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# PATIENTS AS PARTNERS

# EUROPE 2020

February 2020

# Patients as Partners Europe 2020

## The 4th annual Patients as Partners EU was the first European event dedicated to:

> How to involve patients throughout the entire medicine development lifecycle

> How clinical trials have progressed thanks to patients' feedback.

The program was co-produced with industry, patient advocacy and public sector voices. Each session put ideas into action.

PATIENTS SPEAK TO HELP CLINICAL RESEARCH

# PATIENT KEYNOTE GUEST: INSIGHTS AND LESSONS FROM A PATIENT ADVOCATE WHO EXPERIENCED THE GDNF TRIAL

**Lesley Gosden.** *Parkinson's patient and advocate*

Lesley was diagnosed with Parkinson's disease (PD) in 2004, and the condition has had an immense impact on her and her family. PD dictates her entire lifestyle and she regularly experiences muscle twisting and exhaustion, affecting her dignity and self-esteem.

Lesley took part in a clinical trial for PD, which consisted of an experimental delivery device (so the drug could cross the blood brain barrier), an experimental surgery and an experimental drug that was compared with placebo. She stated that most patients who received the experimental drug showed life changing improvements and the trial reminded her of what living truly meant. Her symptoms have since remained stable for the last 3 years. Despite these seemingly positive results, the trial failed to meet its primary endpoint. Unsurprisingly, this left the participants who had seen a benefit with a feeling of huge disappointment.

Lesley described the trial as invasive due to the amount and length of scans and procedures. Speaking on behalf of the other patients who took part, Lesley described how the study had failed because it did not put strong emphasis on patient reported outcomes (PROs) or quality of life. Lesley felt that the physical symptoms assessed in the study were open to interpretation, and that the healthcare professionals would often struggle to rate these on the scale used. This could have impacted the results if data weren't accurately captured. Lesley also noted that vital symptoms weren't included in the study assessments, including dystonia, and that she went from needing a wheelchair to get around the study site to being able to walk there.

Those who felt they benefitted from the study agreed a bigger emphasis should be on PROs and quality of life. PROs empower patients to share their own views about their health and wellbeing and allow truly meaningful data to be collected. Standardised tests that have been used historically do not necessarily capture the essence of PD. Researchers need to understand the true reality of living with a condition during the study planning stages. For example, before the trial, Lesley took her PD medication in the morning and had to wait an hour for it to kick in. For her, that was an hour where she could physically not get out of bed.

## Key takeaway

> Ultimately, asking the wrong questions can lead to obtaining the wrong conclusions. This can have a drastic impact and PROs should therefore have a strong presence in research.

# ASK THE PATIENT PANEL: **MY IDEAL TRIAL DESIGN**

**Matt Eagles.** *Parkinson's Patient and Advocate*
**Lesley Gosden.** *Parkinson's Patient and Advocate*
**Alfred Samuels.** *Prostate Cancer Patient Advocate*
**Andrew Warrington.** *Type I Diabetes and Digital Therapeutics Patient Advocate*
Moderator: **Kay Warner.** *Patient Engagement Lead, GSK*

Patient advocates took the lead in this panel, where they talked through how they would design the ideal trial and their views on giving feedback.

## The ideal trial design

The patients felt that better communication was vital in clinical trials, noting that too much jargon is used in informative materials. Transparency between everyone involved in research is crucial, and all organisations and individuals involved in clinical research should talk with patients, rather than to them. The way research findings are distributed to patients was also discussed, with one patient describing how he never received correspondence with the outcome of the trial he took part in. This meant he was discouraged from taking part in future clinical trials. The follow up period and data sharing are more important than ever, with many patients now being more aware of their own rights and wanting to own their own data and healthcare information.

It was suggested that study sponsors should pay for patients to take a partner, family member or friend along to any clinical trial appointments, to help make the process less overwhelming. Issues such as diversity were also raised. The patients discussed how trials need to focus on what is really important to patients and quality of life, and consider the patient from a holistic point of view. Further, it was highlighted that clinical trial designs are too reliant on what has been done in the past; designs should be more flexible and trials should be as easy, comfortable and attractive as possible.

## Providing feedback

The majority of patients said that their feedback has never been requested and all patients felt that qualitative feedback would be valuable. Carers should also be able to provide feedback, particularly for conditions where carers might notice symptoms (such as memory loss) that the patients themselves would not necessarily be aware of.

## Key takeaways

> Communication needs to improve between all parties, patients' whole lives and quality of life need to be considered more in clinical trial designs, and patients and carers should always be given the opportunity to feed back on their experiences.

# JOINT PATIENT PERSPECTIVES ON HOW TO BEST COLLABORATE WITH INDUSTRY FOR PATIENT BENEFIT

**Alain Cornet.** *General Secretary, Lupus Europe*
**Avril Kennan.** *CEO, Health Research Charities Ireland*
**Natasha Ratcliffe, PhD.** *Research Involvement Manager, Parkinson's UK*
**Jon Spiers.** *CEO, Autistica*
Moderator: **Ana Maria Arboleda, MBA.** *Shard Next Generation Patient Engagement Lead, Rare Disease, Pfizer*

## Patient engagement in industry and current challenges

The panel highlighted that patient engagement should start as early as possible in the clinical trial process and should be meaningful (not just a box-ticking exercise). An education process is needed among all organisations where patient engagement should exist to show how effective it can be. It's important for organisations and patients to have a shared understanding of why they are working together. Understanding that co-creation means shared responsibility and shared decision-making is very important int building long term partnerships.

There was also discussion around the concept of expert patients. The panel expressed concerns about expert patients becoming so far engrossed in the world of patient advocacy groups, the pharmaceutical industry and patient engagement conferences that they are no longer representative of the general patient population. Additionally, patients who are not working with patient advocacy groups or charities should be recognised as experts – they have expertise in their condition but currently this isn't recognised. Overall, the focus on non-trained patients needs to continue.

Again, it was noted that the 'why' of patient engagement needs to be focussed on and possible outcomes needed to be considered. Organisations need to reach a broad range of people who are representative of the general disease population and tailored approaches are needed. Engaging with people who have received a lower level of education has proved a difficult challenge. People are often not aware that they can participate in research, but a solution to overcome this is to engage people with their patient community, so they can become aware of best practices and options like clinical trials.

## Achieving smooth, efficient, complaint and auditable data gathering

One of the major changes that has come with the latest technology is that everyone is now consuming their own data. This affects how people think they should access their healthcare data, and people are increasingly aware of their rights. By taking part in clinical trials, data are collected about patients that they wouldn't otherwise have. It was suggested that organisations should sell this as a benefit to taking part; participants can learn something about themselves and this could benefit them.

Strong emphasis was again placed on the power of qualitative data collection and how it can help study sponsors understand the emotional and practical aspects of a condition. In addition, independent parties should be employed to obtain feedback from patients and caregivers throughout the clinical trial process, from eligibility checks through to follow-up.

## Key takeaways

> Increased understanding around patient engagement is still needed throughout the industry – people need to know why they should engage with patients and how impactful this can be. Organisations need to be transparent and share their best practices so that the industry can move forward.

PATIENT JOURNEY MAPPING

# HOW TAKEDA IS USING DIGITAL JOURNEY MAPPING TO DESIGN PATIENT-LED TRIALS

**Ilaria Piubelli.** *Global Patient Engagement, Takeda*
**Lu Zheng, PhD, MBA.** *Senior Director, Head of Global Strategic Patient Services, Takeda*
**Dumitru Drumea.** *Head of Consulting, Observia*

Through a design-thinking approach, Takeda created and implemented a digital journey map to ensure a continuum of care throughout the medicine development life cycle for patients. Takeda shared how and why they developed this approach.

Takeda have developed platforms for patients and caregivers that provide information, and they are continually learning how best to communicate with people in a personalised way. One example of this is in a study that Takeda are currently running. Patients in the study commonly experience morning brain fog, which makes it very difficult to complete PRO questionnaires. Therefore, Takeda empowered caregivers to support the data collection process. In another study in pre-term babies, PRO questionnaires were originally being administered daily, and this was identified as being extremely overwhelming to the parents. Takeda took this feedback on board and re-worked how data was collected.

