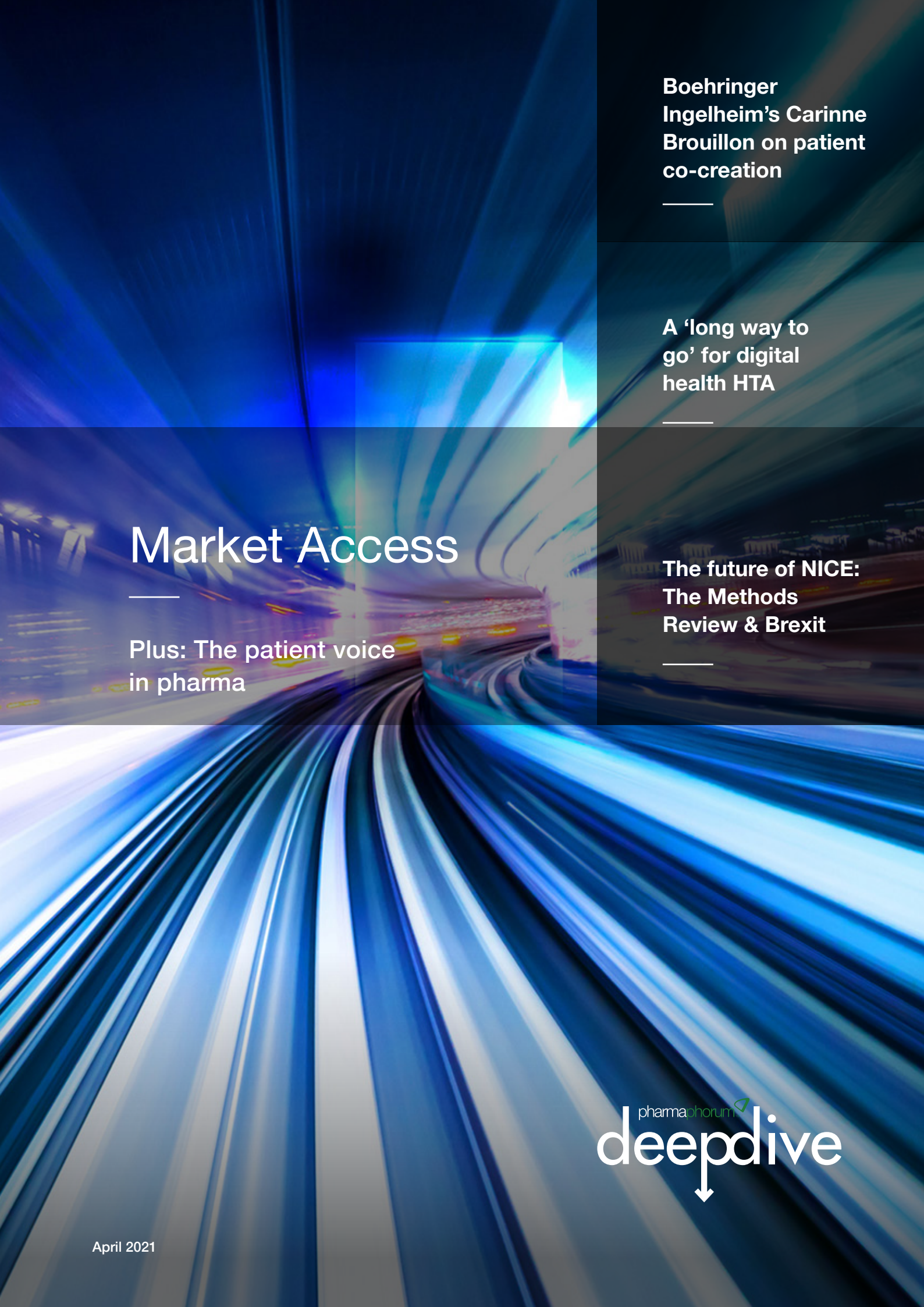

Market Access

Plus: The patient voice in pharma

Boehringer Ingelheim's Carinne Brouillon on patient co-creation

A 'long way to go' for digital health HTA

The future of NICE: The Methods Review & Brexit

pharmaphorum deepdive

April 2021

Deep Dive:
Market Access 2021

Pharma market access has been a key focus for pharmaphorum ever since the company was founded 12 years ago, but it's fair to say there has never been a more interesting time for the area than now.

Not only has COVID forced companies, governments and healthcare systems to work towards approving drugs and vaccines in record times, the sector is also facing an influx of digital therapeutics and advanced drugs that don't fit neatly into existing access frameworks.

We take a look at all these intersecting topics in this issue of Deep Dive – with experts from across the industry sharing their predictions for the future and their advice on how companies can navigate these paradigm shifts.

This month we've also focused on new ways to bring the patient voice into the industry, from drug development to post-marketing support – and it's been encouraging to see several of our thought leaders highlight how patients are becoming increasingly important for HTA approval.

It's just another example of how the industry needs to think more broadly at every level – when it comes to success in the current environment, every solution, stakeholder and function counts.

I hope you're all staying safe in these unpredictable times!

Kind regards,

George Underwood
Editor, *Deep Dive*

Next issue:
ASCO & Oncology 2021
Plus:
- Improving cancer outcomes post-COVID
- Digital health startups to watch
- New approaches to oncology trials

Catch up on recent issues:

R&D – February 2021

Patients & Partnerships – December 2020

Digital Health Innovation – October 2020

Communications – September 2020

The Future of Oncology – July 2020

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Turning words into actions with patient engagement

Boehringer Ingelheim's Carinne Brouillon tells us how the company co-created its recent Global Patient Partnership Summit with patients and how the meeting brought representatives together to design actionable projects for pharma-patient engagement.

As pharma seeks new and better ways to listen to the patient voice – particularly in the context of COVID-19 – it needs actionable goals, co-created with patients, for how it can improve its engagement efforts.

That was the philosophy behind Boehringer Ingelheim's recent Global Patient Partnership Summit, according to the company's head of Human Pharma Carinne Brouillon.

Brouillon says that the summit was the natural next step for a company that has continuously ramped up its patient engagement efforts over the last few years.

This has included running ad boards in connection with patient groups in order to understand the patient perspective on specific therapy areas.

"That's been a positive relationship that has helped us develop programmes on everything from access to medicines and understanding diseases, to developing medical information leaflets for patients," says Brouillon. "We want to differentiate ourselves by the inclusion of patients in as many different activities along the development and the commercialisation pathway as possible.

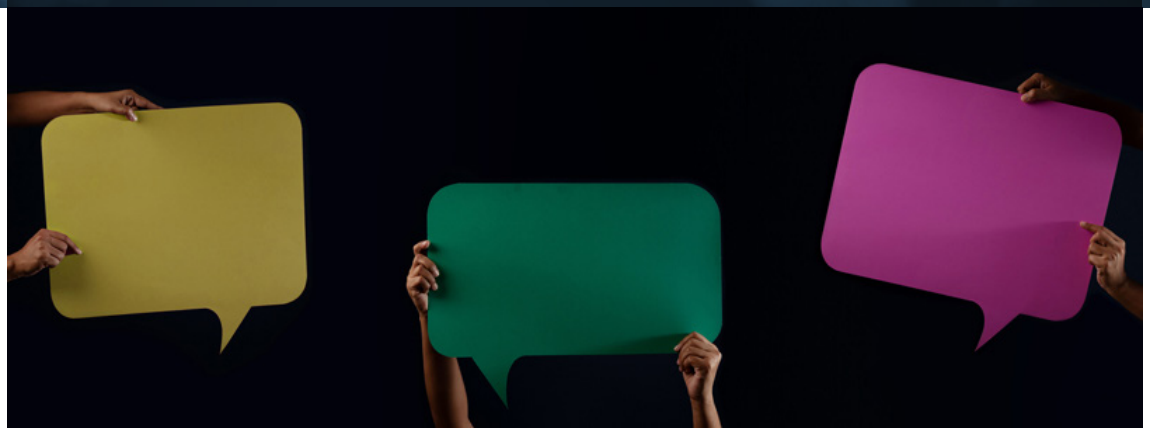
The idea for the Global Patient Partnership Summit came about after the company spotted common themes across all these activities in terms of what patients were saying was important to them. These included raising awareness, access to care, collaboration and innovation, and digital.

The goal was to provide a platform for a global patient organisation and a meeting of groups who may have never met.

Boehringer Ingelheim worked with a patient representative steering committee to co-create an agenda for the Summit that reflected what the attendees wanted to discuss and the challenges they faced. Brouillon says that the company wanted to take a "listening" role to hear the patient voice directly.

"In the past – for example with the ad boards – we've often been focused on the questions that were relevant to us as a company, or only relevant to one specific group of patients," she says.

"The idea here was instead to look at common themes and to come up with actionable goals for how Boehringer Ingelheim and the industry can help patients."



She says there were several topics Boehringer Ingelheim might not have covered in such an event if it weren't for patient input.

"If it hadn't been for the co-creation element, we would probably have spent more time on outcomes that make sense for patients in clinical trials and other aspects that are more useful for us as a company, rather than discussing how to navigate diseases and diagnosis, what the next steps after a diagnosis might be, what having a particular disease means, etc.

"That patient lens has been really important in helping us to define that understanding of how people actually experience disease. As a company we obviously have a strong medical understanding of diseases, but actually navigating a condition when you live with it is an entirely different situation."

The winning projects

The original plan was for the summit to take place in the company's hometown of Ingelheim, but the COVID-19 pandemic meant it instead took place online.

In the end, more than 500 people attended, including 270 patient representatives from 50 countries.

"The enthusiasm of everyone involved was unbelievable," says Brouillon. "It was so humbling to feel that energy and see everyone coming at it with a proactive mindset."

Forty-eight different workshops took place over the two days, with participants eventually agreeing on eight 'winning' project proposals to be pursued further with project teams. Representatives will also work with the steering committee to identify the next steps from the outcomes of the event as part of a report.

Some of the winning projects included:

- The creation of a navigator programme for remote clinical trials, to provide a best-practice toolkit which can be implemented across different disease areas and geographical regions. It was identified that making participation in trials as convenient as possible would lower the likelihood of geographical bias and encourage a more diverse participant group.
- Through multi-stakeholder development across, the creation of disease-specific roadmaps to help guide patients through their interactions with the healthcare system. The project will begin as pilots in a small number of disease areas and by geographical area. Learnings will then be taken by the case study projects and adaptations made to further improve the delivery for patients.

- Exploring the use and testing of digital solutions with patient representatives right from the start, so that the patient voice is actively included in all stages of development. The end result being a solution which has already been identified as a viable benefit for the patient in managing their condition or treatment.
- Providing solutions for patient organisations to be able to engage and maintain a relationship of open dialogues with their stakeholders, in order to break down barriers and identify new approaches to collaboration. Solutions will focus on supporting both parties by creating a framework to follow as plans of what they hope to achieve are developed.

Brouillon says her goal now is to take that energy and momentum and continue to reinforce patient engagement within Boehringer Ingelheim, whilst also ensuring these projects can continue helping patients.

"We want this to be a long-term effort," she adds. "A key part of the summit was helping us create more relationships with patient organisations, and it's important that we maintain continuity with these connections and make sure that all these activities are not just one-shots

"Now the goal is to continue the dialogue around what patients need and make sure we put extra effort into listening to each other and discussing how we can collaborate further."

Brouillon also hopes that the insights from the Summit will help Boehringer Ingelheim to "meet the patient where they are".

"We are an innovative therapies company and we want our innovation to meet the right patients at the right place. The more we understand how patients navigate diseases, the better we will be able to do that.

"For example, although we already have a strong presence in digital, it's important that we work with patient organisations to find the best ways to help them actually use these tools, and learn how we can gather data that will really help understand their disease. We have to make sure these technologies are actually properly designed for how patients want to use them."

She adds that the company is planning to run another summit in a couple of years' time.

"We do want to make sure we're not doing the event too often, because we need time to get the projects off the ground and assess how they're doing."

Meanwhile, Brouillon wants to make sure the company continues with its other patient engagement efforts.

"Last year we ran 26 patient ad boards, and this year we'll hopefully run at least that many again. We also want to make sure that all our development programmes include the patient voice so that we are not missing any important points of view.

"We've learned a lot from actually talking to patients like this – just spending time with them, asking about their disease, and finding out what the pain points are.

"Best practice for patient engagement is not rocket science. It's really just about talking to each other."

