challenging the validity of endpoints, raising awareness about clinical trials, enriching understanding of patient burden, and creating new perspectives on what quality of life means to patients affected by diseases. Many patient organizations are now also launching sponsored initiatives to close the gaps in clinical innovation.

In this session, panelists shared their perspectives on how patients and advocacy organizations are playing an active role in shaping clinical innovations to address unmet medical needs of patient communities. Session panelists offered unique insights and lessons learned when incorporating the patient voices into clinical research, patient care, and access, and examining how patient-centricity provides the ability to impact the development and adoption of new therapeutics. Furthermore, priorities and perspectives from a nonprofit research organization were added to the conversation. Panelists represented National Brain Tumor Society, Patient Centered Outcomes Research Institute, Cystic Fibrosis Foundation, National Hemophilia Foundation, and IQVIA Pediatric and Rare Disease Center of Excellence.

Key themes from the session:

How patients themselves influence and shape clinical trial design

- One national patient society is focused on mobilizing and uniting its patient community to discover a cure, deliver effective treatments and advocate for patients and their care partners.

- An important mission for the society is to make research better and more powerful, trying to bring forward transformative treatments for patients.

- Patient advocates are critical players in this endeavor as they are active in influencing and shaping clinical trial design.

- Four different patient stories were presented as examples of how different patient advocates have positively changed the way research is being conducted.

Amplifying the patient voice as crucial to all research efforts

- An international chronic disease foundation has ongoing activities in multiple areas, including a national patient registry, a care center accreditation encompassing 280 programs at 130 centers, a learning network, a therapeutics development network, and patient support.

- The foundation views the patient voice as crucial for all research efforts and works to amplify the patient voice in drug development by emphasizing quality of life, ease of use, reduction of time and administrative burden, and the cost burden.

- One of the major priorities for the foundation is to reduce the economic burden of the disease to patients and their families. The cost of care is a challenge no matter the income and age of patients, as patients have to spend approximately two hours on treatments per day in addition to lengthy care visits and hospitalizations. Furthermore, insurance is not fully protective of all costs, including significant costs of transportation.

- The foundation uses patient-centered data to advance the understanding of social, economic, and access barriers for patients and their families.

Transforming the lived experience into evidence

- A national foundation is dedicated to finding cures for disorders and to addressing and preventing complications of these disorders through research, education, and advocacy, enabling patients and families to thrive.

- The foundation views patients as partners and as human subjects in research, subject matter experts who are engaged to ensure that their lived experience is at the center of everything the foundation does.

- Through its community powered registry, the foundation collects quality of life data with the goal to transform patients lived experience into evidence.

  The patient registry has several functions:

  – Helps define what it means to live with a bleeding disorder.

  – Identifies research opportunities.

  – Encourages participants to become active partners in research and their health care.

– Serves as a mechanism to recruit and promote other patient-centric research opportunities.

  As an example, the patient registry was utilized in a survey of patients and physicians to help define poor outcomes. Interestingly, physicians defined poor outcomes mainly in clinical terms, such as death and disease. In comparison, when patients were asked the same questions, they were talking about more practical examples of poor outcomes, such as reproductive choices, financial issues, and how the disease affects their family and travel. Both physicians and patients provided important perspectives, but they were quite different.

- The foundation activates patients as lived experience experts (LEE) in all aspects of research, community engagement, policy, and activities around health equity, diversity, and inclusion.

Putting patients at the center of clinical innovation and patient care

- An independent research institute has as its mission to help people make informed healthcare decisions and improve healthcare delivery and outcomes, and by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

- The institute commissions and funds research that is patient-centered and engages patients, clinicians, and other stakeholders from across the entire healthcare enterprise – from the development of research priorities to the dissemination and uptake of research findings.

- Examples of funded research projects include:

  – Enhancing participation in a study to evaluate interventions to improve the quality of life for Latina breast cancer survivors.

  – Assessing the benefits and long-term effectiveness of adaptable aspirin dosing.

  – Comparing the effectiveness of non-surgical treatments for lumbar spinal stenosis in reducing pain and increasing walking ability.

Overall, a meaningful partner engagement delivers value in multiple ways:

  – Influences the design and conduct of research studies.

  – Impacts research in meaningful ways.