Personalised engagement plans can include how much detail is given in guidance as well as the tone of voice. For example, in a reminder tool, some patients would like to be told 'take your drug' and others would prefer 'don't forget to take your drug!'. Ethnographic research was performed by professionals so Takeda could fully understand what the patient and their caregiver is experiencing as a result of their condition, to develop a robust solution.

## Key takeaway

> It is important to understand the current unmet needs in the patient journey, otherwise there is no chance of success.

# REPRESENTING THE PATIENT VOICE TO CAPTURE RWD TO HELP INFORM DRUG DEVELOPMENT & DISEASE AWARENESS

**Jeremy Sayers.** *Managing Director, Origins Insights*

Real world data and evidence are playing a bigger role to support clinical trial design and clinical trial decision-making. Capturing the patient voice as authentically as possible on how they live with a disease is crucial in supporting RWD/RWE. Origins Insights shared how they capture and represent the patient voice and how this can impact clinical research.

Origins Insights engage with patients through patient advisory boards, research and conferences, but the real insights can be gained from seeing a patient in their own environment. This can help to understand what life is really like for patients as individuals, rather than how they act in a focus group in a corporate location.

Ethnography is the main tool Origins Insights use, which can be utilised throughout the entire product lifecycle. Patients are videoed in their own home (either with someone there, or they video themselves) to give the opportunity for patients to describe their condition and how it affects them, while removing the burden of having to travel. Videos also capture emotion and the impact of physical symptoms on patients in a way that a quantitative survey cannot, and these insights can help identify and understand target populations as well as shape the most effective study design.

Regulatory authorities are working to give patients a greater voice in the development and evaluation of medicines. Other major organisations are now recognising the value of patient experience data, going beyond the value to patients and caregivers. It is also becoming clear that understanding the patient experience can help reduce the burden of clinical trials, and prevent delays and subsequent costs.

## Key takeaway

> Engaging with patients in their own environment provides insights that can be used to understand how patients are impacted by their condition.

# PHARMA PERSPECTIVE: HOW PHARMA IS COLLABORATING WITH THE DUCHENNE PATIENT COMMUNITY TO BOOST DRUG DEVELOPMENT

**Vanessa dos reis Ferreira, PhD, MBA.** *Head of Patient Advocacy Europe, Santhera*

Santhera explained how they set up a Systematic Patient Engagement (PE) Framework to continuously listen to what matters to people living with Duchenne muscular dystrophy (DMD) and their family members.

Santhera performed cross-function listening in different countries, and identified many challenges: there is a lack of awareness about DMD, a lack of multidisciplinary teams, patients who are geographically dispersed, limited knowledge of the patient journey and the patient perspective, and limited clinical trial awareness among patients and their families.

After listening to DMD patient groups, Santhera realised that dots needed to be connected and questions answered, such as could DMD education materials already used in one country also be used in another?

Santhera received input from organisations such as WHO and EUPATI, and regularly communicated with patient groups to help develop the framework. Patient groups were identified through desk research, and it was established that their profiles focus on research, care, community building, education and advocacy. The framework also allowed Santhera to identify where patient groups are involved throughout the design of a study.

Through the development of the framework, Santhera found that mixed methods research has particularly high potential for rare diseases like DMD and allow the whole picture to be captured. Santhera conducted surveys to gather broad, initial data, and were followed by face-to-face meetings and interviews to gain a deeper understanding of key themes. Santhera spoke to families affected by DMD and have shared the findings of their research at many conferences.

The framework has transformed the internal cross-functional work at Santhera and has helped to increase awareness of DMD. In conjunction with Pharmaforum, interviews have been conducted and led to 10 articles being published from 2018 to 2019, reaching 25,000 views.

## Key takeaways

> Patient engagement methods should be used alongside scientific data to understand the whole picture of a condition. Frameworks can be used to increase understanding and awareness of rare conditions.

# PATIENT PERSPECTIVE: DUCHENNE PARENT PROJECT SPAIN ON HOW THEY COLLABORATED TO BOOST DRUG DEVELOPMENT FOR DUCHENNE'S MUSCULAR DYSTROPHY

**Marisol Montolio, PhD.** *Scientific Director, Duchenne Parent Spain*

Duchenne Parent Project España (DPPE) is a non-profit organisation set up in 2008 by parents of children affected by DMD. They work to find a cure or treatment for Duchenne and Becker muscular dystrophy and improve the quality of life for those affected and their families by promoting and financing research clinic, psychosocial care services, awareness campaigns and educational programs.

From 2014 to 2020, DPPE has supported 14 external research projects and 12 laboratories through grants. Applications for grants were reviewed by an independent scientific committee. DPPE has also directly created 7 projects and supported 3 laboratories through internal research. A patient registry and Services Attached to the Registry of Associated Patients (SARA) has also been set up to support clinical trials. The representatives from DPPE are passionate that collaborations should continue as they can be very beneficial.

DPPE has run workshops with hospitals, universities, industry (not pharma) and patient organisations. Examples of initiatives they have implemented are unifying protocols across different hospitals to prevent the deterioration of lung function in people with DMD, and allowing RNA-targeting nucleic acid drugs to reach their full potential and become a mainstream therapeutic option. The creation of a webinar series and newsletter in Spanish allowed DPPE to reach Spanish speakers across the globe, such as people in South America.

## Key takeaway

> DPPE aim to fill the gap between science and business, create their own projects and achieve a financial return.

# MULTI-STAKEHOLDER PERSPECTIVES ON DESIGNING A TRIAL TO FIT A PATIENT'S LIFESTYLE

**Alessandro Monterosso.** *CEO, PatchAi®*
**Joyce Moore.** *Director, Patient Recruitment Services, ICON plc*
**Graham Wylie.** *CEO, Medical Research Network (MRN)*
Moderator: **Greg Robertson.** *EU Patient Advocacy Lead, Spark Therapeutics*

Contract research organisations (CROs) and technology platforms are working with pharma to adapt clinical outcomes and real-world data and pave the road forward for designing patient-centric trials. The panel discussed the changes expected to take place in the coming years and the potential impact these changes will have.

It was repeatedly highlighted that patient engagement needs to take place as early as possible, not once the protocol is final or the study is already ongoing. Potential solutions to this are to educate protocol designers and to make patient engagement part of standard compulsory procedures from the early study planning stages and onwards. By making patient engagement a compulsory requirement, pharmaceutical companies would have no choice in order to remain compliant. Methods such as patient focus groups should be used early on to gain valuable insight. A team environment should also be created between patients, advocacy groups and clinical research organisations.

Future clinical trials should give patients the freedom to be seen where and when they want appointments to take place. For example, if aspects of a study can occur in the patient's home or in a nearby healthcare setting, rather than the study site, this should be made possible. If it is necessary for patients to travel, it is vital that individual patient needs are understood – reimbursement needs to be timely and seamless.

**Many solutions to designing trials to suit patients' lifestyles are already in existence:**

+ Digital therapeutics
+ Outcomes that are defined as valuable by patients
+ Technology to collect data at home – record symptoms and PROs
+ Point of care equipment that can be used at home
+ Remote, self-administered treatment
+ Adaptive study designs

With advancements in technology, there needs to be an overhaul of the traditional approach to preparing study protocols. In the future, protocols may not include visits but just the recording and collection of data through smartphones. It makes sense that patients shouldn't have to wait until visits to report symptoms, and with technology, real time assessments are possible. If a patient chooses, they may have very minimal interaction with healthcare professionals, and procedures such as imaging scans will take place in local healthcare settings rather than patients having to travel to the study site. Overall, the approach will see a clear switch from being visit-driven, to data-driven. Consequently, the patient experience will improve, trials will be cheaper, quicker and easier to run, and products will get to market faster.

## Key takeaways

> Although the future seems bright for improving the patient experience and more remote trials, patients do feel frustrated by these changes not happening more urgently. There is still an ongoing education process to ensure everyone in research recognises the importance of progress in patient engagement.