About the interviewee

Carinne Brouillon is a member of the Board of Managing Directors at Boehringer Ingelheim with responsibility for the Human Pharma Business. During a 20-year career with Johnson & Johnson, Carinne held various positions of increasing responsibility in sales, marketing and general management. In her following role as president of Janssen Therapeutics Carinne had responsibility for the US commercial Infectious Diseases business. She was then global commercial strategy leader at Janssen. Carinne joined Boehringer Ingelheim in 2018 as head of Global Therapeutic Areas.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

“A long way to go” for digital health reimbursement

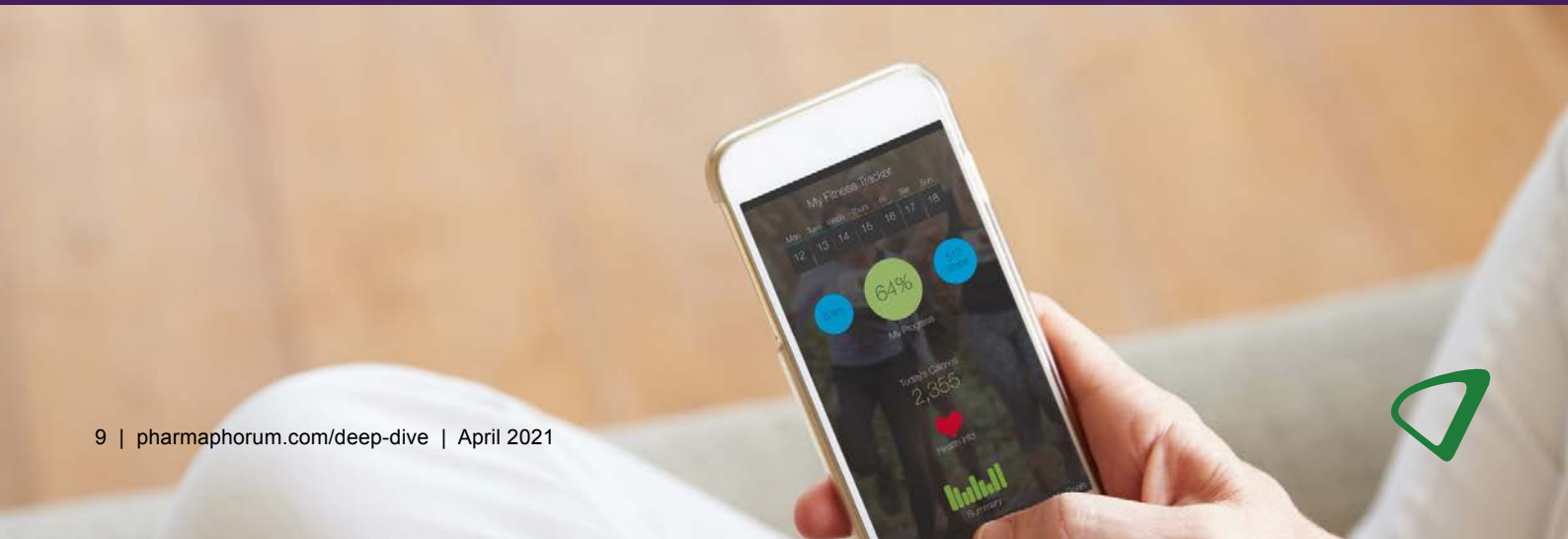
A recent analysis delves into the challenges companies face in getting digital health technologies reimbursed and presents recommendations for how these products can find a smoother path to market.

In many ways, the healthcare sector is still wrestling with how best to regulate and reimburse digital health technologies (DHTs) – which include artificial intelligence, cloud-based services and digital devices – and as a result uptake of these technologies has been slow with patients and payers alike, despite clear interest in their utility.

Most existing value assessment methodologies are designed for pharmaceutical products and are not well-suited to DHTs. Compounding this problem is the fact that the healthcare sector is often glacial in its pace of change, while digital innovations are evolving rapidly every year. Nevertheless, with many DHTs showing great promise in helping patients and creating value for payers, a more pragmatic approach to reimbursement is needed soon.

Progress is being made in several markets, and Dr Lorenzo D’Angelo, principal in CRA’s Life Sciences Practice, notes that COVID-19 could act as a catalyst for national and local reimbursement systems to start tackling this issue.

“While there are still roadblocks, the pandemic has opened up opportunities and accelerated the acceptance of this technology – and with that acceptance comes pressure on payers to see more of these technologies funded,” he says.



But uncertainty remains over optimal trial designs for gathering evidence, the kinds of value payers actually want to see, and the reimbursement and coverage options available. The FDA, for example, has gone back and forth on whether to keep a truncated digital health pathway implemented during COVID-19 in place after the pandemic.

In a recent report, 'Challenges and solutions to bringing digital health technologies to market', analysts from CRA take a look at the major access and regulatory barriers facing companies attempting to launch DHTs, and ask how the industry can overcome these challenges and work with payers to make access pathways easier to navigate.

The authors note that there is a "long way to go" to achieve this – but there is a strong public health need for standardisation to encourage the development and appropriate utilisation of digital health innovations.

And within this environment, the pharma and digital health industries have an opportunity to show leadership in helping reimbursement for DHTs find its footing.

Dedicated pathways for digital health

A key issue identified by the analysis is the lack of dedicated access pathways or value assessment processes for DHTs in many countries.

"The situation varies greatly on a country to country basis," says D'Angelo, who was one of the report's authors.

Across Europe there is a mix of nationally- and regionally-funded programmes, with different payers having different guidelines for DHT adoption.

"While most countries are moving towards standard value assessment frameworks, in some countries it is evolving more quickly than others," D'Angelo adds.

The analysis contrasts Germany's efforts with the UK's as an example.

Germany's Digital Healthcare Act, approved in December 2019, allows doctors to prescribe health apps to patients and mandates that all insured persons have equal and self-determined access to the advantages offered by digitalisation.

Doctors are obliged to connect to the centralised system, and the new law introduced financial penalties for those not connected by 1 March 2020.

Germany also implemented a structured HTA process for apps. After the evidence package is reviewed, and if comparative trials are available, the apps are included in a public list. Conditional reimbursement is available for apps where trials still need to be developed. The assessment forms the basis of price negotiation, which occurs within a year.

"For a long time Germany was behind other European countries in terms of reimbursement pathways," says D'Angelo. "Now the new act clearly defines how digital therapeutics can be reimbursed, and we're already seeing apps being reimbursed through that pathway."

In the UK, meanwhile, funding sources are not unified under a national effort. For example, NHS England's Innovation and Technology Tariff (ITT) and the Innovation and Technology Payment (ITP) are available at the national level, while clinical commissioning funding exists at the regional level.

NICE Medtech Innovation Briefings do include the DHT's role in the treatment pathway as well as a review of published evidence and the likely associated cost. The aim of these national briefings is to ease local decision-making by providing a factual overview – but they ultimately do not make recommendations.

D'Angelo adds that the UK's approach is still falling under existing pathways for medical devices, which is the case in many countries at the moment.

"The problem is, these pathways are usually more applicable to traditional medical devices where software is either a side aspect or is not present at all, whereas many DHTs today are fully software-based," he says.

Moreover, these pathways are often actually designed to reimburse a procedure rather than a specific device.

"The way that DHTs are often prescribed, however – especially in the case of apps – is that they are used directly by the patients themselves. It's actually closer to a pharmaceutical than to a traditional medical device.

"What would be helpful in countries like the UK is having a pathway that is more specific for digital healthcare, so that it's more transparent and easier to use for manufacturers."

D'Angelo adds that DHT manufacturers have a role to play in working with policymakers to raise awareness that these specific pathways are required.

"They need to be made aware of this gap, as well as the pressures from both the increasing acceptance of digital in other industries and the changes from COVID-19. Thankfully, I think this is a process that is already happening."

Evidence of value

Even with a dedicated reimbursement pathway in place, DHTs will need to demonstrate evidence of patient outcomes and value to healthcare systems – but many manufacturers face difficulties in gathering such evidence, especially when HTA bodies often apply the same evidence standards for pharmaceuticals to DHTs.

CRA's report says that HTAs expecting results from multiple randomised clinical trials (RCTs) to be available at the time of launch for DHTs only creates hurdles. Often much of the data for these products is collected post-launch as real-world evidence.

On top of that, there is a lack of standardised and dedicated value assessment methods for DHTs. The analysis notes that there is currently "little consensus" on what defines value in digital innovations, with a lack of clarity on evidence requirements.

Part of the issue is the huge variety within the DHT space – some provide indirect value to the patient, e.g. through reminding them to take their medication, while others focus on direct treatment such as cognitive behavioural therapy, and some are designed more to provide value to a healthcare system itself – for example, by collecting more precise information on drug response.

"DHTs need a robust and transparent validation process that could benefit the whole healthcare system," the analysis says in its recommendations. "Payers should provide a standardised, objective, rigorous, and transparent process describing what evidence should be submitted and how it needs to be collected depending on the indication and the specific aspect the device is targeting."

Nevertheless, improved patient outcomes and potential cost savings for healthcare systems will always be key for payers, and the analysis recommends that manufacturers try to define and demonstrate both indication-specific value and implications for healthcare systems in order to increase the likelihood of reimbursement.

D'Angelo says that to drive a consensus on the value of DHTs in specific indications, and identify how this value can be measured, companies need to be engaging with influential stakeholders.

"You need to be able to understand from their perspective what value looks like – what they're interested in and how they think it is most likely to get reimbursed. At the same time, you can discuss what pathways exist and what opportunities there are to get a strong revenue stream from that technology."

He says that in addition to payers, these stakeholders could include consumers and opinion leaders in the indications being addressed, as well as patient advocacy groups and physicians.

"Patients can advocate the technology, either in discussions with physicians or in discussions with payers. It's also important to get their feedback on how the product functions, and what it needs to be providing in order for patients to find it useful.

"Meanwhile, speaking to physicians and carers can help you understand what value that specific technology could bring in routine use, and what endpoints and trial designs could demonstrate that."

He adds: "Depending on the technology, I'd also want to reach out to partners in the sector to see if they could be interested in using the technology for their own purposes. You might not even need a payer to reimburse it if you can open a revenue stream with a pharma company, for example.

"Technologies that can monitor data in conjunction with a drug can be enormously valuable to pharma by helping to extend the life cycle of a product. They might be willing to pay for the application even if they allow patients to use it for free."

Planning for the future

As the value assessment of DHTs becomes more standardised and transparent, the process will become easier to navigate for companies. As a result, the analysis says, DHT manufacturers should become more targeted in their development and testing as they keep in mind payer value drivers.

Until then, the authors suggest that companies explore and prioritise the multiple decentralised reimbursement routes currently available, as well as seeking partnering opportunities at an early stage of development to help create and test products.

"Companies should keep a close eye on the latest developments in DHT reimbursement," says D'Angelo. "Things are developing quickly in terms of new pathways and tenders, and knowing what different regions are offering can help in both planning for reimbursement and for designing trials and pilots."

About the interviewee

Lorenzo D'Angelo is a principal in the Life Sciences Practice of CRA based in Munich. Dr D'Angelo is an experienced consultant helping global pharmaceutical and medical device companies with their commercial strategies.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

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Time for a NICE change

The National Institute for Health and Care Excellence (NICE) is world-renowned for their work assessing the clinical and cost-effectiveness of new treatments. In recognition of how the world has changed over its history, NICE is coming to the end of a period of review and consultation on their processes and methods. Leela Barham takes stock.

Drivers for the reviews

NICE has never stood still since it started its work in 1999. The current consultation – review of the health technology processes – is just the latest and closed on 15 April. An earlier consultation that closed on 18 December 2020, covered methods. NICE also consulted on topic selection as well as a case for change for methods for health technology evaluation.

NICE's consultations have generated attention because what NICE says matters, not only for treatments launched in the UK, but because the institute makes their guidance available to anyone, anywhere. Many agencies model their own work on what NICE does. Some countries even formally reference the agency's recommendations when making their own pricing and reimbursement determination.

NICE itself has positioned the latest methods and process reviews as helping the institute support the healthcare and life sciences ecosystem. Lots of buzzwords feature: flexible, agile, robust, future proof, rapid access. From an internal perspective, the process review is a chance to help the institute deliver quality, dependability, speed, flexibility and cost. And anyone interested in a game of policy bingo will be able to mark off all the important policy documents and every agency too.

Taking a health technology management approach

The proposals set out in the process consultation includes the shift to NICE taking a health technology management approach, including disinvestment, and suggests a move to 'living' guidelines.