  – Makes partner involvement dynamic, non-linear and iterative.

  – Benefits the patient and stakeholder partners, communities, and researchers involved.

  – Influences the culture of healthcare research.

  – Addresses the known challenges, including those related to infrastructure and resources, people and teams, organizational policies, and balancing views and priorities.

  – Enhances the learning regarding methods and impact of inclusive engagement.

KEY TAKEAWAYS: INCORPORATING THE PATIENT VOICE IN ALL ASPECTS OF RESEARCH

Incorporating patients, their insights and voice in all aspects of therapeutic discovery, research, clinical development and care is critically important for designing clinical trials, translating the lived experience of patients into evidence, and changing the culture of healthcare research for all involved stakeholders.

Amplifying the patient voice and putting patients at the center of innovation in clinical development and patient care is ultimately about improving health outcomes for patients.

4. Making best bets: Prioritizing expenditure for maximizing impact on patient communities

Patient organizations are best positioned to make their decisions on the most important areas for investment based on a strategic understanding of where to achieve the most impact for their patient community while acknowledging that culture matters more than size. The power of collaborations can "leverage the dollars," and investing in people and the organization is critically important for maximizing impact.

Balancing the competing priorities of investing in research, services, fundraising, and running an effective organization has long been a challenge for patient organizations. This session explored how patient organization leaders are investing where they can have maximum impact for patient communities — both now and in the future. With a particular focus on medical research funding, the session also explored how these leaders are prioritizing investment

of precious resources in a way that is informed both by the research landscape and patient views.

Panelists represented Friedreich's Ataxia Research Alliance, Association of Medical Research Charities, Juvenile Diabetes Research Foundation, and FasterCures (Miliken Institute).

Here are highlights of the perspectives during the session:

PATIENT ADVOCACY ORGANIZATIONS EVOLVE AS SCIENCE MATURES AND TREATMENTS ENTER THE PIPELINE

- **Pivoting from funding basic research to investing in treatments:** In the past, many patient advocacy organizations focused primarily on funding basic research in their disease(s) of interest. Originally many of the groups were small, grassroots, and volunteer-run, and this model still exists; however, progress in the basic scientific understanding of many of these diseases has prompted organizations to evolve and take a more active role across the development pipeline. This may mean working more actively with life sciences organizations to support a patient organization's financial or data needs, and/or working more closely with regulators to educate them about the needs and preferences of patients.

  – A large patient organization in the U.S. focused on a chronic disease used to be a solely discovery-based research organization with a focus on finding a cure for the disease. Initially, the organization realized that there is not just one pathway to find a cure for the disease, but multiple pathways. Furthermore, the organization recognized the need to advance treatments for the disease while still waiting for a cure, and pivoted to also funding research regarding the development of therapies to improve outcomes and the quality of life for patients. At first, this was a controversial shift in the focus of the organization, but as scientists within saw the opportunities for using new technologies to improve the treatment of the disease, the organization began a process of considering investing in a treatment in addition to

continuing to invest in research to find a cure. The board performed an entire landscape analysis to assess what would happen if the organization did not pursue this opportunity, and ultimately decided to pursue the therapy in a targeted, strategic way. The decision led to the creation of new partnerships and investment in new areas of expertise, including regulatory and health policy expertise. The decision paid off for people with the disease as there are now new technology-based therapies on the market that generally are reimbursed by health plans and are enabling patients to have better control of their disease and improve outcomes.

- **Shifting focus to access to therapy:** A few organizations have also found themselves in the position of having treatments and/or vaccines for their disease come to market. In these cases, organizations are still focused on serving patients, but they tend to pivot toward focusing on access to treatments as well as advocating for those patients who are not served by the available treatments and/or vaccines, and therefore still need new options.

- **Generating new research and therapies based on patient insights:** Another way patient organizations are evolving as the science and treatment landscapes mature is an increased focus on ensuring patient priorities are reflected in research and funding decisions, rather than relying solely on investigator-initiated questions. Many organizations have begun surveying their patient communities to surface which issues are most important to them, and then designing new initiatives to address the needs that surface. Examples include:

  – An arthritis organization in the UK found chronic pain was the top complaint for patients, and now they have made it a key research funding priority for their organization.