DEVELOPING A PATIENT-INCLUSIVE CULTURE

# HOW JANSSEN IS CREATING AND SCALING A PATIENT-CENTRIC CULTURE

**Daniel De Schryver.** *Patient Engagement & Advocacy Lead, Janssen*

In this session, Janssen provided learnings on how they specifically worked with patients themselves to establish a set of principles.

At Janssen, a global, multidisciplinary leadership team has been put in place to lead change and coordinate efforts in patient engagement. Janssen choose to not set up a patient engagement department because they decided that it was everybody's responsibility. The objectives of the initiative were to drive behaviour to incorporate direct patient voice early, and to understand barriers within disease area strategy, medicine development and clinical trial planning.

The systematic approach of the program means that patient insights are proactively incorporated early and throughout the product lifecycle. In addition, tools and processes incorporate patient perspectives throughout the development process. These include patient insights and an integrated patient journey template, target product profiles, customer value framework, and factoring in the patient voice in clinical trials. The adoption of all of these methods is accelerated by best practice sharing, and Janssen encourage all organisations to communicate about how they are effectively engaging with patients. An example of where Janssen used key patient insights is in the development of a new PRO measurement tool for people with psoriasis. A symptoms and signs diary was developed in collaboration with patients, clinicians and Janssen with input from regulators.

**Janssen have introduced a number of initiatives since embarking on their patient engagement mission, including:**

- A global patient advocacy council
- Plans to launch an online tool for all employees: the Patient Engagement Model tool. For each person's role and responsibilities, there will be a relevant action they can take to help contribute to patient engagement
- A patient advisory committee to give feedback on all functions at Janssen. Patients with any condition can take part (even if Janssen aren't researching it)
- Four animation series aiming to address the common issues that people with IBD face after diagnosis
- A 10,000 steps program, which aimed to support Hungarian men with prostate cancer increase their physical activity to improve their quality of life and chances of survival

## Key takeaway

> Introducing patient engagement throughout healthcare organisations help to inspire employees and create new initiatives.

UP-SKILLING COMMUNICATIONS IN PATIENT ENGAGEMENT

# THE PROGRESS OF GSK'S TRAINING PROGRAM ON BUILDING PATIENT ENGAGEMENT, COMMUNICATION SKILLS & COMPETENCIES

**Kay Warner.** *Patient Engagement Lead, GSK*

The level of communication skills needed to work with patients is not consistent across industry. In response, GSK ran a pilot program in January 2019 across Europe, training site staff on communication skills and facilitating group discussions. This session focused on the development of patient engagement skills within the industry and highlighted the progress of GSK's training program.

There is limited skills training in patient engagement for Pharma professionals and an unmet need to achieve common understanding of basic principles. With this in mind, the GSK programme was outlined as:

**Step 1: Face-to-face pilot training (Jan 2019)**

- Co-designed and delivered with external vendor
- Included 7 attendees from 5 countries – these were all from patient engagement or patient advocacy groups
- Received feedback from attendees to refine the programme for future training
- Able to practice skills in a safe environment
- The main aim of this was to: increase competence in patient engagement, increase capacity of patient engagement professionals, provide an inclusive and open forum

**Step 2: Complementary virtual platform delivering bite-size training on the process, skills and competencies (ongoing)**

- "Keep Growing Campus" is a platform that provides a one-stop shop for all employees wanting to engage patients
- Provides social interactivity and ability to contact experts in patient engagement
- Third-party training content from PFMD and EUPATI

The overall delegate feedback from the training was positive and there is a continued high engagement score for the virtual platform. New tools, templates and guidance are continuously being added to the platform and changes to the training are being made based on learnings and feedback.

## Key takeaway

> This session emphasised the importance of training staff in patient engagement, and that internal advocacy is important: employees should be encouraged and made aware when training is available.

# JNJ ON HEALTHCARING CONVERSATIONS FOR CLINICAL RESEARCH™: AN EVIDENCE-BASED APPROACH TO PATIENT-CENTERED CONVERSATIONS

**Snigdha Taduri.** *Program Manager, Clinical Insights and Experience, Janssen*

Enhancing the patient experience in clinical trials is nothing new. However, unless it's a scalable concept, it's not going to be effective. To combat communication challenges in clinical trials, Janssen developed a soft-skills training platform to help sites adequately communicate with study participants. This mini training is virtually based on three pillars around behavioural science, where communication style of site staff will help patients understand interest and motivation, foster a deeper connection and feel empowered.

Janssen explained how they established and deployed the framework. Firstly, they conducted research and spoke to patients and principal investigators to understand areas that could be improved in clinical trials. Janssen identified that there is a significant opportunity to connect patients with their sites and to empower them.

**Patients can be influenced by:**

- Practical issues e.g. travel
- Childcare
- Side effects
- Receiving the placebo and no treatment
- Complexity of trials
- Awareness of clinical trials (this is low to non-existent)

**Sites are influenced by:**

- Limited dedicated study staff
- Increased site burden with clinical trials
- Limited dedicated resources for eligible participant identification

Janssen believe that HealthCaring conversations can help, because it is a simple framework that provides a structured way for site staff to have consistent, focussed and personalised conversations with patients. It also encourages sites to have a people-focussed approach (instead of task-focussed), and lastly, it facilitates more effective conversations between site staff and patients.

The framework has been designed to seamlessly fit into how sites currently operate, so that it can be easily implemented. It helps sites have meaningful conversations, interpersonal relationships, and personalise the patient experience. Through research and developing the framework, Janssen identified that conversations that are too factual do not create a positive experience. The conversation can be turned around so the patient is at the heart (for example, by saying "let's start by discussing why you might be interested in participating") – giving the control back to the patient. **The initial impacts from using the framework are:**

- Increased patient satisfaction (with their care)
- HCPs reported increased job satisfaction (less stress and burnout)
- More pertinent information is shared
- Patients are more likely to follow HCP advice and recommendations.

## Key takeaway

> Initiatives such as HealthCaring Conversations for Clinical Research can lead to benefits for patients and healthcare professionals.

# NORGINE ON THE ROLE OF SOCIAL MEDIA AND THE RISE OF THE EXPERT PATIENT

**Liz Clark, MB BS, MSc, FFPM.** *VP of Medical Affairs, Norgine*

Social media has risen as a viable channel for patients in increasing awareness of rights and their collective voice throughout medicines development. From an industry vantage point, social media is a solid tool for 'listening' to the patient voice, providing insights into their experience and expectations. Nonetheless, industry is generally wary of navigating the compliance-driven atmosphere and stipulations regarding data security and privacy. This session highlighted the role of social media and the impact of the 'social age' on pharma industry.

Three main points were presented: forces of change, social influence and how pharma should react.

## Forces of change

The world is changing as we transform into the 'social age':

- Most people get their news from social media
- People will soon trust Amazon more than their bank
- Over 90% of buying decisions are influenced by social media

Experts in their own healthcare space are actively contributing and getting involved in discussions during 'Healthcare Tweet Chats' and even 'The BMJ Opinion' has a patients perspective slot. Ultimately, there's a huge amount of information available that can help with the industry's decision-making.

## Social influence

Previously, doctors were considered the only experts in medicine. But now, patients are experts too – they're experts on their own disease. Beyond this, patients are also 'influencers.' Social media is influencing the industry in a huge way – and it's not the technology, it's our behaviour within it. From an evolutionary perspective, trust has enabled us to survive in communities, and this is still relevant now. We trust the people that are close to us and it is in these "campfire" discussions on social media where people have the most useful exchange. At this point, it's an opportunity for pharma to gain insight from these conversations.

## How should pharma react

Social listening can be used to gain insights that inform action and gives opportunity to engage with patients. Norgine performed social listening in a study about liver health:

- They found that patients engaged primarily through Instagram
- The 'itch' was the most disruptive symptom
- From their insights, Norgine created a paper-based passport for patients to share their information easily

## Key takeaway

> There is an opportunity for pharma to benefit from the social age, but they need to remain GREAT: genuine, respectful and responsive, ethical, authentic and transparent.