The idea of disinvestment is not new and resonates with the arguments that have been tabled in the past about providing headroom for innovation – showing that what goes around, comes around.

These process changes are complementary to much in the methods consultation. NICE has pitched the idea of modifiers that capture severity of disease and refining how uncertainty is dealt with, which will mean allowing for evidence generation. It's the evidence generation that will link to the idea of managing an intervention over time, and responding to how the evidence changes is part of the approach to a technology over its lifecycle. That's not all that new, but perhaps the change in emphasis is welcome.

Aiming for consistency

NICE has recognised that it has, mostly because of the different starting points of the different areas of its work, been using different terminology and different processes. The process review is not just for the Technology Appraisal programme, and that means it's a chance to remove some anomalies and bring in some consistent processes across programmes including diagnostics and digital. For example, allowing for a technical engagement process across the work of the organisation.

But there is a danger too from this; every technology will get the same opportunity but the opportunity might be a pick and mix approach determined by NICE – and that may worry some stakeholders. NICE is going to decide if they'll be doing a long consultation (20 working days), short (5-20 working days) and/or having a scoping workshop.

For companies, the underpinning message from NICE is clear; they need to get their ducks in a row far in advance of a NICE evaluation, both in terms of planning ahead to put together the most appropriate analysis of the data to provide the best evidence, but also for any commercial discussion with NHS England & Improvement (NHSE&I).

Companies need to recognise that much of what NICE sets out in their process consultation is really about NICE driving the process. For example, NICE will determine if an evaluation is to be terminated not because of a company not submitting – the approach taken to date – but because the value proposition is higher than the standard threshold and it can't be solved through a commercial deal. It's going to be a NICE – and NHSE&I – run show. Even more advantageous discount rates and more modifiers as proposed in the methods consultation won't change the drive for better value for money.

But allowing for differences

Whilst consistency is an underpinning theme, there is scope for differences when that makes sense too. The methods proposals allow, for example, for consistent modifiers to apply across evaluation programmes but acknowledges that how they are used may differ.

The process proposals include the option for some steps to be skipped. For example, the proposals include opportunities to route promising technologies directly into management access without a full health technology evaluation. The challenge is that it's hard to unpick from the consultation document when this might be an option and whether that would be attractive to a company.

Industry may need to pay more to NICE

Companies already have to pay a charge – that differs according to type of Technology Appraisal and size of company – to pay for the work that NICE does. The NICE process consultation recognises that some of the proposals for change they are making could save them time, but others may need more. The net impact hasn't been worked through, but the institute has noted that the changes could result in modified charges to companies. My money is on higher charges.

My money too is on companies needing to do more in their submissions; the danger of the methods proposals is that NICE is extending the range of data and analysis, and hence the evidence, that might be needed for an appraisal. The problem with that is this increases the existing temptation for NICE staffers, those at the Evidence Review Groups (independent academics), and committee members to go fishing; just to see what change a different approach makes to the results of economic models. It's easy to ask for (another) analysis or tweak when it's not your time and money. With many companies using agencies to help them do submissions, it's likely to become a more costly exercise.

Proposals need to become concrete

The NICE proposals, both on methods and process, are light on detail. For example, NICE proposes to develop a single list of considerations for use by NICE technology evaluation committees to guide the development of recommendations for use in managed access. But what these considerations are isn't spelt out.

For methods, to illustrate the same point, the proposals suggest that there may be a case to adopt the same 1.5% discount rate for both costs and health effects, but that the wider consequences of this change – affordability being one key issue cited – need to be worked through before a change can be made by NICE.

In some cases, NICE is tying itself in knots to express its intention too – with the idea that new criteria to determine what will be considered under the Highly Specialised Technology (HST) programme should be "precise, carefully defined, 'yes/no' criteria where possible, but worded such that flexibility and judgement is permitted when appropriate". Good luck to the NICE staffer who can deliver on this.

This makes it hard to know whether the changes proposed are really an opportunity for industry and other stakeholders, not least patients, or simply warm words.

Next consultation in August 2021

NICE's workplan still includes a great deal more to deliver, including a consultation on the draft programme manual in August 2021. December 2021 is the pencilled in day for the publication of the final version and that will be a key document shaping future submissions to NICE and how they'll manage the process and ultimately inform how decisions will be made by committees. Changes won't come through until July 2022.

Whatever people think about the proposals, NICE staffers should be acknowledged for their staying power as the in-house work on these consultations must feel never-ending. Realistically whatever NICE does change, it won't deliver on the different agendas of the stakeholders with an interest in the institute's work.

About the author

Leela Barham is a researcher and writer who has worked with all stakeholders across the health care system, both in the UK and internationally, on the economics of the pharmaceutical industry. Leela worked as an advisor to the Department of Health and Social Care on the 2019 Voluntary Scheme for Branded Medicines Pricing and Access (VPAS).

How will NICE fare in a post-Brexit world?

NICE has a global reputation as a pioneering HTA – but is that influence at risk now that the UK has left the EU? Experts from ICON give us their views on the past, present and future of NICE's standing on the world stage.

One of the earliest and most pronounced impacts of the UK's vote to leave the European Union was the loss of the European Medicines Agency (EMA). Long headquartered in London, the EU drug regulator is now severed from the country both physically and systematically, with its base now in Amsterdam and the UK now assessing drugs through its own regulatory agency, MHRA.

This leaves the UK's drug pricing watchdog, the National Institute for Health and Care Excellence (NICE), in an uncertain position. Once a leading voice in health technology assessment (HTA) in both Europe and the wider world, the body now finds itself unable to work as closely with the EMA – and by extension countries in the EU – as it did in the past.

Emmanuel Lacharme, previously a technical analyst at NICE and now senior consultant, global health economics and outcomes research at ICON, is confident that NICE's existing reputation will help it maintain its global standing in the immediate future – but notes that there is still a risk the body will become "isolated" as a result of Brexit, particularly as companies are no longer able to rely on the EU's centralised approval system for the UK.

"There will be uncertain times, but also new opportunities," he says.

"I believe that NICE will try to do as much as possible to support our industry, by offering early engagements and a streamlined MHRA and NICE parallel approval process. Practically though, there are risks of additional processes and requirements (and expense) for pharmaceutical companies. This additional effort could lead to poorer patient access.

"It is also possible that companies may prefer to enter the EU market through the EMA process and avoid additional processes involving the MHRA and NICE. It's important to remember that the UK market represents only 2.5% of global pharmaceutical sales, while the entire European market represents more than 20%."

Caroline Delaitre-Bonnin, senior principal, global health economics and outcomes research at ICON, notes that this could lead to companies no longer seeing the UK as an early launch market.

"The UK has traditionally been an early launch market for pharmaceuticals partly because UK prices are often used as a reference price for other countries, and also because NICE assessments are held in high esteem. Brexit could change this.

"It's difficult to say whether NICE will maintain the same leadership position in Europe that it has in past years."

A strong foundation

But uncertainty goes both ways, and at the moment it is also too early to say that NICE won't be able to maintain – or perhaps even strengthen – its influence under the right circumstances.

Lacharme points out that NICE does, at the very least, have a very strong foundation to build upon.

"NICE has always been a great reference in HTA for cost-effectiveness analysis (CEA) and cost-utility analysis (CUA) of new medicines," he says. "For example, its 2013 guide to the methods of technology appraisal is still a valid reference to understand the key principles of cost effectiveness within an HTA context, and NICE decision support unit documents are a useful resource for any health economist needing technical or educational support.

"While NICE's first target is the UK NHS, this information can also be useful for other EU health services – especially when a country has not developed its own guidance – and even the US, where evaluation from the institute for clinical and economic review becoming more important, using NICE principles of CEA/CUA."

He adds that NICE's strong reputation is also due to its openness to industry involvement and collaboration.

"The current public consultation period for the NICE methods review is one example of this."

With NICE having had a significant influence on the development of other HTA authorities' methodologies over the last 20 years, Manpreet Sidhu – ICON's executive principal, global health economics and outcomes research – says the agency's influence has a good chance of remaining robust over the coming years.

"NICE is still considered to offer a transparent and clear methodological framework for regulators and companies, and so whilst the intricacies of working in a post-Brexit world are ironed out it would seem unlikely that pharma companies/regulators would move away from continuing to engage with NICE."

And while the largest impact is likely to be companies launching in the UK no longer being able to use centralised EMA submissions process, Sidhu notes that the evidence requirements of NICE and EU countries have not changed.

"There is some work to do with respect to aligning methodologies and streamlining these requirements across all of the EU to facilitate the process of evidence generation for pharma companies, but that need exists without Brexit."

Because of this, Sidhu says it will be important for NICE to continue to lead discussions on methodological perspectives within HTA bodies if it wants to maintain its reputation.

"Being a flagship HTA, where other HTA bodies look to NICE methodologies as a signpost, has been great for the agency," she says.

"To continue on that trajectory NICE needs to continue engaging with other HTA bodies on joint commissioning of advice and guidance, and utilising cohesive methodologies."

In fact, NICE has not stopped working with global agencies on collaborative methodologies – one example being the Orbis Project, a joint programme involving the US, Canada, Switzerland, Singapore and Brazil that aims to review and approve promising cancer treatments months ahead of the EU.

"NICE is conscious of its key role in HTA," says Lacharme, "and it wants to be sure that the UK remains a destination of choice for the life sciences sector. It is still working collaboratively with global health system partners, and more particularly with the MHRA, to design a streamlined process for licensing and evaluating new medicines."

Early scientific advice

One key way NICE has worked with other HTAs in the past is through parallel scientific advice – and this may now be another strength it can leverage to maintain its global standing.

NICE has long had significant clout in these early dialogues. The agency was one of the first HTA bodies to offer regular activity on early scientific advice, which strengthened its influence across Europe.

When the European Network for Health Technology Assessment (EUnetHTA) was founded in 2006 to help provide parallel early dialogues from multiple HTAs across the region, NICE was one of the most frequently selected bodies for companies to consult with, further strengthening this influence. In fact, getting advice from NICE as part of a parallel dialogue was more popular than talking to the agency alone.

Currently there are two main options for parallel early dialogues via EUnetHTA – EMA-EUnetHTA consultations, where companies consult with both HTA bodies and the EMA itself, and EUnetHTA multi-HTA dialogues, which take into account the opinions of HTA bodies only.

Pre-Brexit, NICE was particularly invested in early dialogues involving the EMA, but now, although it remains a part of Europe's Early Dialogues Working Party (EDWP), it can only take part in parallel dialogues that don't involve the regulator.

Delaitre-Bonnin says that NICE's continued relationship with the EDWP will be key to mitigating the potential negative impacts of its isolation from the centralised EMA system.

She notes that the agency has already announced new approaches aiming to deliver scientific advice in similar timelines to the EMA process to help with this.

"This new way replaces the parallel advice service that NICE delivered with the EMA and is performed via the EUnetHTA.

"Companies can use this approach in two different situations – either they've requested regulatory advice from the EMA and need advice from NICE at the same time, or they've requested a European parallel consultation with the EUnetHTA and have been rejected."

Over the last five years the European Commission has repeatedly proposed to strengthen cooperation across HTAs in the EU, suggesting new regulations for common European HTA methods, sharing data and expertise, and common procedures across the EU.

"As the UK will not be an EU Member State, NICE and the other HTA agencies in the UK – the Scottish Medicines Consortium (SMC) and the All Wales Medicines Strategy Group (AWMSG) – will have a limited role to play in this new strategy," Delaitre-Bonnin says.

"That is why NICE needs to organise its services in closer collaboration with EUnetHTA for multi-HTA early dialogues and introduce concurrent advice in similar timelines to the EMA."

NICE is also looking to apply similar parallel-advice principles to other global regulators – one example being through its relationship with the Canadian Agency for Drugs and Technology in Health (CADTH).

"Moreover, NICE continues to offer advice in collaboration with the MHRA," says Delaitre-Bonnin.

Delaitre-Bonnin believes that NICE's continued expertise in areas such as providing detailed feedback on companies' evidence generation plans, helping companies understand the perspective of decision makers, and providing access to patient, NHS and academic experts, means that early dialogues delivered by the agency will still be both "relevant and expected" by pharmaceutical companies.

But she adds that it's important to keep in mind that some other HTA bodies like Zorginstituut Nederland (ZiN) in the Netherlands, which is also a member of the EDWP, have been more innovative in reviewing and changing their guidelines, and could soon emerge as strong competitors in this space.

"Meanwhile, we need to remember that multi-criteria decision analysis and value frameworks highlight that there is more to HTA than an ICER."