  – A Parkinson's disease foundation has a robust patient platform that supports patient surveys. Through a survey, they identified the Parkinson's symptom 'off time,' which happens between scheduled doses

of medication, but no one was paying attention to it. When they started asking more about it, that symptom rose to the top of patient priorities, but no one in the academic research or industry communities was working on addressing it. The foundation created a funding stream to develop further treatments for this patient need, and now there are products on the market for that symptom.

FORWARD LOOKING PATIENT ORGANIZATIONS RECOMMEND A HOLISTIC PIPELINE VIEW TO DETERMINE OPTIMAL INVESTMENT STRATEGY

- **Taking a broad view:** To prioritize investments for maximum impact, panelists agreed that taking a broad view of the full treatment development pipeline, from basic science to translational research to clinical trials and finally regulatory approval and market access, was needed to ensure that the appropriate barriers and bottlenecks along the pathway were identified. This is not to say that patient organizations should take an active role in removing every barrier along the development pathway, but rather that an awareness of the full pathway is a necessary starting place. Once a full view of the barriers along the pathway is achieved, patient organizations ask themselves a few key questions to guide investments:

  – What is the outcome we are looking for? What needs to change to achieve that outcome?

  – What will happen if we do nothing?

  – Which gaps or barriers need our concerted attention? Which will be addressed by someone.

  – Which investments would be best aligned with our mission and culture?

- **Understanding that 'no one size fits all':** There is not a single recipe for success. All organizations are dependent on the differences in the state of science, the condition the organization serves, and the particulars of the organization. The best bets for investing time, money, and other resources will look different for every organization, depending on the treatment and scientific landscape, the organization's size, budget, and appetite for risk, and the priorities and activities of other stakeholders in that disease space. Additionally, successful organizations tend to re-evaluate their priorities and investments every few years to ensure they are being nimble and responsive as the landscape continues to evolve.

- **Realizing that culture is more important than size:** There is a common belief that the size, amount of money, and footprint of the organization are what matter most. But participants in the panel agreed that culture matters more than size, a view that was shared across large and small organizations. Being nimble, disciplined, and willing to respond quicky to opportunities were thought to be more important as drivers of success than size.

- **Determining areas of investment with most impact:** Making decisions on which areas are most important for a patient organization can be daunting as there are so many unmet needs and gaps to overcome. Overall, panelists agreed that decisions on investment priorities should be driven by considerations on where investment will have the most impact. Panelists shared a range of areas of investment they have recommended, including:

  – Research funding focused on patient priorities.

  – Domain-level resources that can benefit multiple stakeholders, such as patient registries or clinical trial hubs, but where the patient community prefers that the organization is taking the lead.

  – Supporting early-career scientists in your disease area.

  – Investing in your organization internally to ensure that your programs are appropriately resourced.

  – Investing in building a patient community (if you have not already) – future opportunities to work with and support industry will require patient organizations to have strong connections with their patient communities to engage them in research.

- **Leveraging the dollars through collaborations:** The most impactful research investments are the ones that persuade other institutions to invest in research, for example by going to Congress to persuade the federal government to conduct research, or motivate private investors, companies, or broader advocacy organizations to take on an issue.

IN ADDITION TO FINDING THE BEST PLACES TO INVEST, IT IS ALSO IMPORTANT FOR PATIENT ORGANIZATIONS TO KNOW WHEN TO STOP INVESTING

- **Stopping investments:** Panelists shared a couple examples of when organizations decided to stop investing in a particular area:

  – A rare disease organization initially pursued a "venture philanthropy" model, where they provided resources to a biotech company which needed support to continue the development of their therapy. After entering into this investment, however, the organization realized that investing so heavily in one particular company, with the potential of a payout if the company's product succeeded, may have made good financial success, but it was detrimental to their overall culture. The organization wanted to be able to support each potential industry player and science-backed treatment equally, rather than having a vested interest in one particular product. They exited from that arrangement and have not pursued venture philanthropy again. Instead, the organization pivoted to creating a business development strategy, including developing relationships with venture capital funders and other large pharma companies to make sure they are aware about the opportunities in the space of the rare disease.

  – A melanoma organization had been an early supporter of immunotherapy research and development. As the field progressed, more and more industry players and other research organizations started developing and funding

immunotherapies, and the organization realized their support was no longer needed. They stopped funding immunotherapies and directed those resources toward other priorities.