# MULTI-STAKEHOLDER COLLABORATION TO ENHANCE PATIENT-FOCUSED EDUCATION, RESEARCH AND DRUG DEVELOPMENT

**Catherine Bouvier.** *CEO, NET Patient Foundation/President Elect, INCA*
**Lucie Keeber, PhD.** *Global Medical Affairs Director, Rare Diseases, Ipsen*

There are currently gaps in the perception of disease burden and quality of life between HCPs and patients. This session explored how these gaps can be bridged and how we can help patients and the public understand clinical research.

Neuroendocrine tumours present numerous complex clinical problems, but since their occurrence is relatively rare, research and patient care guidelines have been lacking since the 1990s. As a result, the European Neuroendocrine Tumor Society (ENETS) was founded in 2004. The main stakeholders involved in the ENETS conference are HCPs, alongside patient advocacy groups and patients themselves. By holding a symposium, the ENETS gained insights on patient reported challenges, the gap between patients and HCPs, the patient journey and how stakeholders can move forward together. The faculty comprised a nurse, physician, quality of life specialist and a patient. Four domains were considered potential perception gaps between patients and HCPs: diagnosis (length of time from first symptoms to official diagnosis), symptoms (burden), therapy (access to a wide range of NET-specific treatments) and their view of the future. Potential solutions to bridging the perception gap are putting the patient experience first, facilitating patient-doctor communication, sharing resources and positioning nurses.

**The key learnings from ENETS were:**

- Holistic management of patients is key to maximise quality of life
- HCP's perceptions of the impact on quality of life vs the patient perspective may differ
- Asking the patient about the impact of their condition and therapies on their quality of life is fundamental to holistic management
- Patient-centred care must focus on quality of life as well as quantity of life.

The speakers recognised that research in neuroendocrine tumours is extremely complex and can be difficult for patients to read and understand. **The proposed solutions to this were:**

- Continue to raise awareness about the importance of lay summaries
- Make information easily accessible
- Involve patient organisations
- Learn from patients during all stage of research.

## Key takeaway

> Communicating feedback from patients to HCPs is an important aspect of patient engagement, and further efforts are needed to bridge any gaps in perceptions.

PATIENT INVOLVEMENT IN REGULATORY DECISIONS AND VIRTUAL TRIALS

# HOW REGULATIONS ARE INCORPORATING THE PATIENT VOICE

**Domenico Merante, MD.** *Patient & Physician Advocate*
**Sol Yates.** *Associate Director Regulatory Affairs Development, Grünenthal*

International regulatory bodies like the FDA and EMA recognise the value of patient inclusion, and in recent years have adopted guidance for industry to involve patients meaningfully. This session discussed how regulations are incorporating the patient voice.

**Firstly, the importance of engaging patients in trial design was outlined:**

**Patients**

- Increased motivation to join clinical trials by better understanding of setup
- Opportunity to influence trial design
- Better understanding of study objectives
- Trial design can be tailored to patient needs

**Sponsors and regulators**

- Increased recruitment rate
- Better understanding of which potential endpoints are clinically meaningful
- Patient-reported outcomes and quality of life data
- Improved patient communication

Currently, there is no guidance specifically related to clinical trials, but patient engagement has established a larger presence within the EMA and FDA over recent decades. The industry is now increasingly encouraged to involve patients in identifying meaningful endpoints for clinical trials, and quality of life endpoints are being used more frequently. There is also greater emphasis on how to protect patients' privacy, and to address that there's more patients wanting to know more about the trials they participate in. This is particularly relevant as patients are becoming more powerful in their awareness of their rights and wanting to access and own their own data.

Grünenthal presented a case study on a fibromyalgia study. The pathophysiology of fibromyalgia is unknown and only small numbers of patients have been studied in Europe. It is a prime example of a condition where outcomes that are most important to patients may not traditionally correspond with researchers' study endpoints. Grünenthal presented a case study of when a briefing book for fibromyalgia was prepared and rationale presented to main regulatory agencies (including the EMA and FDA). The briefing book presented a case for including study endpoints that were of most value to patients. The FDA approved pain as a primary endpoint, providing that two, phase III clinical studies were successful. In this study, patients were placed into subgroups on the basis of pressure-pain thresholds and psychological factors. Grünenthal spoke to many patients and invited them in-house to raise understanding of their condition. Although this study produced negative results and there was evidence of a placebo effect, the patient engagement was considered successful.

**Lessons learned include:**

- Listen to patients, understand the relevance of their symptoms
- Apply learnings from patients in clinical study design and endpoints
- Build the reputation of clinical research and trust between patients and pharma
- Work closely with patient groups, regulators and physicians to produce one global, harmonised disease/patient focused guideline for the clinical development of new medicines
- Follow all necessary steps of clinical development, improving on patient engagement
- Choose appropriate size of studies, feasible procedures and frequency of study visits

## Key takeaway

> There has been progress in patient engagement among regulatory agencies, but there is still a gap to fill for a global regulatory guidance for the clinical development of new molecules aiming to treat conditions such as fibromyalgia.

# VIRTUAL TRIALS: PUTTING PATIENTS AT THE HEART OF CLINICAL RESEARCH

**Rosamund Round.** *VP, Patient Innovation Center, Parexel International*

This session explored the benefits of virtual trials based on Parexel's insights.

Virtual trials are designed to make it as easy as possible for patients to participate in clinical research by taking all or part of the study to them in their homes. Technology and logistical support can all help make virtual trials successful, including: patient apps, transportation support (not necessarily financial but also practical), home healthcare, sensors (e.g. wearable devices to measure outcomes such as heart rate), direct-to-patient medication or laboratory shipments.

**Virtual trials can drastically reduce patient (and site) burden, by addressing the practical, financial and geographical barriers to participation:**

- Travel
- Time
- Care responsibilities (such as childcare)
- Work commitments
- Cost – petrol, public transport, parking. Even if costs are reimbursed, people are out of pocket.

Virtual trials are viewed as patient centric because the strategies are created based on patient feedback. They consider all the practical difficulties patients and caregivers may face if a patient chooses to take part in a clinical trial and are designed to maximise convenience and consider their current lifestyle and commitments. By considering the patient perspective, virtual trials will likely have fewer dropouts, thus increasing the likelihood of clinical trial success.

Moving forward, Parexel intend to meet with regulatory bodies and present examples of the positive impact that virtual trials have had on the patient experience.

## Key takeaway

> Virtual trials can bring huge benefits to both patients and the industry. Simple changes and considerations can make taking part in clinical trials much more convenient for patients (and caregivers).

# A GLOBAL AND LOCAL PERSPECTIVE ON THE IMPACT OF PATIENT INPUT ON SERVIER'S ICFS

**Marta Garcia, PharmD, MBA, MPH.** *Director of Patient Services, R&D Clinical Development, Servier*

Patient information sheets (PISs) and informed consent forms (ICFs) are a vital aspect of clinical trials. This session explored why patients should be involved in their development and presented a case study.

**When patients are involved in the development of PISs and ICFs, these factors must be considered:**

- Regulatory guidance
- Cultural differences
- Health literacy
- Timing
- Budget
- Mindset of companies involved
- Experience

The case study presented by Servier surrounded an oncology study whereby patients were involved in developing the PIS and ICF. Initially, three core countries were selected based on their interest in the study, their understanding of the study, their co-creation mindset and logistics. The patients who took part had a range of experience in clinical trials, knowledge about their condition, and health literacy. National and local patient associations and patient advocacy groups were also part of this collaboration.

To obtain patient feedback, all patients received the same information (but local languages were used) and the documents were reviewed. A focus group comprising two patients provided additional insight. Overall, most patients felt the length and clarity of documents was important. They wanted to receive general safety information and understand why they should be motivated to take part, the impact it would have on their family lives and other options. The insights were used to develop the PIS and ICF in the core countries before the global versions were prepared.

**Benefits of the entire process were:**

- Strong patient involvement
- Documents were more representative of patient needs
- Servier were able to anticipate recruitment difficulties
- Reduced amendments
- Local involvement

Challenges included the time and budget required, the role of CROs and training needs.