She says that now the country's market is separated from the rest of the EU, the UK could be further highlighted to the industry by strengthening early engagement and offering a fast-track MHRA/NICE parallel approval process.

"We could also imagine that this early approval in the UK might lead to opportunities to generate early-stage real world evidence that could be supportive for any other HTA submissions and pricing negotiations in Europe."

Combatting uncertainty

There are, of course, still many unanswered questions – and Sidhu notes that the industry is generally operating in a "wait and see" mode.

"They are preparing for NICE and EU HTA submissions as they have done previously, knowing that things may change but also that this change is likely to be incremental."

Nevertheless, Sidhu says it's important that companies read up on insights from people knowledgeable about working with NICE, like those on ICON's blog, to "future proof" development plans for assets.

Meanwhile, Delaitre-Bonnin says that ICON is advising clients in the current climate to seek parallel engagement with NICE and other regulators and HTA agencies on early scientific advice.

"The quality and the relevance of early dialogues with NICE have already been recognised," she says, "and their value for some other European countries is likely to remain for several years to come."

About the interviewees

Caroline Delaitre-Bonnin – senior principal, global health economics and outcomes research, ICON
Caroline has over 15 years of healthcare experience spanning industry and consulting. Her primary focus is on HTA and value communications projects with extensive experience in oncology, haematology, gastroenterology and orphan drugs. She worked on projects in HTA submissions, developing HTA evidence development strategies to support launch products, strategic integrated scientific advice, strategic advice and developing value communication tools.

Emmanuel Lacharme – senior consultant, global health economics and outcomes research, ICON
Prior to joining ICON in 2020, Emmanuel's experience included positions in various Paris Public hospitals (AP-HP) in clinical trials, early access (ATU), quality insurance, medical device, purchase, and clinical pharmacy (psychiatry, neurology) departments. At Novartis he worked as a project manager on market access, pricing and reimbursement in the oncology department. At the French National Authority for Health (HAS) he was a HTA analyst in economic and clinical drug assessment. At NICE he was a technical analyst in the scientific advice department.

Manpreet Sidhu – executive principal, global health economics and outcomes research, ICON
Manpreet has over 20 years of experience in health economics, including strategic evidence generation planning, project management, systematic reviews, economic modelling, strategic market access, and dossier development. At ICON, Manpreet is business lead for global health economics and outcomes research, incorporating patient centred outcomes, health economics and HTA, and value communications, and is responsible for overall project delivery and strategic direction of the practice.

About ICON

ICON is a global provider of consulting, and outsourced development and commercialisation services to pharmaceutical, biotechnology, medical device and government and public health organisations. ICON focuses on the factors that are critical to clients – reducing time to market, reducing cost and increasing quality – and its global team of experts has extensive experience in a broad range of therapeutic areas. ICON has been recognised as one of the world's leading contract research organisations through a number of high-profile industry awards. With headquarters in Dublin, Ireland, ICON employs approximately 16,000 employees in 93 locations in 41 countries. Further information is available at www.iconplc.com.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

AI meets RWE: The future of drug assessment?

Savana's Dr Ignacio Medrano explains how machine learning and natural language processing technology is opening up huge amounts of previously-inaccessible real-world evidence for pharma and regulators.

Real world evidence (RWE) is rapidly evolving. Having gone from being an innovation buzzword to becoming a core part of drug assessment processes it offers health technology assessment (HTA) bodies a way to assess the value and impact of drugs outside of an artificial clinical trial setting.

"For years we've been aware that what happens in clinical trials is just a fragment of the reality of how medicines and healthcare work in the real world," says Dr Ignacio Medrano, co-founder and CMO of Savana. "You realise that when you talk to doctors and listen to their real-life experiences.

"That's not to say that clinical trials are redundant. It's still important to understand cause and effect without bias. However, once clinical trials are completed, there has to be something else.

"There are a huge number of variables that are difficult, if not impossible, for pharma and regulators to account for in traditional clinical trials. Ultimately, these studies are always going to feature limited populations that can't represent the full spectrum of backgrounds, situations and behaviours of patients and doctors in the real world – and all those factors can have massive impacts on how successful an intervention actually is."

Now, Medrano says, the industry finally has the technology to analyse the large-scale patterns arising from these variables.

But not all forms of RWE are created equal, he says, and in fact current methods of collecting RWE data don't always produce insights that are useful to researchers or HTA bodies.

"One problem is that most RWE is contained in inflexible, siloed databases. Companies have to pay to access these databases, but they can't always be sure that the exact data they need will be found within.

"That's a pity, because when the information isn't there, it's usually not because it doesn't exist – in fact it's being generated every single day by hospitals and electronic health records (EHRs). It's just not in that particular database."

Luckily, the sector is evolving to a point where life science companies can use AI tools to directly analyse electronic health records and extract the exact information they need for their particular market access purpose.

"That could be compared effectiveness, epidemiology, the patient journey, etc. – the important thing is that it can be tailored for each company," says Medrano. "We can design a study that uses AI models to dive into these texts and search for whatever a company needs. Then you can analyse it as many times as you want."

This is the focus of Savana's own technology, which uses natural language processing (NLP) to extract and read unstructured information buried within the free text of clinical notes and EHRs.

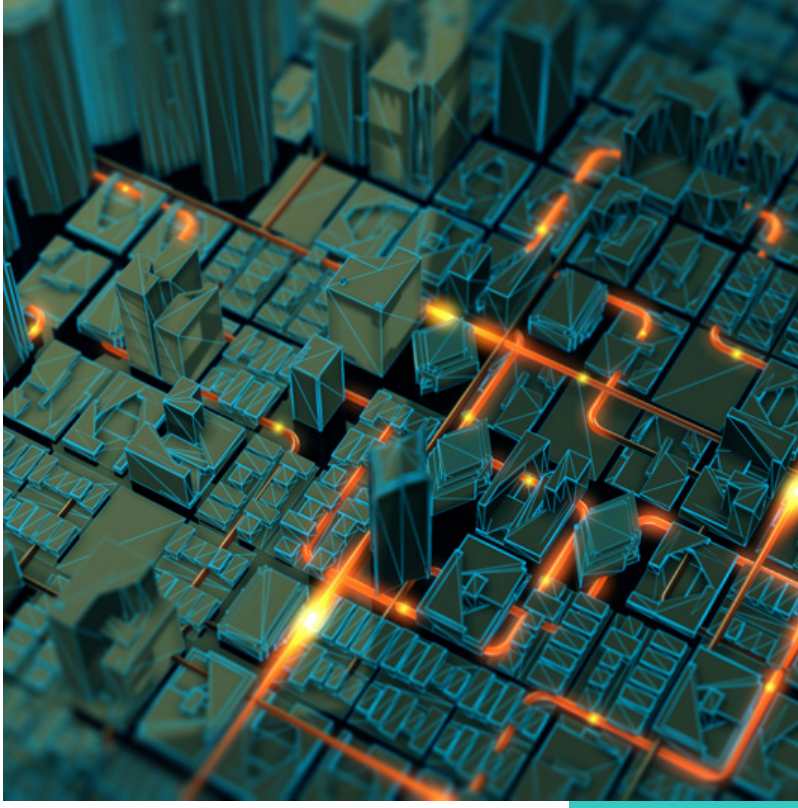
The technology and clinical research methodology can therefore enable clinicians and health researchers to analyse vast amounts of previously inaccessible clinical data.

The idea came from Medrano's personal frustrations while working as a neurology consultant.

"I got weary of entering data in the electronic health records and not getting anything in return. There was a lot of valuable information that was lost to me."

Now, computational linguistics is making this information readily available to pharma companies and regulators – and the benefits could go beyond ease of access.

Faster data collection



Value-based contracting – where post-launch RWE is used by payers to link prices to how a drug performs in a real-world setting – is another area that could benefit immensely from a shift towards more AI-based approaches.

"One of the problems with value-based contracting at the moment is that gathering RWE on how well a drug is working often involves doctors manually populating databases with each outcome case, usually at the end of the working day," says Medrano.

"Why don't we automate that? Why don't we apply natural language processing to extract the outcomes automatically, allowing us to adjust the reimbursement with the support of AI? That's now completely doable, and in fact we've already begun working on that in North America and some Eastern countries."

On top of this, while Medrano says traditional databases can be "incredibly limited" for observational studies across wide populations, AI tech is allowing this kind of research to thrive in new ways.

"Observational studies can be extremely expensive and very slow. Now with this technology, we are able to quickly connect massive populations across primary care, specialised care, inpatient, outpatient, pathology – every single time the patient touches a point of care, we can capture that. For the first time, we can gather all those variables – tens of thousands of them – and connect them to understand what is happening.

"That can be extremely useful for market access purposes."

Predictive modelling based on machine learning will also be a huge boon to RWE collection – and a useful tool for HTA bodies.

"That can be really important for positioning in a pathology, by understanding the risk of an outcome not based on classical statistics, but on neural networks that have already been used by other industries for years and can now be applied in medicine.

"That might even include 'deep screening' to find patients that are undiagnosed."

Changing attitudes

Of course, none of this will be useful if HTA bodies themselves don't embrace RWE and associated AI tech – but Medrano says things are looking bright in that sense, and in areas where regulators are falling behind it is mostly due to culture and existing regulations, rather than technology.

"There's really not a technological or methodological reason not to trust these RWE databases for making decisions. We have evaluation methodologies where we can demonstrate how reliable the databases are, and with random sampling we can check how good the information extraction is. It's very transparent.

"The only progress that needs to be made is from a purely human, regulatory perspective – but we are already proving how useful and high-quality the data can be, which is important for being able to move forward in this regard."

Medrano adds that regulatory offices are generally "open, positive and optimistic" with regards to RWE and future AI applications.

"Of course, they still have to be cautious – that's their job, after all – and companies working in this space will have to continue to prove their reliability. There have been a good number of poorly designed machine learning-based algorithms, and we see many low-level publications that don't follow the standard guidelines for scientific publications. That's always something we have to be aware of, and make sure we're not using these technologies to somehow bypass mandatory regulatory steps.

"But regulators are certainly interested in the potential of these new methods and collaboration is starting to bloom. The situation is very clearly moving in this direction."

About the interviewee

Dr Ignacio H Medrano, CMO and founder, Savana is a consultant neurologist with training in healthcare management and experience in clinical research strategies – formerly responsible for over 500 researchers. A Singularity University graduate, he is also a founder at Mendelian in the UK which is utilising AI in the diagnosis of rare diseases. Ignacio is in demand as an international speaker at digital health, clinical research, science and technology events and congresses.

About Savana

SAVANA
Accelerate Health Science

Founded in 2014, Savana is an international medical company that has developed a scientific methodology that applies artificial intelligence (AI) to unlock all the clinical value embedded within the free text of electronic health records (EHRs). With the largest AI-enabled, multi-language, multi-centre research network in the world, Savana generates customised descriptive and predictive, Deep Real World Evidence research studies. Engineered by doctors for doctors, Savana is built following the highest privacy-by-design standards. Savana constitutes a clinical research ecosystem that aims to advance personalised and precision medicine worldwide.

Visit www.savanamed.com or follow the company on LinkedIn and Twitter

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

Research predicts the post-COVID landscape for HCP engagement

IQVIA's Liz Murray takes us through the company's latest research into HCPs' attitudes to pharma engagement, and asks how pharma sales forces can adapt for future changes.

There's no doubt that remote engagement has been extremely important for pharma sales teams over the course of the COVID-19 pandemic, but adapting for the future HCP landscape will require reps to do more than simply take their engagement online.

Recent ChannelDynamics data from IQVIA shows that while there has been a marked positive change in HCPs' views of remote rep platforms over the past 12 months, there has not actually been much change in their views of face-to-face engagement, and there has only been a small positive change for telephone, email and automated online detailing.

"Physicians are not saying that their views of in-person interactions have gone backwards," says Liz Murray, global lead, HCP Engagement Centre of Excellence, Contract Sales & Medical Solutions, at IQVIA. "That's a really interesting point to keep in mind."

Murray also notes that HCPs' attitudes to engagement have varied wildly between different countries both before and during the pandemic.

"Countries like the UK saw a huge slowdown in HCP engagement over COVID, supplemented by remote engagement, and things haven't really changed in the last 12 months," she says. "Meanwhile, countries like Italy are now seeing a shift back towards face-to-face engagement – almost to pre-pandemic levels – while countries like Spain sit somewhere in-between.

"Even before the pandemic, there was an expectation from HCPs in Italy that they would talk to reps on a regular basis, and that doesn't seem to have changed."