KEY TAKEAWAYS ABOUT LESSONS LEARNED:

- Think and probe deeply about the impact you are trying to drive, the outcome you want to achieve, and for whom you want to achieve that impact.

- Analyze the options for achieving impact. Then focus on how you can realistically achieve that impact.

- Acknowledge the power of collaborations, especially with governments, industry, or other patient organizations. If you want to go fast, travel alone; if you want to travel far, travel with others.

- Embrace that culture matters more than size and that being nimble and disciplined may generate more impact than having a large footprint.

- Understand that knowledge is power and that informed patient communities that can participate as peers and opinion leaders with industry, regulators, and academics are powerful. Your organization is the ultimate source of knowledge about your patient communities.

- Invest in your organizational capacity. It is hard to make that transition from raising research money to investing in the organization, but if you want to go far and achieve results more quickly, you need the people and the support to do that.

5. Evolving real world evidence to elevate the patient voice and move toward patient-centricity

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Turning patient-generated health into evidence and translating patient voices into metrics are fundamentally important when elevating the patient voice in research. Capturing insights that matter to patients requires direct engagement with patients and recognizing that each patient is unique and different. This means aggregating information from many different patients and weaving them together to generate truly patient-centric evidence, ultimately translating the patient voice into meaning metrics and a format that regulators and the healthcare community can understand.

As the drug development landscape is evolving, the impact of real world evidence and its role to improve patient health is more important than ever. In parallel, our healthcare industry is continuously striving to be more patient-centric. Increased efforts to improve the quality of real world data provide an opportunity to increase availability and quality of patient-generated health data. The panel session explored how real world evidence is evolving in a direction that helps patient organizations meet their missions to bring therapies to patients, improve lives and outcomes, and ensure that patient needs are met. Panelist organizations were Clinical Trials Transformation Initiative (CTTI), Sarepta Therapeutics, NKH Crusaders, and Foundation Fighting Blindness.

Key themes from the session included:

- **Understanding stakeholder needs:** For patient advocacy leaders to use real world evidence in the best possible manner, it is important to understand the needs of the different stakeholders: What data is most relevant to researchers? What really matters to patients? How can you aggregate authentic patient voices from a variety of data sources, EMR-data, claims data, and patient generated health data?

- **Turning data into evidence:** Turning data into evidence that really matters to patients requires involvement directly with patients. If the authentic patient voice is not included, important evidence is missing.

- **Capturing patient variations:** When aggregating evidence from the patient voice, it is fundamentally important to recognize that patients are different. What matters may be different from one patient to the other, and some patients may be more willing to take risks than others. Some children can articulate their experience with a disease better than others. The challenge is how to capture these variations in different patient voices and weave them together authentically and meaningfully. Blending diversity and harmony is the real challenge.

- **Applying technology in a patient-focused manner:** Technology can serve as a powerful enabler of capturing and aggregating the patient voice, using the plethora of technology tools that are available – from wearables, sensors, and smart phones – to make the patient experience come alive. However, technology can set a patient organization up for failure if it is

pushed down on patients. Technology is merely an enabler and meaningful use requires human touch and support for patients to understand and embrace technology and generate benefits from practical use.

- **Engaging directly with patients:** Involving patients directly is critically important for determining which data (for example, patient reported outcomes) are most relevant to patients. There are fundamental elements of the patient experience with a disease and the use of drugs that does not show up in clinical trials and through clinical care, and there are important observations and insights that researchers may miss. Patient organizations can be important advocates for engaging directly with patients.

- **Determining relevant longitudinal endpoints:** Defining the most relevant longitudinal endpoints that matter most to patients can be challenging, especially when patients live longer. It is easy to collect survival data but determining what is most relevant to the patient over several years can be more difficult. Celebrations of personal milestones and events may provide important insights (for example, whether a child living with a rare disease is able to go to the prom or go to college). This also requires identifying ways to motivate patients to continue to provide feedback over the years. Patients tend to forget when they are feeling better and making it valuable for them to contribute with stories about their lived experience to help others can be a useful approach.