## Key takeaway

> Patients should be involved in developing recruitment materials for clinical trials, but there are some challenges to overcome to streamline the process of doing so.

PPI AND THE IMPACT ON HTA

# WHAT TO KNOW ABOUT GOOD PATIENT PUBLIC INVOLVEMENT (PPI) AND THE IMPACT ON HEALTH TECHNOLOGY ASSESSMENT (HTA) DECISIONS

**Jennifer Dickson.** *Public Involvement Coordinator, Scottish Medicines Consortium (SMC)*
**Šarūnas Narbutas.** *President, Lithuanian Cancer Patients' Coalition (POLA)*
**Donna Walsh.** *Executive Director, European Federation of Neurological Associations (EFNA)*

Across the board, medical organisations are recognising that the patient voice needs to be heard and implemented. However, there are still a number of challenges faced.

**The SMC have been given government funding to revise and strengthen their patient engagement processes, and have used this to set up an improvement program, and so far:**

+ An advisory group has been established with public partners, patient engagement representatives and clinical experts
  – The advisory group were instrumental in creating a new framework for evaluating medicines to treat rare disease
+ Involvement of patient groups has been encouraged
+ The impact of new medicines on quality of life has been discussed
+ Patient carer representatives attend committee decision-making meetings, which are held in public
+ There has been an increased focus on transparency.

Best practices such as these above should be able to be adapted for use in other countries. In some European countries, discussions between patient associations and the HTA are happening too late in the process, so this needs to change. It was also highlighted that the involvement of patient engagement organisations needs to be strategic, with a focus on groups talking about what they know best.

## Key takeaway

> Patient engagement organisations currently face a number of challenges when trying to increase their involvement with the HTA.

DIVERSITY IN CLINICAL TRIALS

# HOW BIOGEN IS ENHANCING DIVERSITY AND REPRESENTATION AND THE IMPACT ON CLINICAL RESEARCH

**Narinder Chopra.** *Director of Patient Feasibility and Enrollment, Biogen.*
**Harry Yeates.** *Strategy Director, Langland.*

Diversity and representation are essential metrics to have in clinical trials, yet there's a lot more that needs to be done to improve this. In this session, Biogen and Langland explained how they're engaging underserved populations in clinical trial awareness and participation. **In 2019, they began to improve the diversity and representation issue through:**

- Community engagement and awareness
- Site and patient insights
- Regulatory/industry working groups
- Internal awareness and education.

## Learnings from community outreach

Biogen found that establishing trust is central to engaging underserved populations in clinical trials through consistent, meaningful community engagement. **This can be achieved through:**

- **Simple, clear language** – using down-to-earth messaging and simple language, tailoring the conversations with supporting materials
- **Consistent brand and messaging** – reliable and clear messaging around the events, with a clear focus on the disparities in clinical trials and how to improve it
- **General health education and disparity** – addressing specific topics of concern with the target audience and bringing together other healthcare individuals who are passionate about health equality
- **Clinical trial education** – sharing key clinical trial information to support discussions and giving them a clear call to action, such as websites where they can find available clinical trials.

## Looking further into social listening

Langland described the social listening approach that was conducted by a third-party specialist social listening agency over a 12-month period, to explore the rates of engagement online around clinical trials in underserved populations, specifically African Americans and Hispanics.

Overall, the results showed that only 0.001% of all the conversations from patients/caregivers around clinical trials were from African American and Hispanics, therefore, a lack of representation. After delving deeper into the conversations, they found a disparity between the level of interest in the pre-trial period than actually being involved in the clinical trial. And by looking into a framework that has previously been used, they realised that while 'low awareness' is an issue across the population, for minority it groups it could be more than that. For example, potential barriers to patient acceptance such as: medical mistrust, time constraints, financial constraints, or competing responsibilities such as work or childcare. To assess this further, Langland analysed the ratio between the offer and acceptance for underserved populations with multiple sclerosis (MS). They found that 67% of African American children grow up in single parent households. Langland began to understand that the availability of childcare is likely to have an influence

on the acceptance to take part in MS clinical trials, and therefore should be considered at study initiation. This shows there are more barriers to clinical trial participation than 'low awareness', and social listening can begin to uncover more answers.

## Key takeaways

> Diversity and representation in clinical trials are low, and a combination of community engagement, social listening and internal awareness are all key steps towards improving it.

# ACCESS TO TREATMENT AND MEDICATION IN EASTERN EUROPEAN EU MEMBER STATES

**Viorica Cursaru.** *Founder and President, Myeloma Euronet Romania*
**Theodora Weisz.** *Patient Advocacy Director Europe, Akcea Therapeutics*

This session highlighted the healthcare needs for Central and Eastern European countries. It was emphasised there are barriers to access to treatment and medicine in Romania, and this issue lies within subsidiarity. So far, EU Policy makers have declined to take effective action towards harmonisation of the health system within EU Member States. However, Myeloma Euronet Romania concluded with solutions that could help with this issue on both a national and European level.

**On a national level:**

- Allocation of higher GDPs to the Health System
- Improvement of infrastructure, particularly in remote areas
- Better offers for medical corporation

**On a European level:**

- Harmonisation of the national health services in the EU
- Establishments of benchmarks for Mandatory Standards of Health
- Mandatory benchmark for GDPs
- More resolutions and more directives in the field of Health System.

The session continued with Akcea Therapeutics, who highlighted the challenges and benefits for healthcare access in the Central and Eastern Europe (CEE) region, spanning economic, commercial, legal, logistical and political factors. Before entering these countries, there are a number of things to consider such as the population, healthcare spends, general market structure, and the market landscape.

### Patient engagement recommendations

Akcea Therapeutics then highlighted examples of what patient groups can do for treatment access. These include reaching out to local health authorities, private sick funds and payors to explain the population. Further to advocating for access, organisations should advocate for general improvement of patients' quality of life and leverage media to raise public awareness. In addition, support groups should be offered as integral components of treatment plans for patients with genetic disorders and their families.

## Key takeaways

There are two important things to be verified before entering the CEE countries.

1. Analyse the situation based on criteria, such as prioritisation of health budget, number of potential patients, and patient organisations.
2. Leverage the opportunity: explore individual funding requests per country, address unmet medical needs, gain support of local KOLs, payors and patient groups, ensure initiatives for accurate and early diagnosis, increase patient finding initiatives.

# CAREGIVER AND PATIENT EXPERIENCE IN A CLINICAL TRIAL

**Alfred Samuels.** *Prostate Cancer Patient Advocate*
**Grace Samuels.** *Caregiver and Patient Advocate*
Moderator: **Rosamund Round.** *VP, Patient Innovation Center, Parexel International*

When a patient is participating in a clinical trial, the caregiver is often not considered. In this session, Alfred Samuels and his wife Grace shared their perspectives around the clinical trial experience when Alfred was diagnosed with prostate cancer.

## Grace's perspective on the clinical trial

Grace emphasised that it was only after the initial shock that they began to think more clearly about their options, and the STAMPEDE clinical trial was one of them. Grace's initial concerns were that the trial was "hit and miss" depending on where they lived, and the concerns of being a "guinea pig" were discussed. But after a lot of reading, Grace realised that they didn't have anything to lose. It's important to note here that Grace was the person that was researching into the clinical trial as she wanted to know as much information as possible, whereas Alfred was adamant that he didn't want to know.

When the trial began, Grace described it as "up and down", and initially, Alfred's symptoms worsened. Although it was Alfred taking part in this clinical trial, Grace was experiencing it too. As Grace was still working in a senior position within the NHS, she emphasised that it was difficult to balance between work and Alfred's situation. While this didn't happen often, Grace had to leave in-depth meetings to help Alfred. Eventually, she went to the doctor and was signed off work for 2 weeks; both mentally and physically, she couldn't balance work and Alfred's situation.