These statistics might go against conventional wisdom that digital dominance is here to stay, but Murray says that examples like Italy show that future strategies will not be a question of digital versus face-to-face.

Instead, companies will need to consider how those channels can be blended to fit different HCPs and environments.

"There will certainly be plenty of examples of standalone digital engagement, but we're also going to see reps' roles evolving into a more multichannel-orientated way of working."

And this seems to be something HCPs are keen to see themselves – IQVIA's data suggests that, more than anything else, doctors believe flexibility will be key for the future of engagement.

"HCP satisfaction feedback on remote interactions delivered by IQVIA showed that 96% of HCPs are highly satisfied or satisfied with the remote interactions and 93% are highly likely or likely to repeat the experience in the future," says Murray.

"And HCPs who participated in recent qualitative research by IQVIA, and who are used to regular meetings with representatives, said that face-to-face meetings remain essential to building and maintain trust with the industry.

"What they like about new, remote approaches is that it allows engagement to happen at times that are convenient for them – for example, outside of clinic hours."

"A blended approach might involve using face-to-face interaction only a couple of times a year, then using remote touchpoints in between that."

She says that this will ultimately mean reps are talking to HCPs in a "more connected way".

"The tradition of reps just pushing key messages is probably not going to work anymore. HCPs want content with true added-value; they want it to be solution-focused and considerate of what they actually want and need."

Indeed, Murray notes that IQVIA has already seen a shift away from promotional activities to more medically orientated solutions in some markets.

"In the past, the industry's relationship with HCPs was on industry terms – now companies need to be having relationships on the HCPs' terms."

She adds that companies' vision should ultimately be to personalise each HCP interaction.

"To do that, pharma needs to bring together all its existing customer data – which is often quite fragmented – then apply more advanced business intelligence to shape engagement experiences.

"They can then start to build an understanding of what their customers want, where they want it and when they want it."

But not all companies are as far along this path as they could be.

"The pandemic has been the motivation missing for the past 15 years, forcing the industry to look at alternative ways of doing things, but the speed at which a company actually changes is dependent on how forward-thinking they were beforehand. For example, did they already have e-consents in place, or were they completely reliant on face-to-face and are now having to start from scratch?

"It's going to take longer for those in the latter camp to get up to speed," says Murray. "It might help for them to learn from what other companies have done, or bring in specialists who can help run pilots and look after the back office elements."

Beyond that, companies who need to transform their sales teams from scratch can also look at the existing structures they have in place and build upon those.

"There are some fundamentals those companies need to wrap their heads around, such as getting their strategy together and thinking about the technology infrastructure they need, but the easiest way to get started is to look at the rep teams they've already got in place, which already work well, and build out from there.

"Some reps have already been using phone calls or emails to follow-up with HCPs, but that can be broadened further, towards a more integrated omnichannel approach."

This again shows the importance of a blended approach – and Murray notes that it's sales teams should not overemphasise digital and remote engagement as a catch-all solution,and remember that holistic strategies will be key.

"I hear a lot of people talking about omnichannel now, but omnichannel itself isn't the endgame – it's a means to an end.

"That end is the customer having a good experience and getting what they want to achieve out of the engagement."

About the interviewee

Liz Murray is global lead, HCP Engagement Centre of Excellence, Contract Sales & Medical Solutions, at IQVIA. Liz is a specialist in HCP engagement, and has significant expertise in new channels and remote interactions. Prior to joining IQVIA, she worked at Pfizer for 13 years in sales, marketing, account management, market access, customer strategy, business effectiveness.

About IQVIA

IQVIA is a leading global provider of advanced analytics, technology solutions and clinical research services to the life sciences industry. Powered by the IQVIA CORE™, IQVIA delivers unique and actionable insights at the intersection of large-scale analytics, transformative technology and extensive domain expertise, as well as execution capabilities. Formed through the merger of IMS Health and Quintiles, IQVIA has approximately 68,000 employees worldwide.

Learn more at www.iqvia.com.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

A year on: pandemic driven trends in HCP engagement

IQVIA's John Procter explores why the industry should be paying attention to the changing HCP customer experience in the wake of COVID-19.

It is now just over a year since the WHO declared COVID-19 a global pandemic. For many nations it has been their first experience of responding to such a global emergency in living memory. It has also represented the first time that the sales operating models for global, mass-market pharmaceuticals have been severely and consistently disrupted around the world.

A year on, that disruption continues as third and fourth waves of cases affect many countries and vaccination rates vary markedly between nations. I wrote last year about the impacts and trends we at IQVIA had seen in HCP engagement across the globe, many of which have continued into 2021 as the pandemic effects continue to be felt.

As we reach the end of the first quarter of 2021, our customers continue to be challenged by the question of what they need to do to be successful whilst the uncertainty induced by pandemic disruption continues. Data from across the globe is increasingly giving clues as to the likely answer.

Managing uncertainty

The first thing to recognise is that we are still some way from an established 'new normal' and in most countries our industry remains in a transition period, somewhere between the initial 'crisis response' and an established future state (see Fig 1).

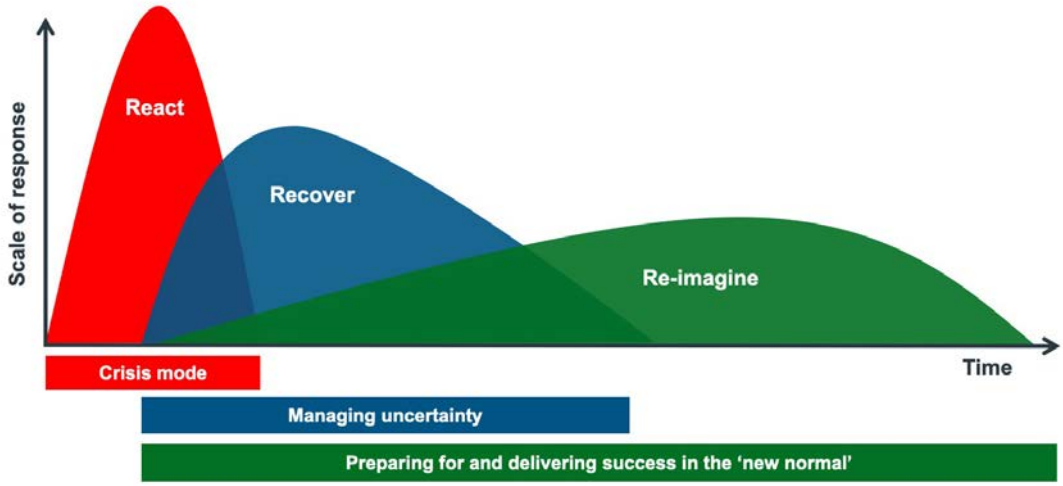


Fig 1. Adapted from Gartner 2020

Managing effectively during this period of uncertainty, both in terms of what is done and the way it is done, will likely set the trajectory for post pandemic performance. So, those companies who for example make a better job of introducing multi-channel HCP interactions in terms of volume delivered and perceived value to the HCP will have an advantage when it comes to deploying these channels in the re-imagined salesforce of the post-pandemic future. They will have the practical experience and customer insights necessary to build successful future strategies. More importantly, successful companies will also pay equal attention to the customer experience, a theme I will return to later.

The current state of promotional activity volumes

Back in October I used data on sales activity collected across multiple countries by IQVIA ChannelDynamics since the start of 2020 to identify three main themes for the impact on direct promotional activity with HCPs (data covers face to face detailing and meetings, postal and email, phone detailing, live and automated e-detailing, live and automated e-meetings). Using data from March 2021 I can update this picture:

1. **Promotional activity volumes** have recovered somewhat compared to a year ago but in most countries are still well below those pre-pandemic (Fig.2). Spain and the US are the only countries yet to see recovery to where they were a year ago, whilst Germany and Japan have actually increased to levels above where they were pre-pandemic. More granular data from the US demonstrates a wide variation across specialities, with oncology particularly lagging behind in total promotional activity.

	% Change in promotional volume March'20 – March'21	Total activity volume compared to pre-pandemic
Brazil	+22%	↓
China	+11%	↓
France	+8%	↓
Germany	+35%	↑
Italy	+26%	↓
Japan	+24%	↑
Mexico	+24%	↓
Spain	-13%	↓
UK	0%	↔
US	-5%	↓

Fig 2. Data source: IQVIA European Thought Leadership; ChannelDynamics 21/03/2021

2. The proportion of **promotional activity conducted** via channels other than face to face has fluctuated across the year to fill some of the gap. In Spain, other channels went from 12% of total activity in January 2020, to 94% in May and back to 62% in March 2021. Italy has seen a larger fluctuation, from 9% to 96% and back to 28% in March 2021. Meanwhile in the UK there has been virtually no change across the last 12 months, with other channels running around 95% of total promotional volume. Interestingly, the two countries showing growth in total promotional volume in March 2021 compared to pre-pandemic levels – Germany and Japan – have both experienced growth in already large non-personal interaction volumes. In Germany use of postal and email has grown to 82% of total promotional activity and in Japan automated e-detailing has risen to 61% of the total (see Fig 3).

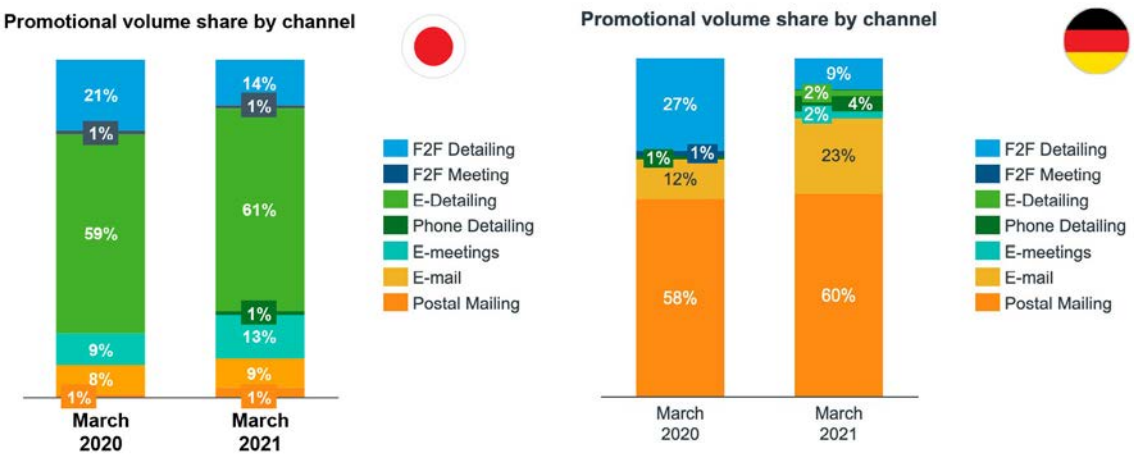


Fig 3. Data source: IQVIA European Thought Leadership; ChannelDynamics 21/03/2021

3. **Face to face activity levels** have continued to recover across most countries, with the notable exception of the UK. The strongest recoveries have been seen in China, Brazil and Italy, with the latter coming close to the activity level pre-pandemic. In the US we continue to see significant variation across specialities with overall activity levels at 52% of their pre-pandemic levels (see Fig 4).