- **Translating voices and noises into metrics:** Capturing and aggregating patient-generated health data, filtering out the noise, and turning voices into metrics that matter to a regulatory body represent a unique challenge. Therefore, it is helpful that the FDA has developed guidelines for the application of real world evidence, but translating patient voices into meaningful insights that matter to patients is about more than structuring the data the way FDA prefers; it is ultimately about deciphering insights about patient needs and issues that are relevant for determining

which therapeutic interventions and patient services that potentially will have the most impact on enhancing health outcomes and improving the patient experience.

- **Conducting patient evaluation of clinical trials:** During the panel discussion, the idea was brought up about creating a patient evaluation scorecard to assess whether a clinical trial is truly patient-centric. This may help to add positive elements to surveys and trial designs so that endpoints are not limited to negative aspects of the disease but focus on celebrations of patient achievements, such as whether a child can stand or eat. In other words, making a celebratory moment a KPI.

- **Ensuring protection of the privacy and integrity:** When patient data is being used in the application of real world evidence in research, it is vitally important to ensure the privacy and integrity of the information – both to ensure that patient data is handled securely and protected against breach and misuse, and to enhance public trust in the interpretation of the authenticity and accuracy of the data.

KEY TAKEAWAYS ABOUT ELEVATING THE PATIENT VOICE

Evolving real world evidence to elevate the patient voice has the powerful potential to accelerate progress toward patient-centricity. This requires:

- Giving patients the "microphone" and an opportunity voice their experience.

- Translating the patient voice into metrics and structured research insights.

- Capturing insights that matter to patients, regulators, and healthcare community.

- Highlighting the unqiue differences among individual patients.

- Aggregating disparate data about diverse patient segments and individuals and weaving them together to generate truly patient-centric evidence.

6. Looking ahead for patient organizations: How can tech help?

Technology has the potential to help patient organizations transform care and research, bring patients to the center of research, shift the focus from the data to the way data is being used by researchers, regulators, and consumers (including patients), and bring people and organizations together to improve health. In this context, patient organizations can help shape these technologies and their use to better deliver value to patients and caregivers.

Over the next five years, we can expect to see the further expansion of technologies that have the potential to transform approaches to research and advance therapeutics and cures for patients. Patient organizations have a critical role to play in understanding, leveraging, and informing these technologies, and bringing full value to their constituents.

This session's panelists discussed how technology applications are shaping the future and brought together leaders of tech companies with a common interest in supporting patient organizations to achieve their goals. Panelists represented Digital Medicine Society, Microsoft, Meta, and IQVIA Global Strategy.

Key themes in the session included:

- **Transforming care and research with technology:** Today, patients are surrounded by an expanding array of technology-driven tools that can be transformative for care and for research. Technology can help in a plethora of ways:

  – Building and sustaining social communities.

  – Decentralizing care delivery.

  – Democratizing research through registries and data platforms.

  – Developing and deploying predictive analytics.

  – Defining and validating digital biomarkers.

  – Generating and harnessing the power of digital technologies.

- **Bringing patients to the center of research:** According to the Digital Medicine Society (DiME), technologies offer the potential to bring patients to the center of research by providing digital measures that matter to patients, including data on personal choices and preferences, outcomes to be measured relevant to different symptoms, and endpoints that may be supported by multiple sensors.

- **Shifting the focus from the data to the way consumers use them:** Technology is helping facilitate a paradigm shift in the way consumers interact with health and healthcare to enhance the consumer health experience. Microsoft sees six different pillars in this paradigm shift:

  – Enable consumer-centric operating models to meet market demand.

  – Provide personalized, high quality, convenient service.

  – Deliver the consumer an intuitive and just-in-time service.

  – Liberate data to drive meaningful employee and customer interactions.

  – Create customers for life with full spectrum, longitudinal experiences.

  – Respond to consumer demand for a healthcare ecosystem that recognizes choice.