## How did the healthcare team consider Grace during Alfred's treatment?

Grace explained that they didn't consider her much throughout the trial, because Alfred was the patient. However, she believed it was extremely important for her to attend the appointments to explain different aspects that Alfred didn't voice to the doctor. Further to this, Grace felt that nobody seemed to be mindful of how she was feeling, and all surveys throughout the trial were only aimed at Alfred.

## Alfred's perspective of the Grace's role during the treatment

In an emotional speech, Alfred explained the role that Grace played throughout the clinical trial journey. He said that as a caregiver, Grace was responsible for everything and took over when things got difficult. Grace highlighted that Alfred was often embarrassed of his symptoms – he wouldn't want to do anything. But with Grace being there, she offered the support and empathy he required. Alfred strongly emphasised that the caregiver's role needs to be looked at much more closely.

> *"She's my rock. There are no ifs or buts... she's my solid rock. Without her, I wouldn't be here today. My partner took over and was in charge of everything."*

### The impact of the practicality and rigidity of the clinical trial for the Grace and Alfred.....

Grace explained that every week they had to go to the hospital for regular check-ups and to receive treatment. However, the hospital was 1 ½ drive away and the protocol was inflexible. Due to this, Alfred moved from the trial and moved to what they called an "NHS option". This offered increased flexibility, and Alfred could eventually get back to work and have a better quality of life.

## Key takeaways

> This session clearly highlighted the importance of the caregiver's role in clinical trials, from the initial diagnosis and throughout. And as the caregiver is primarily the person who reads all clinical trial information and provides constant support to their patient, it is important that all materials (including surveys) factor in the caregiver.

# DEMONSTRATING PATIENT VALUE IN WORKING WITH INDUSTRY: NOTHING ABOUT YOU, WITHOUT YOU

**Christine Janus.** *CEO, International Alliance of Dermatology Patient Organizations (IADPO)*
**Camilla Krogh Lauritzen, MSc, MCC, MMBA.** *Chief Patient Officer, LEO Pharma*

When measuring patient engagement value, key performance indicators tend to aim towards the organisation, not the patients themselves. This session gave insight into how the industry are ensuring disease experience experts are an integral part of all key decision-making in R&D efforts, what's in it for the patient community to engage with pharma, and how the industry can continue to relay the patient value in the work that we do.

GlobalSkin – an alliance of patient organisations that work within dermatology – and LEO Pharma, their industry partner who also has a focus in dermatology, work collaboratively. LEO Pharma highlighted that their role is to ensure that GlobalSkin can influence them and are a key part of the decision-making process. GlobalSkin and LEO Pharma shared the same goal of changing the way the world sees dermatology.

## Why partner with patient organisations?

GlobalSkin highlighted some important statistics regarding clinical trials, which lead to longer timelines, greater resource usage, and an increased cost. Ultimately, new medications are significantly delayed to the patients who need the most. Therefore, engagement with disease experience experts needs to start before clinical trial development. By partnering with patient engagement organisations at the right time, it's more likely that recruitment targets will be met and significant delays to treatment access can be avoided.

## Nothing About You, Without You in action

This session ended with why and how patient organisations make a difference, what GlobalSkin are doing, and next steps for the industry. GlobalSkin work to change societal norms, governmental policies and entire systems. So, they have an ambition, what if:

+ Quality of life could be measured using a tool that's developed by patients
+ A credible patient voice was built upon by verifiable data
+ Policymakers never made a decision without including a patient-led measure.

Currently, quality of life and burden of disease measurements are designed by doctors. To change this, GlobalSkin created the first global patient-initiated and patient-led impact research study in dermatology, measuring the true impact of skin diseases from the patient perspective: GRIDD. GRIDD aims to measure global disease burden across a wide spectrum of dermatological diseases using an innovative measurement instrument, derived through a novel methodology called Global Research of Impact on Patients (GRIP). The questions are co-created, by gathering patients' views on the impact of their disease both on themselves and their family members.

## Key takeaway

> By patient organisations and the pharmaceutical industry working collaboratively from the beginning, this brings benefits from both perspectives: outcome measures can be relevant to the patient, and the industry can include the patients' and caregivers' voice into key decisions by health authorities, backed with valid information and data.

# ROCHE ON MERGING ENGAGEMENT & ADVOCACY FOR AN END-TO-END PATIENT PARTNERSHIP FUNCTION

**Sanja Njegic.** *Global Head of Patient Partnership, Roche*

This session explored Roche's strategy for merging engagement and advocacy, to create an end-to-end partnership function. It was explained that to work on their new function, three steps needed to be done: to listen, identify shared interest, and co-create.

By first listening to patients, Roche found that patients wanted one main point of contact when it comes to working with the industry. Therefore, Roche decided that the team needed to be adjusted and agreed on two main teams for patient engagement:

+ **Global product development medical affairs** – delivers value by integrating external clinical and patient perspectives into Medical Strategy
+ **Global partnership team** – ensures the integration of the patient perspectives across the lifecycle.

For their Global Patient Partnership strategy, Roche wanted to create a mutual value as a trusted partner for global patient communities by integrating the patient perspective across the value chain. They broke their strategy down into four strategic pillars, created with different functions inside the company and patient communities:

1. Early and systematic engagement when it comes to research and development
2. Access to data – to collaborate and work together to build data that really matters
3. Personalised healthcare/precision medicine
4. Being viewed as a patient-centric company – KPIs, reputation, speed of the co-creation and transformation.

### How are they bringing patient perspective and engaging with patient communities early and systematically across the life cycle?

Roche designed a new operating model, the standing Patient Councils, otherwise known as standing advisory boards. These councils provide a platform for systematic engagement with patient community, understanding of the patient needs, co-creation and partnership in common areas of interest across the whole life cycle.

## Key takeaway

> By successfully bringing engagement and advocacy functions under one roof, Roche changed their internal infrastructure to set a strategic framework for patient partnerships – showing that adjustments in internal organisations can create trusted partnerships.

# HOW NOVO NORDISK IS CAPTURING THE PATIENT VOICE WITH ADVISORY BOARDS TO BUILD A SUSTAINABLE PATIENT ENGAGEMENT MODEL

**Charline Coquerel Couniot.** *Head of Global Patient Engagement and Advocacy, Novo Nordisk*

To improve their patient engagement, Novo Nordisk focused on key areas to change their internal functions and build an improved 2020 R&D strategy. From a value chain perspective, they set up an R&D patient engagement task force, leveraged commercial colleagues within their team, and leveraged FDA/EMA guidance to create more in-depth discussions and ensure shared value. From a therapeutic area and project perspective, they conducted workshops with project leads to identify priorities and opportunities. Lastly, from a patient perspective, they gained insights through DEEP summits and advisory boards, to understand how they want to work with disease experts.

## Developing a compelling metrics framework

Novo Nordisk worked with PARADIGM to develop The Global Patient Relations Metrics Framework, creating metrics and measurement tools to ensure the impact during their patient engagement can be measured. The three main stages where they engage with patients are:

1. **Preparation**
   - **a.** Define objective, scope and agenda
   - **b.** Approach, select and engage participants, both internal and external
   - **c.** Ensure compliance and plan logistics
2. **Execution**
   - **a.** Lead and moderate the event
   - **b.** Secure that objectives are met
   - **c.** Ensure findings collection and agreement over next steps
3. **Outcome**
   - **a.** Interpret results and translate the findings into action
   - **b.** Follow up with participants for next steps
   - **c.** Implement actions according to the objectives
   - **d.** Measure the impact.

Some of the tools and methodologies that Novo Nordisk have implemented in this three step strategy include: pre-activity surveys to ask participants about their expectations, where they believe the activity will have an impact, and where and when they plan to implement it further down the line. Novo Nordisk also created a clear template for their outcome reporting, to ensure their insights are implemented into actions and recommendations.

## Key takeaway

> This sessions highlights the importance of having shared values both internally and externally, and how creating a compelling framework alongside clear templates can really improve patient engagement. It ensures that impact can truly be measured, helping to identify that objectives are being met, the insights and results are transparent and presented clearly, and actions can be implemented.