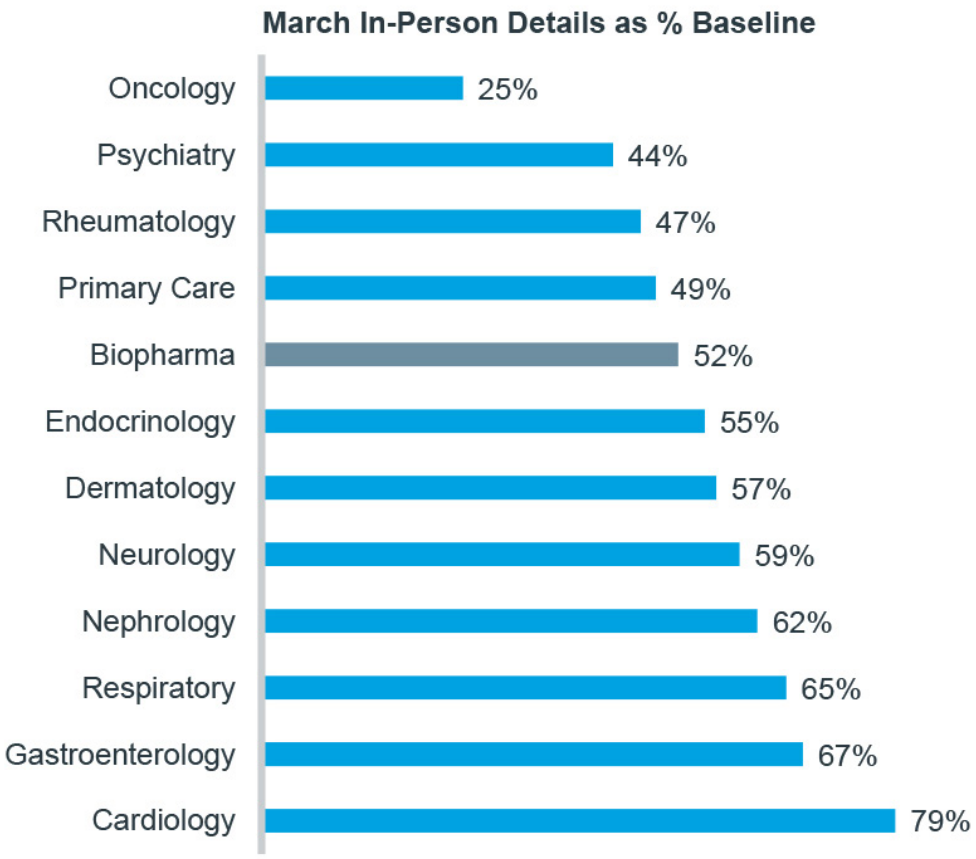


Fig 4. Data source: IQVIA Monitoring the Impact of COVID-19 on the US Pharmaceutical Market April 2021

Alongside this it is worth also taking note of what is happening with the use of remote interactions such as e-detailing, e-meetings and phone detailing. These multichannel engagements grew significantly in response to the pandemic across every country where we capture data and in most cases this increase has remained a feature of the promotional mix even as rates of face to face engagement have recovered. In the US latest data suggests the use of these channels is steady at around 23% of the promotional volume, up from just 2% pre pandemic. This supports the argument I made back in October that the increased use of these channels will likely continue in many countries even as face to face interactions recover, driven by increased experience and comfort with their use by pharma companies and HCPs.

Delivering success in 2021 and beyond

Learning from the experience of 2020 and its impact on healthcare systems around the globe, my colleague Sarah Rickwood has written about nine themes that will drive change in our industry in 2021 and beyond. Two of these are particularly pertinent to discussion of promotional channels and how to successfully practical deployment of resources.

The first is a focus on customer engagement impact. We have consistently seen feedback from HCPs that they have become more favourable to the use of remote engagements by pharma company representatives as their experience of their use has grown. In addition, the increased use of telehealth for patient consultations during the pandemic has further helped drive acceptance of online interactions in healthcare. Recent research conducted by IQVIA in the US demonstrates how perceptions have evolved since the beginning of 2020, when video enabled activity represented less than 1% of total promotional volume. Now, around a third of HCPs view videoconference interaction with pharma representatives as more valuable than face to face meetings (see Fig 5), and most expect their use to remain high due to factors such as convenience and policy changes in hospitals.

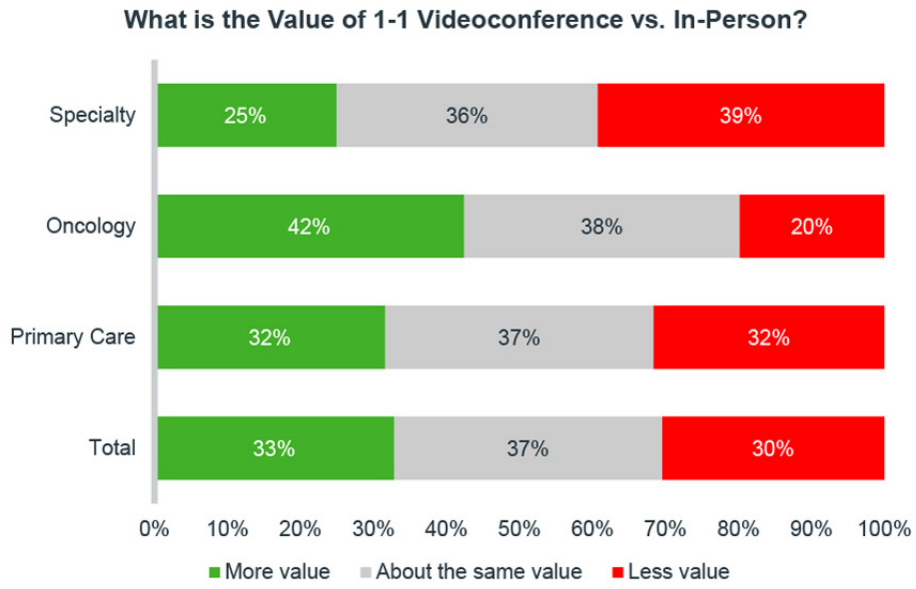


Fig 5. IQVIA Primary Market Research Feb 2021

Shifting to these channels is one thing, but doing it successfully is another. Inadequate subject matter, poor content delivery and a lack of online communications skills are all factors that can significantly affect the customer experience of these interactions. This can particularly be the case with younger and more digitally savvy HCPs who have trained in an online world and who from their personal experience will have high expectations of online delivered content.

So, shifting the focus of promotional activity to alternate channels alongside face to face is an important strategy for the industry but will not be a successful one without careful thought and preparation going into developing the right skills, content and approach to deliver an excellent experience that each HCP wants to repeat.

The second relevant theme concerns the non-COVID patient backlog that healthcare systems around the world will be facing for some time ahead and the associated impact on prescribing dynamics. As an example, in the US it is estimated that over one billion diagnostic visits were lost in 2020 and as many as 300 million will be lost in 20211, contributing to a projected reduction in prescription volumes of over 100 million in the first six months of the year. As well as creating significant opportunities for pharma to be active in helping to address this backlog, through initiatives such as supporting the use of telehealth, facilitating patient pathway changes or increasing the efficiency of diagnostic activity, it almost certainly will add to the access challenge. HCPs are likely to be overstretched and under-resourced in many key specialities, reducing the opportunities for, and likely duration of, promotional interactions.

In this context making effective use of every in-person or digitally enabled interaction with HCPs becomes critical for success. Finding the right combination of channels and skills of your salesforce to deliver the right messages to the right audience whilst ensuring an excellent HCP customer experience will be a significant challenge in increasingly time constrained healthcare systems.

Pay attention to customer experience

As our industry grapples with managing uncertainty whilst re-imagining promotional models in the post pandemic world I believe paying attention to the HCP customer experience is as important, if not more important, as thinking about the practicalities of channel mix.

The term 'Omnichannel' is increasingly used across our industry to describe the type of co-ordinated, integrated set of promotional activities companies will need to move towards in the 'new reality'. Whilst this is undoubtedly true, it is important to recognise the risk of this creating low value noise for HCPs already overburdened with too much to catch-up on in too little time.

Smart use of the channel mix will be the differentiating factor. Paying attention to the changing way that HCPs want to receive information, segmenting approaches to recognise the differing needs of different specialties, of those comfortable using telehealth in everyday practice versus those who are not and making sure face to face visits are optimised for the right objective all become part of that smart approach.

Behind all of this are the most important resource companies have: the skills, experience, and commitment of their people. It is their job to bring the value of every product to patients and their HCPs, to use great communication, technology, data and analytics to bring this value to life in everyday practice. If they are to deliver a great HCP customer experience it is equally important for companies to pay attention to equipping them with the right tools and skills to be successful in this changed environment.

The acceleration in the use of digitally enabled engagement we have seen in the last twelve months has challenged pharma to adapt at speed and with this speed comes risk – a risk that in their haste companies fail to pay attention to the importance of developing their people as much as they develop their methods, something that may ultimately determine success or failure.

1 Source: IQVIA Medical Claims data analysis

About IQVIA

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Learn more at www.iqvia.com.

About the author

John Procter is VP offering development, IQVIA's Contract Sales & Medical Solutions Global Business Unit (CSMS GBU). John leads global strategy and service development for IQVIA's CSMS, covering patient services, medical affairs and contract sales. His expert knowledge in health solutions comes from 30 years in the healthcare industry. He joined Quintiles in October 2010 to run the Patient Services, Medical Affairs and Market Access business in the UK and then moved on to work in global service development and then head of Europe for these businesses. He took up his new role in the GBU global team in January 2018. Prior to joining Quintiles John spent eleven years at Pfizer.

How the patient voice is becoming vital for drug approval

Rebecca Sanders from Lipodystrophy UK tells us how the patient voice helped convince NICE to approve a much-needed drug for this rare disease, and explores how regulators and pharma companies can help make patient involvement in HTA more impactful.

When England's drug reimbursement watchdog NICE was on the fence about approving lipodystrophy treatment metreleptin, it was stories from patients that helped it get over the line and into routine use.

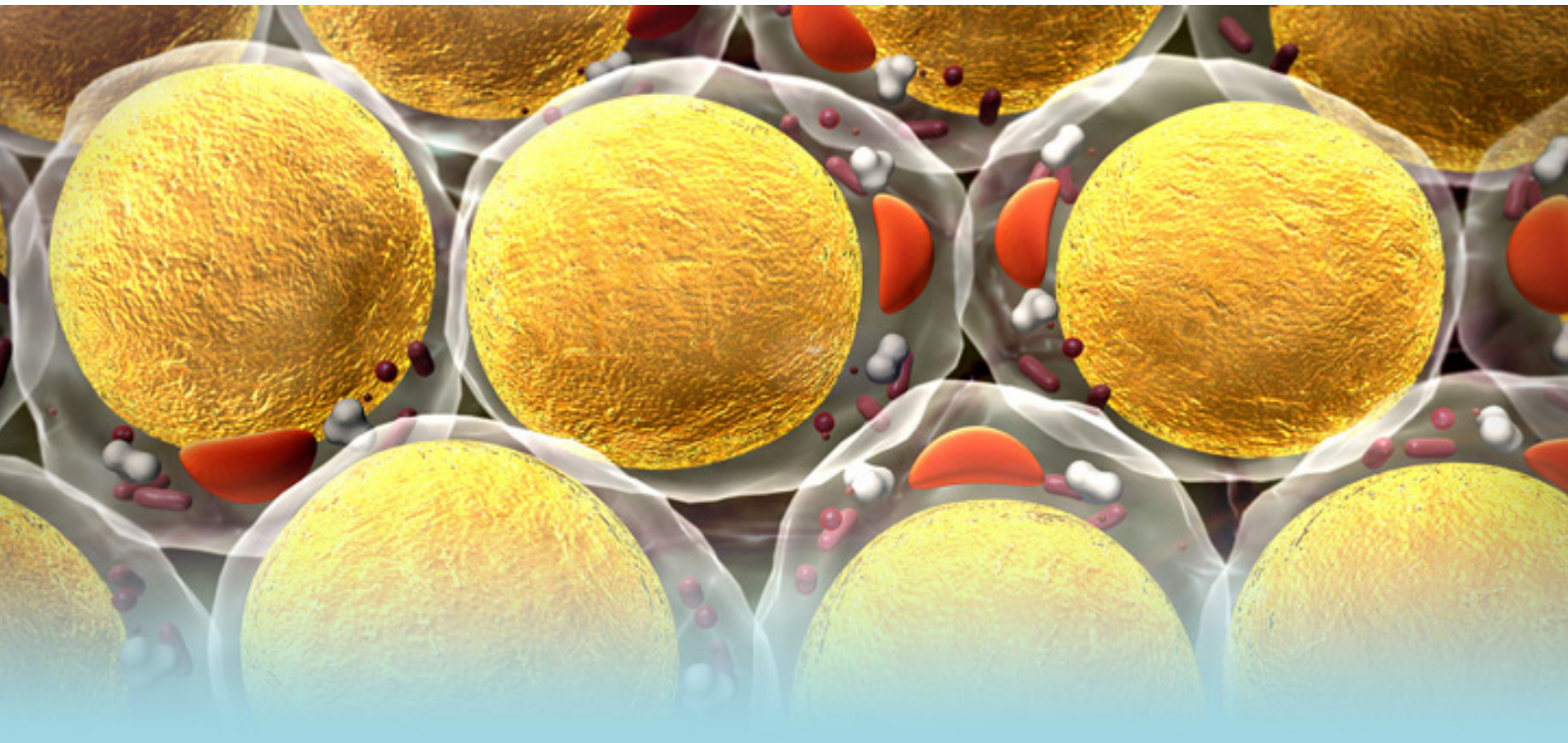
Rebecca Sanders, CEO of Lipodystrophy UK, the charity that spearheaded the patient involvement at NICE's committee meetings for the drug, says that perspectives from people who live with the disease every day were essential for overcoming the shortcomings in the data.