- **Evoking the different stages of the patient journey:** Technology also helps evoke the different stages of the patient journey:

  – Health, wellness, and prevention.
    - Seeking health and wellness information
    - Using wearables and trackers
    - Participating in health-related activities
    - Utilizing retail health services
  – Illness – diagnosis and treatment.
    - Remote interactions with providers
    - Accessing and sharing electronic health records (EHR)
    - AI-based provider support
    - Care team collaboration

  – Maintenance, equilibrium.
    - Filling, refilling, and approving prescriptions
    - Remote health monitoring
    - Clinical trial participation
    - Value-based care or health coaching

  – Connected ecosystem.
    - One data record across consumer health footprint
    - Net new insights via analytics
    - Secure collaboration healthcare ecosystem

- **Bringing people and organizations together to improve health:** Technology has the potential to bring people and organizations together to improve health. This is the aim of Meta (formerly Facebook), and they are doing this by:

  – Working with experts to help people find reliable and helpful information and remove harmful content.
  – Giving people new ways to look after themselves and look out for others.
  – Providing public health officials with real-time data to inform their constituents.

- **Creating new research paradigms for patient centricity:** Technology can help research evolve to support new designs — and even new paradigms — that embrace and promote patient centricity. IQVIA shared examples from its innovative partnerships with patient organizations, highlighting how far patient-centric approaches in observational research have evolved over just the past decade. These enabling technologies also have the potential for disruption in clinical development through their use in decentralized clinical trials that, according to IQVIA, are heralding a new patient-centric paradigm in clinical research.

KEY TAKEAWAYS ABOUT THE TECHNOLOGY-DRIVEN TRANSFORMATION IN CLINICAL DEVELOPMENT AND PATIENT CARE

The application of technology in healthcare is ubiquitous as it has the potential to transform all aspects of healthcare, including basic scientific discovery, pre-clinical, clinical and translational research and development, patient therapy, delivery of care, and access to care.

Therefore, patient organizations have the opportunity to generate game-changing value from utilizing technology to achieve their goals.

Patient organizations can also play an important role in shaping technologies to better generate value for patients and caregivers since patient organizations work in the intersection of technology and patient care, and have hands-on experience harnessing the benefits of technology as well as mitigating the challenges when using technology.

Speakers

Arthritis Foundation
Cristina Schaefer, Arthritis Patient Advocate,
Patient Leadership Council

Breast and Ovarian Cancer Patient Advocate
Jo Nunn

Clinical Trials Transformation Initiative (CTTI)
Morgan Hanger, Director of Strategy

Cystic Fibrosis Foundation
Olivia Dieni, Health System Innovation and Navigation
Senior Specialist

Cystic Fibrosis Patient Advocate
Reid D'Amico, Ph.D.

Cytokenetics
Mary Pomerantz, Senior Director Patient Advocacy and
Engagement

Digital Medicine Society (DiMe)
Lucy Cesnakova, Program Lead

Foundation Fighting Blindness
Steven Ringel, co-chair, Strategic Council

Friedreich's Ataxia Research Alliance (FARA)
Jennifer Farmer, CEO

**JDRF (formerly the Juvenile Diabetes
Research Foundation)**
Cynthia Rice, Chief Mission Officer

Meta
Roni Zeiger, Head of Health Strategy

Microsoft
Antoinette "Toni" Thomas, U.S. Chief Experience Officer
& Industry Advisor

Milken Institute
Kristin Schneeman, Senior Director, FasterCures

National Brain Tumor Society
David Arons, CEO

National Hemophilia Foundation
Maria Santaella, Director of Research

NKH Crusaders
Kristin Archibald, Parent Caregiver and Founder **Parent**

Project Muscular Dystrophy
Megan Freed, Director, Data and Technology Strategy

**Patient-Centered Outcomes Research Institute
(PCORI)**
Jason Gerson, Senior Program Officer

Project Sleep
Julie Flygare, JD, President & CEO, Narcolepsy
Patient-Perspective Leader and Patient

Progressive Surpranuclear Palsy Caregiver Advocate
Carmelo Gordian

Sarepta Therapeutics
David Miller, Executive Director, Head of Real World
Evidence

SMG Consulting
Susan Gorky, Principal

UK Association of Medical Research Charities
Nicola Perrin, CEO

Speakers

IQVIA SPEAKERS

Murray Aitken, Executive Director,
IQVIA Institute for Human Data Science,
Summit Executive Sponsor

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

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Gregory Dennis, SVP,
Public Health & External Affairs

Harvey Jenner, Principal,
Real World Networks

Sarah Johnson, Head of Patient Advocacy,
EMEA

Christina Mack, PhD, Chief Science Officer,
Head Integrated Surveillance and Agile Analytics