# DIGITAL HEALTH MANAGEMENT: EXPLORING THE BENEFITS OF VIRTUAL PATIENT ADVISORY BOARDS

**Tom Pulles, MD.** *VP, Medical Affairs and Patient Advocacy, Ultragenyx*

In this session, Ultragenyx presented a case study that highlights one of the initiatives they have in place to engage with patients, patient advocates and caregivers in the rare disease industry.

## Case study: virtual advisory board

The participants for this online session were global Mucopolysaccharidosis VII (MPS VII) patient advocates from three different continents, with Ultragenyx Medical Affairs acting as the internal moderator. The session began with a 2-hour webcast with simultaneous translations from Spanish to English. Following this, the two-week online session was launched directly following the webcast for advisors to answer seven predefined questions. They had the opportunity to see each other's answers and post comments or additional questions. Due to this being an online session, they were able to enter the session at their own convenience and time zone.

**The objectives of this virtual advisory board were to:**

- Better understand the experiences, priorities and unmet needs of MPS Patient Advocacy Groups from different countries and global regions
- Determine the opportunities to increase engagement with the MPS VII community
- Identify appropriate avenues to collaborate and ways for Ultragenyx to improve communications, interactions and engagements with the MPS VII community at regional and global levels.

The outcome from this online advisory board was successful and a report was developed from the total of 81 participant responses.

## Ongoing research from Ultragenyx: The MPS VII Disease Monitoring Programme (DMP)

One of the main of discussion points during the virtual advisory board was the DMP. This is an ongoing global, prospective, multi-centre, longitudinal programme that began in early 2018 and is expected to last 10 years. The programme will include patients diagnosed with MPS VII and it's expected that 35 paediatric and adult patients will enrol globally.

The programme is designed to understand how MPS VII presents and how people may experience MPS VII differently. It will study changes using tests, assessments and patient/caregiver reported outcomes to better understand quality of life and disease progression and mortality. During the programme, patients will also receive regular, personalised reports, including test results can that be shared with doctors and specialists seen outside of the programme.

## Key takeaway

> With certain factors such as travel restrictions, caregiver responsibilities, financial restrictions, time restrictions and more, it can be challenging to gain insights from rare disease communities. Ultragenyx have demonstrated the benefits that a virtual advisory board can provide and how the learnings can be taken further, such as into future programmes like the MPS VII DMP.

# LEARN HOW PHARMA AND ADVOCACY GROUPS ADDRESSED THE NEEDS OF PATIENTS IN CONNECTING THEM TO CLINICAL STUDIES IN A NEW AND INNOVATIVE APPROACH

**Lisa La Luna.** *SVP Patient Advocacy, SME & Senior Advisor, WCG*

With patient recruitment still being a predominant barrier to patient participation, WCG created an innovative approach to connecting patients to clinical studies. WCG believed that when patients have taken the time to go through pre-screening, they should easily be able to find another one that's relevant for them – so they developed CenterWatch iConnect.

WCG acquired CenterWatch, the leading consumer site for clinical trial listings. Following this, they upgraded the technology in order to track patient recruitment tactics and performance, the referrals and ROI. They also included the ability for pharmaceutical companies to self-configure, meaning companies can upload their study websites or pre-screeners. Lastly, they needed the technology to be able to match those patients who didn't qualify for their first study, to give them other options that they could potentially screen for.

**In summary, iConnect offers:**

- Trial listings
- Patient pre-screeners
- Digital campaign development and tracking
- Patient follow up status
- Site feasibility surveys
- Study website templates
- Integrated reports on ROI and performance
- Largest syndication network in the industry of patients seeking clinical trials.

**Value for advocacy groups and institutions**

- Comprehensive tracking and reporting, data and analytics to manage programs, upper management and potential clients
- Study website templates and pre-screeners – maximises spend on patient outreach
- Simplifying the connectivity process of patients to sites – lessens the burden/overhead for organisation
- Streamlining the recruitment efforts across organisation – eliminates the silos that result in missed opportunities
- Exposure to patients of the largest clinical trial database – increases patients' probability of qualifying for a study

**Value for sponsors**

- Centralised listing service and tracking platform for performance and ROI
- Eliminates recruitment tactics that result in duplicative spend
- Provides performance predictability for recruitment tactics
- Provides visibility in tracking site level performance
- Provides standard process to match a patient if they don't qualify the first time
- Maximises budget

## Key takeaways

Transformation of the patient recruitment connectivity process will require:

1. A holistic approach that builds awareness, informs and offers patients one direct touchpoint with options to find the appropriate clinical trial for their needs
2. A comprehensive platform to track effectiveness and ROI, across all stakeholders and vendors.

PILOTS, PROGRESS & TOOLS

# A MULTI-STAKEHOLDER TAKE ON DEFINING A SUSTAINABLE FRAMEWORK FOR PATIENT ENGAGEMENT

**Zsófia Bakonyi.** *Senior Manager Partnerships, European Federation of Pharmaceutical Industries and Associations (EFPIA)*
**Mathieu Boudes.** *Public/Private Partnership Coordinator, European Patients Forum (EPF)*
**Derick Mitchell, PhD.** *Chief Executive Officer, Irish Platform for Patients' Organisations, Science and Industry (IPPOSI)*
Moderator: **Berkeley Phillips. FFPM, MRCP, MA.** *Medical Director, Pfizer*

In this panel discussion, EPF, EFPIA and IPPOSI shared their insights on how to define the criteria for systematic, meaningful, ethical, and sustainable patient engagement. EUPATI.

## EPF

EPF explained how patient groups will soon become business partners in generating the data we need, which is one of the key assets that will help to move the value chain. Action is needed to prepare patients for this, and it is essential for all of the different stakeholders involved to drive change. Regarding sustainability, most previously looked to the regulators. However, the regulators say they drive patient engagement through their own practice and through example. EPF highlighted that the FDA are catching up; guidance says that the FDA will help to capture and accept data that comes from different patient groups. Furthermore, data must establish why we engage with patients and the impact of it, which will eventually help us to build a framework for sustainable patient engagement.

## EFPIA

For sustainability, EFPIA agrees that everybody should be responsible, and everybody has a role to play. It's multi-stakeholder collaboration that is going to drive the change to patient engagement and make it sustainable. EFPIA has recognised the performance of patient engagement and made it a priority for all colleagues to define patient engagement, KPIs and objectives and build them into their work plans.

## IPPOSI

IPPOSI agrees that for a sustainable framework, data must be the focus. Despite frameworks and guidelines being updated regularly, patient organisations and communities they represent need to have the capacity to contribute to data and sustained at a national perspective. Patient data can drive innovation, and the patient role will fundamentally change as part of that, eventually leading to sustainability.

## Key takeaway

> According to EPF, EFPIA and IPPOSI, the real keys to sustainability of patient engagement is patient data and shared responsibility.

# TRANSCELERATE PATIENT EXPERIENCE TOOLS: A PRACTICAL GUIDE FOR PATIENT INPUT

**Sherina Kuruvilla.** *Associate Director Global Patient Relations, Novo Nordisk.*

In this session, Novo Nordisk shared their innovative tools that help to implement a more sustainable way of gaining patient input: the TransCelerate Patient Experience Initiative and toolkits.

There are two main components: the patient protocol engagement toolkit (P-PET) and the study participant feedback questionnaire (SPFQ).

## P-PET

This is used during the study design process to encourage co-creation and discussion with patients. It enables input from the patients to help improve the design and conduct of the study. The toolkit includes resources such as:

- **User guide** – all the factors to consider when starting protocol design
- **Resource guide** – a question bank and visual aids that help to structure patient conversations
- **Templates** – email templates, action outcome reports to collect outputs from discussions with patients.

## SPFQ

Once the trial is designed, the SPFQ can be used during protocol execution to gather patient feedback during the clinical study at three separate points throughout the study: start, during and end. The feedback can then be used to identify the burden or impact and take actions to improve the current experience or make future studies less burdensome to patients. The SPFQ includes resources such as:

- **Socialisation deck** - used to help build a value proposition to sell to the leadership team
- **Implementation guide** - consider when implement the questionnaire, such as legal and compliance factors
- **Questionnaire templates** – the same questions can be used across different protocols, or can be adjusted to be tailored to the patients

While the assets from these two components can be used separately, maximum value is achieved when both are implemented.