"The numbers are never going to tell the whole story about quality of life," she says. "There are so many other aspects of the condition that impact not just the patient themselves but their extended family, their carers and their children.

"Without the patient voice it's difficult for researchers and regulators to understand what that all means. Stakeholders are well-meaning, but you need the personal touch in order to really get across the gravity of the situation."

A misunderstood condition

This was particularly important for metreleptin because lipodystrophy is a condition with many misunderstood social and mental health impacts on top of its severe medical effects.



Lipodystrophy is a rare disease with two different forms. Generalised lipodystrophy results in a total loss of subcutaneous fat cells. Partial lipodystrophy results in a partial loss, but the fat that remains is redistributed in inappropriate places around the body.

This can lead to metabolic abnormalities including insulin resistance, diabetes, and elevated levels of blood lipids (such as high cholesterol).

"People often say, 'Oh, that's great. You haven't got fat cells, you can't put on weight' – but they don't necessarily understand that fat cells have quite an important metabolic function," says Sanders. "The impact of that lack of fat is actually very similar to the impacts from obesity."

The loss of fat cells also results in the loss of one of the hormones they produce, leptin – which, among other things, controls appetite. This means that lipodystrophy patients can feel hungry all the time, no matter how much they eat.

"Some people don't see that as a big deal, but in practice that means your body constantly thinks you're starving to death," says Sanders. "That causes all kinds of difficulties, especially when the main mode of management for lipodystrophy is diet."

In addition, the loss or redistribution of fat can have major effects on people's appearance.

"With generalised lipodystrophy, patients can often look quite gaunt and undernourished, and with partial lipodystrophy you can either have an unusual redistribution of fat or a lot of excess fat on your face and chin. That makes people feel very body conscious and uncomfortable.

"Partial patients can also have muscular hypertrophy, which results in extra muscle mass. Many patients, particularly female patients, really struggle with that because as a society we tend to associate muscular appearance with masculinity. That can also have severe mental health impacts."

Patients often experience hot flushes, but can be so conscious of their appearance that they will still dress to cover up their body, even in hot weather.

"I know people that moved to the Middle East so that they wouldn't be questioned for wearing trousers and long sleeves in hot weather," says Sanders. "People go to those kinds of extremes because they feel so uncomfortable with themselves."

Building the patient voice

Sanders was diagnosed with lipodystrophy when she was 17.

"The first thing I did was ask the hospital whether there was a support group, but it turned out there wasn't anything," she says. "So I asked if I could write a letter that they would pass on to other patients if they wanted to contact me.

"For a long time, I contacted other patients in that way. More and more, I felt that we needed something organised, so I put together a website with another patient. Through that, we had a forum and we organised meetups. That was really helpful and people got a lot out of that."

The patient community continued to grow from there, with Sanders working with Addenbrooke's Hospital to organise patient support days where people could talk to specialists and other patients.

"That was an opportunity for people to share things that had worked for them, whether that be medical interventions or more mundane things like body moisturisers that had worked for their skin."

Eventually Sanders felt she would be able to do more with a formal organisation.

"In 2016 I put my ideas together, got a group of people on board as trustees, then went through the application process to become an official charity in 2017."

This allowed the group, now called Lipodystrophy UK, to have a higher level of organisation, apply for funding for particular support projects, and provide a stronger voice in patient advocacy.

Sanders is a geneticist by training, and says that this has helped her in her interactions with scientists and regulators.

"I am fortunate in that by being a scientist myself I understand a lot of the complexities behind the condition that other people sometimes struggle with. It also means I tend to be taken a bit more seriously as a voice when speaking to people like clinicians, researchers, or pharma companies. It shouldn't be that way, but it is."

Fighting for access

As an official charity Lipodystrophy UK has also been able to act as an expert voice in NICE appraisals for the lipodystrophy treatment metreleptin – where patient insights into the misunderstood aspects of the condition proved invaluable.

Before being approved, metreleptin had gone through two previous owners, and had been rejected for funding on the grounds of inadequate evidence in 2019. Eventually it was bought by Amryt who, keen to get the drug licensed, resubmitted it to NICE in 2020.

"One of the difficulties in getting licensing was that early on the drug had been given to patients on a named-patient basis and for compassionate reasons, rather than as a formal clinical trial," says Sanders, noting that this issue is not uncommon in rare diseases.

"Unfortunately, that resulted in not having the organised, rigorous dataset that is usually required for NICE submission."

Sanders has been on metreleptin treatment herself for over 10 years, and from the start has been involved in fighting for access to the drug, engaging with the pharma companies, clinical researchers, and NICE.

"NICE is good at encouraging conversations with patient stakeholders in these types of meetings, and we've been able to speak as a charity at all of the appraisal meetings for the drug. They've also made sure to reach out to patients who are not trustees of the charity," she says.

NICE gave the patient community several opportunities to submit evidence.

As part of this, Lipodystrophy UK did a survey of the community and presented the data to the committee, while also highlighting individual statements and quotes.

Sanders says that these personal stories gave NICE a chance to understand the real impact of the treatment even when, on the surface, the data didn't seem to show much efficacy.

"They definitely helped get the human story across. It's so easy to concentrate on the numbers, the money, and the facts and figures, but that doesn't tell you anything about the people and how a disease actually impacts them on a day-to-day basis.

"For example, if you say to someone you're hungry all the time they often don't understand what that really means because most people, certainly in the western world, have no idea what it's like to feel truly hungry.

"Throughout the process we were involved with NICE and all the other stakeholders to make sure that those aspects were not forgotten about."

More communication, earlier

These patient testimonials helped push metreleptin over the line into approval, and the drug was finally approved for routine use on the NHS in January 2021.

There are plenty of rare disease drugs that aren't so lucky, though, and Sanders says that in order for other treatments to avoid metreleptin's early reimbursement difficulties more communication between every stakeholder at every stage is vital.

In particular, she says, many of metreleptin's initial struggles came down to a lack of communication with patients from the earliest stages of development, which led to the wrong kinds of data being collected.

"The earlier HTAs, researchers and pharma companies can start that engagement the better.

"For example, the first committee meeting for metreleptin didn't touch on how important the hunger aspect was, because nobody had that discussion with us in advance.

"After that meeting, we were able to tell the company how important it is to explain why metreleptin helps with that aspect, and how that impacts quality of life – but of course it would have been best to have that discussion upfront."

And in the case of HTA bodies in particular, early communication means patient groups can be in the best position possible to share the right information with them.

"We were fortunate in that sense because our particular case went on for several committee meetings and we had opportunities to resubmit information, but in cases where there's only one meeting patients might only have one chance to get it right."

This means making sure patient advocates are clear on what opportunities for submission exist, and what kinds of information would be useful for the committee.

"For example, are they interested in what carers think? Do they want to assess quality of life? It's important right from the beginning to have clarity on that, so that the focus of the patient submissions is right and the HTA is getting what they need. Then the patients feel like they've done justice for everybody."

And on a more mundane note, regulators should make sure that meetings themselves are accessible to patients no matter their needs.

"Not everybody will be able to travel across the country to attend a meeting, for example," says Sanders. "For me that means I would have to take the day off work and deal with the fatigue that comes with travel. COVID has shown everybody that there's more we can do remotely to resolve that, and NICE has been good about recognising how important that is, but of course there's always more that can be done."

Going even further back in the process, there can also be benefits to reimbursement outcomes further down the line if the patient voice is taken into account at the start of drug development.

"It's really important for pharmaceutical companies to engage with advocate communities when planning studies or deciding to provide a drug on a compassionate use basis, so that they know they're capturing the right kind of data," says Sanders.

"Again, the kind of data that clinicians are usually interested in, while very important and necessary, doesn't always capture the aspects that matter to patients, and one of the big problems with metreleptin was that the data wasn't collected properly at the beginning."

Sanders' work is not done now that metreleptin has approval in England, and Lipodystrophy UK are also working with clinicians in Scotland to see if a similar appraisal process for the drug can be applied there.

On top of that, the charity is providing guidance to other patient groups around the world, advising them on what they need to consider when getting involved in HTA processes.

"We're also working to develop a global collaboration of patient groups for lipodystrophy," she says. "When you've got more people behind you, the patient voice can carry more weight, and that's especially important in a rare disease.

"There are obviously variations in culture and drug assessment processes in different countries, but at the end of the day patients are suffering from the same conditions and want the same things."

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

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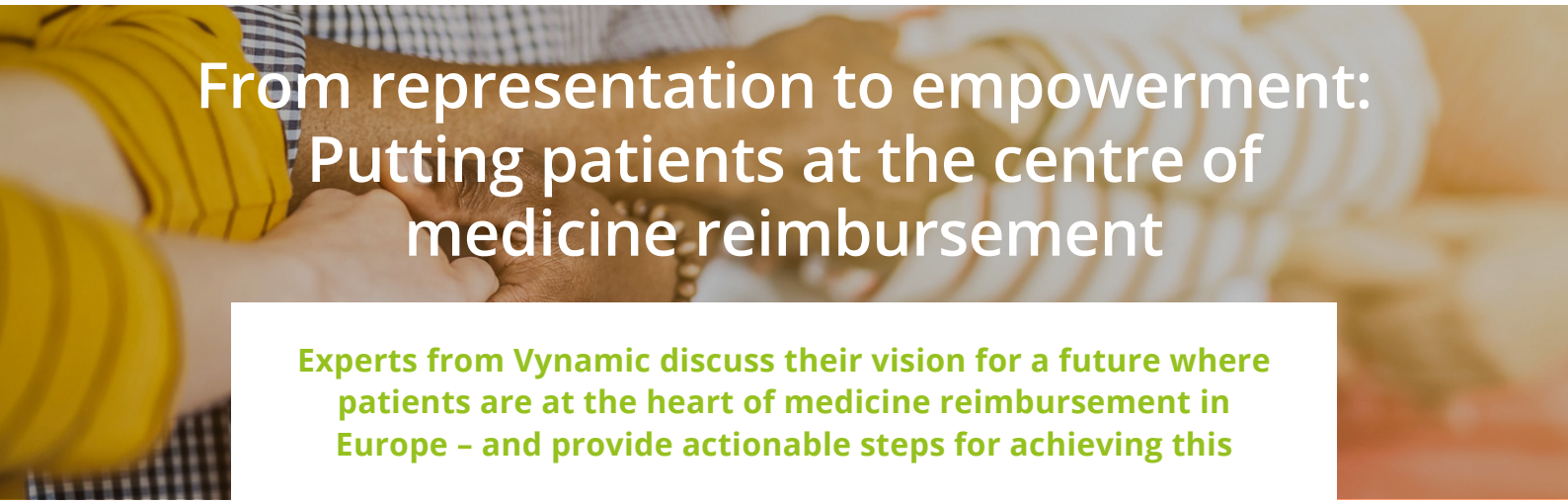


From representation to empowerment: Putting patients at the centre of medicine reimbursement

Experts from Vynamic discuss their vision for a future where patients are at the heart of medicine reimbursement in Europe – and provide actionable steps for achieving this

The reimbursement landscape in Europe is complex. There are different models, medicines are evaluated in different ways and the timelines for approval vary widely. Most crucially, the level of patient involvement is not where it needs to be. This simply has to change.

Patients provide the human face for evidence – identifying outcomes that are important to them, addressing gaps in the clinical evidence base, helping verify or refute assumptions in economic models and informing the determination of added value.

Vynamic's vision for the future is one that places the patient at the heart of the reimbursement process across the full value chain – but to achieve that vision, all stakeholders need to advocate for major changes across the entire sector, and there are many implications to consider before embarking on such a journey.