Ali Smyth, Senior Director,
Pediatric and Rare Disease Center of Excellence

David Voccola, Senior Principal & Strategy Lead,
Integrated Health Practice

Alexandra Weiss, Director,
Nonprofit Strategy, Patient Advocacy

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:
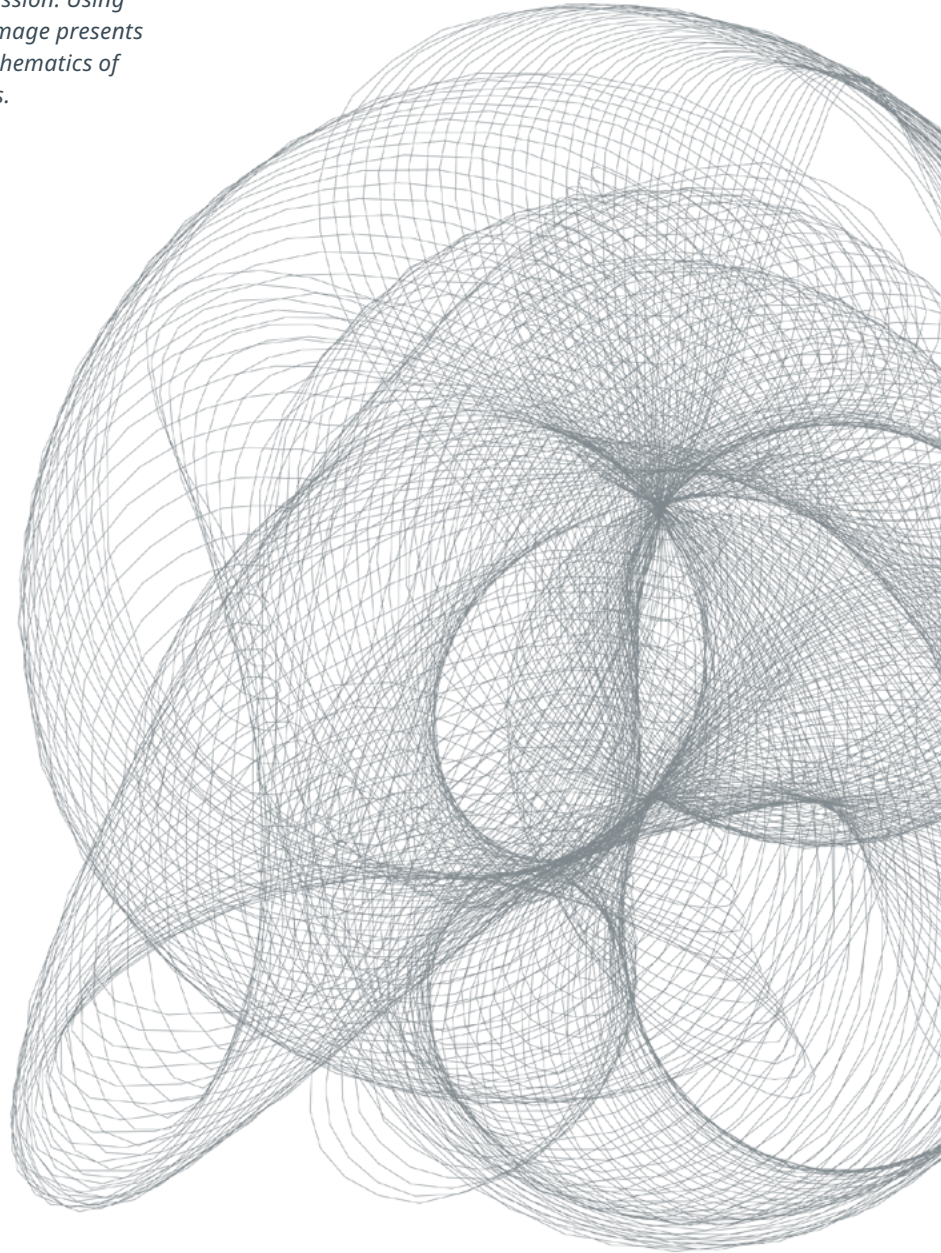
- Improving decision-making across health systems through the effective use of advanced analytics and methodologies applied to timely, relevant data.

- 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.

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