## Key takeaway

> Having toolkits and resources for different stages of the clinical study process can provide structure during patient engagement and a more sustainable way of gaining patient input.

# "NOTHING ABOUT US WITHOUT US" – PATIENT-DIRECTED ENGAGEMENT AT MERCK KGAA

**Trishna Bharadia.** *Health and Patient Engagement Advocate & Consultant*

To live up to their ambition of being the most "patient-directed" healthcare organisation, Merck KGaA partnered with patient advocate Trishna Bharadia to co-design all aspects of The Merck Patient Group Forum. In this session, Trishna gave her perspective on the overall experience.

## What did both Trishna and Merck KGaA have to consider?

There were four key aspects that needed to be considered throughout to ensure that patient engagement was:

+ **Relevant:** The engagement needed to be relevant and ensure that it was going to add value to the event. Engagement needed to be delivered at the appropriate time of the project lifecycle. In this case, right at the beginning of the project lifecycle and right through to development
+ **Appropriate:** Merck ensured all the tasks were suitable for Trishna's skills, expertise and capacities. Meetings were held close to home and scheduled at appropriate times to minimise the impact that the participation may have. Trishna also emphasised that the one single thing for effective collaboration with patients is to never assume, and just ask people simply what will make it easy and appropriate for them to collaborate
+ **Fair:** In terms of compensation, patients are providing a service so this should also be fair
+ **Transparent:** It was important that the aims and objectives were aligned at the very beginning, including the expectations, scope of work, and projected timelines. Relevant standard operating procedures were also explained when necessary. Any potential challenges that Trishna needed to be aware of were also explained – this is important especially if it is the first time co-designing an event. And lastly, they needed to stay compliant throughout the process, so boundaries were put in place.

## What was Trishna involved in?

Trishna was involved in all aspects of the process, from the beginning to the end of the meeting. This included the:

+ **Concept** – the development of the concept into a tangible process, with insights from previous experiences and expertise
+ **Development** – the design, type of language, and the format
+ **Promotion** – such as the invitations; who to invite and how they should be invited
+ **Facilitation** - Co-hosted and facilitated the event

## What were the outcomes?

Overall, the event was successful with positive feedback. In summary, it was relevant and effective and both Merck and Trishna were able to take learnings from the event. From Merck's perspective, they had top-buy in from management as they liked how the event executed. From Trishna's perspective, the event was truly patient-focused and enjoyable.

## Key takeaways:

> Co-designing with patients leads to better outcomes for both the industry and the patients, as it is a collaborative, two-way process.

# A Q&A SPOTLIGHT ON ABPI'S RECENT PROGRESS IN WORKING WITH PATIENTS IN A MEANINGFUL WAY

**Claire Nolan. Patient Involvement Facilitator, Charities Research Involvement Group**
**Sheuli Porkess, MA. Executive Director of Research, Medical and Innovation, ABPI**

**This spotlight discussion focused on the way that ABPI are working to progress with patient engagement.** It began with a general conception that is often heard: "The UK is still a difficult environment to do patient involvement because of ABPI regulations." In response to this, ABPI clearly emphasised that there is nothing in the ABPI code that says people can't work with patients. There's a number of steps that the industry has been working on to guide companies through this issue. For example, helping to understand:

+ What exactly patient engagement is
+ Why we need to do patient engagement
+ How we do implement patient engagement

In order to challenge the misconception that patient engagement is not allowed, ABPI realised that the code needs to be "de-mystified." In 2019, ABPI published the sourcebook – a document that collates aspects of the ABPI code and provides helpful guidance for companies working with patients. And while the content of this sourcebook is beneficial, it also demonstrates that ABPI are supporting patient engagement.

Secondly, ABPI are also working with research charities to gain a better understanding of the barriers, how they can effectively work together and how ABPI can support the charities.

Finally, ABPI often involve patients in multi-stakeholder events. This helped to ground conversations to the fundamentals of patient health outcomes.

## Key takeaway

> The ABPI code doesn't stop patient involvement. Through multi-stakeholder events, working with patient charities, and understanding the practical assets that need to be considered from patient insights, they are working to promote patient engagement. The ABPI are also adjusting their internal policies and processes to discover whether they're fit for purpose, and challenging companies to do this too.

# PFIZER AND NIHR SHARE DATA FROM PILOT STUDY ON A NEW NETWORK APPROACH TO CONNECTING PATIENTS WITH PHARMA TO CONTRIBUTE AT THE PROTOCOL DESIGN STAGE

**Sophie Evett. Feasibility Lead, Study Optimization, Global Product Development, Pfizer**
**Gareth Powell. Patient Engagement in Clinical Development Service Delivery Lead, NIHR Clinical Research Network, National Institute for Health Research (NIHR)**

In May 2018, the NIHR initiated a patient centricity project, which aimed to develop a range of patient involvement services to the Life Sciences Industry.

To date, there have been five patient engagement activities performed for Pfizer studies, facilitated by the NIHR, as part of the pilot study. Two examples include:

## 1. The Atopic Dermatitis comparator study

Following a face-to-face meeting at the Children's Hospital with the young person's advisor group, the clinician gained some insights and beneficial feedback:

+ The frequency of visits was acceptable
+ More explanation was needed in the informed consent document around side effects and why the number of blood tests is required
+ The cartoon pictures in the informed consent document were seen as patronising, and photos showing the study procedures such as blood-taking were preferred.

As a result, the feedback was used to develop the informed consent form with regards to explanations around side effects and the number of blood draws required, in pictorial representations selected for under 18's.

## 2. Two ulcerative colitis studies

A face-to-face meeting was held at the NIHR offices with five patients with ulcerative colitis and an online meeting was set up to ensure Pfizer colleagues could dial-in to the meeting. The outcomes from this meeting were:

+ A greater insight into what it is like to live with a condition such as ulcerative colitis
+ The need for flexibility in the hospital visits
+ Realisation that three colonoscopies was too much to ask for patients, who typically already have one each year as standard of care
+ A varied preference on the route of administration.

Changes were consequently made from this feedback: Pfizer worked with sites to learn more about the potential of greater flexibility around the visit times, and rather than three colonoscopies required, the protocol was changed to two of these being a sigmoidoscopy, which is a less invasive procedure.

## The patient engagement service

The service itself is an enhanced cost-recovery extension of the NIHR Study Support Service, open to the life sciences industry. Depending on the company's requirement, NIHR will seek patient groups suitable to support the request and formalised with

a letter of agreement between all parties. Patient expense, travel, subsistence and venue hire will be arranged to support the requirement. NIHR will also act as a liaison during the even and finally provide an invoice to the company on completion of the event.

## Key takeaways

> It is important to look inwards as well as outwards, because internal buy-in and collaboration is required to enable meaningful patient involvement. Clear objective and realistic timelines are also important for any patient engagement projects.

# Conclusions and future recommendations

The speakers at the event all showed a true passion for patient engagement and a large number of successful patient engagement initiatives were presented. The foundations for effective patient engagement are now in place and it is important that action is now continuously taken to drive patient engagement across the board. Those experienced in patient engagement must continue sharing their knowledge, insights and expertise and inspire others to engage with patients. Without collaborations between patients, regulatory bodies, patient groups, pharmaceutical companies and more, there will be no progress. Everyone involved needs to recognise their shared goals and work together to ensure changes are made.

**A number of key themes appeared throughout the conference, which need to be addressed in the future:**

+ Ensuring patient engagement happens as early as possible
+ Engaging with paediatric patients
+ Capturing qualitative data
+ Diversity
+ The entire patient journey – don't stop engaging after people stop taking part in the study
+ The role of caregivers
+ Funding for patient organisations.

In summary, developments have certainly been made in patient engagement and this is demonstrated by previous and ongoing initiatives. However, there is still a huge amount of work to be done in this area, and collaborations will be vital moving forwards.

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