Current level of patient engagement in local Health Technology Assessments (HTAs) across Europe

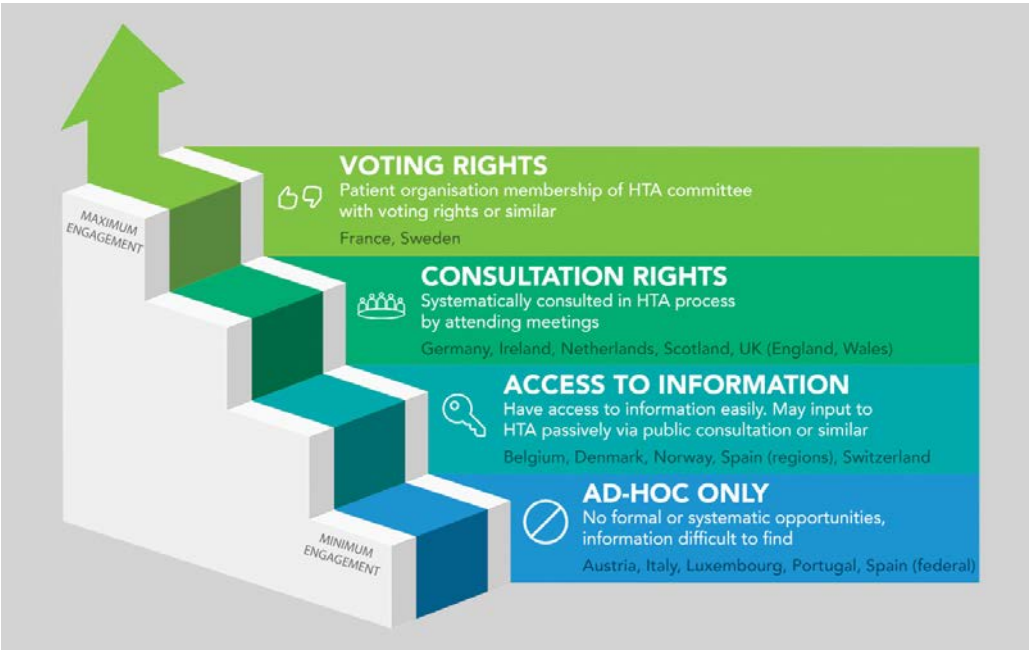


Figure 1: Maturity model of patient and Patient Organisation (PO) engagement in Europe (list of countries not exhaustive)

The degree to which patients and patient organisations (POs) can be involved in the HTA review process varies across Europe, and there are as many situations as there are countries (see figure 1 for a high-level overview). Patients and POs contribute to discussions by highlighting, for example, their experience of the disease, their needs and what risks they would consider acceptable in view of the expected or potential clinical benefits.

Some countries like France or Sweden have a formalised process where patients and POs can vote within HTA committees. In contrast, other European countries like Austria, Italy or Portugal do not usually share HTA information nor consult patient representatives. Following the appraisal of a medicine, in some instances patients and POs have the right to appeal or provide input at re-evaluations. For a summary of the key takeaways on patient and PO involvement in local HTAs see figure 2.

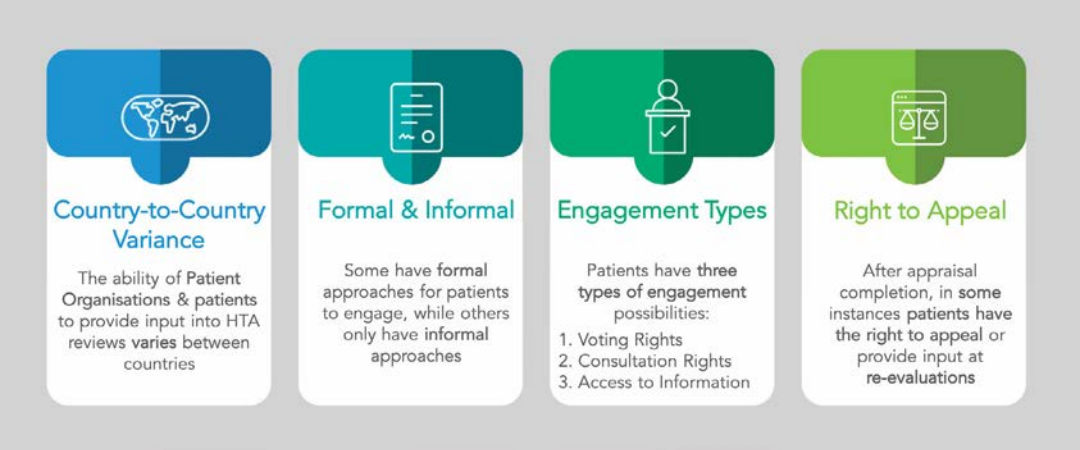


Figure 2: Key takeaways on patient and PO involvement in HTAs

The European Medicines Agency (EMA) offers consultations in parallel with the European Network for Health Technology Assessment (EUnetHTA) to harness synergies between regulatory evaluation and HTA. This collaboration currently focuses on evidence-generation plans and patients and POs are regularly invited to share their real-life perspectives and experiences in relation to a particular medicine in their disease area.

In 2019, simultaneous advice from EMA and HTA bodies was provided upon request during the development of 27 medicines. Patients were involved in two thirds of these scientific advice procedures and provided added value in almost every case; sometimes leading to a modification of the development plan to reflect patient inputs.

Our vision for the future has many implications for stakeholders

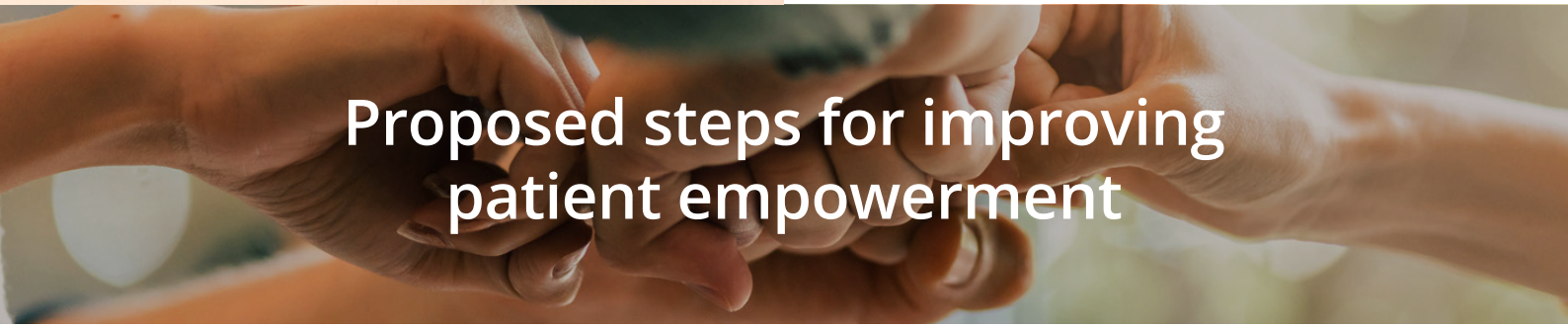
Progress is being made and there is a strong foundation to build upon. However, much more needs to, and can, be done.

Underlying our vision is the concept of augmenting patient empowerment through the lens of 'No decision about me, without me'. We want to see a future where patients and POs are at the centre of medicine development and reimbursement decisions, as they represent real-world patient perspectives and needs. This will help close the gap between "hypothesis" and "reality" when it comes to improving disease outcomes and enhancing health and lives.

Adopting this has many implications. For example, payers, regulators and policymakers will face the challenge of designing decision-making processes that are fair and equitable across all patient representatives and organisations. Negotiations with industry will become more complex and potential budget impacts will need to be balanced carefully.

The life sciences industry will be presented with opportunities for faster access and unique reimbursement models. They will need to adapt their culture and clinical development and commercialisation models to engage with patients and POs much earlier and seek clarity on where decision-making authority sits across patients and payers.

Meanwhile, healthcare providers will need to engage with well-informed patients and POs who may be lobbying for specific treatments. Providers can leverage this increased awareness to enable more patient-to-patient communication, which in turn could lead to improved compliance.



Proposed steps for improving patient empowerment

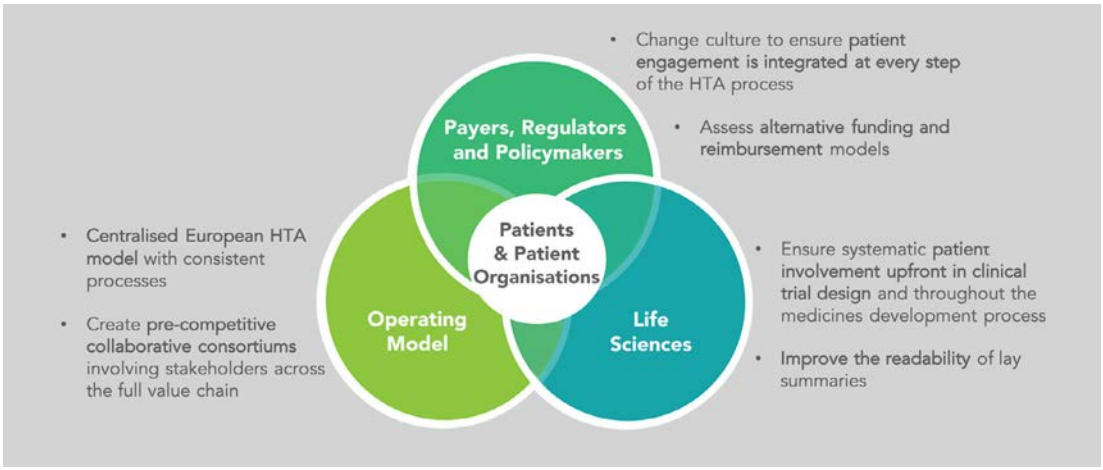


Figure 3: Proposed steps for improving patient empowerment

There are many steps that can be taken to improve patient empowerment. To achieve this, stakeholders will need to rethink every aspect of medicine development and HTA.

All stakeholders should look to establish and adopt a centralised European HTA operating model, building on the cross-border HTA collaboration led by EUnetHTA. This will help drive consistent processes, effective assessments and enable early access to high-value medicines. It will also be important to create pre-competitive collaborative consortiums involving stakeholders across the full value chain, so that they can share lessons learnt and establish best practices.

Meanwhile, payers, regulators and policymakers need to advocate for changes in culture that ensure patient engagement is integrated at every step of the HTA process – as well as assessing alternative funding and reimbursement models to continue to improve patient access to licensed treatments.

Finally, the life sciences industry needs to be ensuring systematic patient involvement upfront in market and disease assessments, clinical trial design and throughout the medicines development process. To facilitate this, readability of lay summaries needs improvement to maximise the value of information exchange and educate POs. This will empower them to provide greater input and influence over HTA decisions.

Most critically, patients and POs should also be looking to engage with these stakeholders as often as they can to advocate and lobby for these changes.

There is a long road ahead for implementing such wide-reaching changes, but the result will be well worth the journey – with better access to treatment, improved ways of working and, ultimately, better patient outcomes.

Contact Us

Vynamic believes there are three critical areas that life sciences organisations should consider as they evolve their patient focus. These are: Define Strategy, Leverage Digital and Measure Impact. Please contact us to learn more about how we might be able to support you in placing the patient at the heart of your business.

About Vynamic

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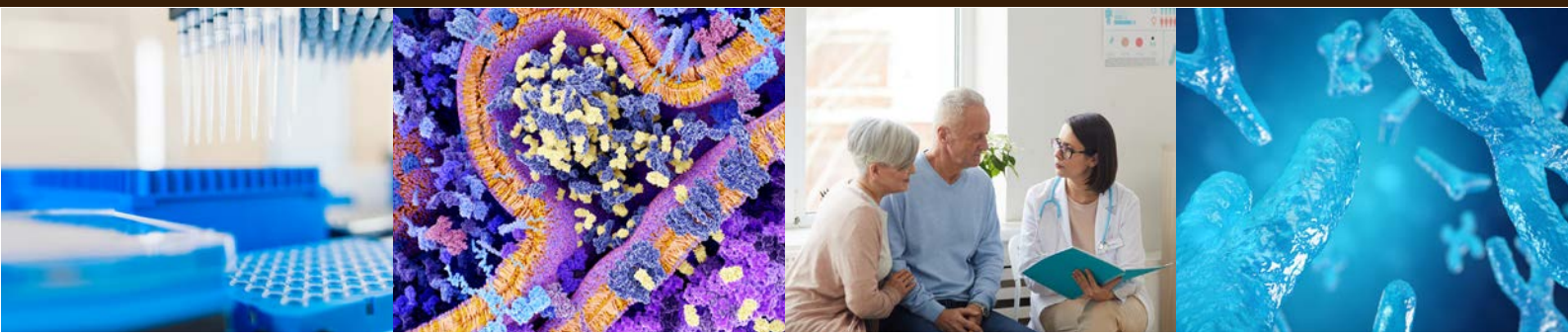


Rethinking access barriers to innovation

Market access issues don't stop once a product has passed HTA – and this is especially true for disruptive therapies like ATMPs. Experts from Executive Insight discuss how a holistic, multi-stakeholder approach can help companies overcome access barriers at all stages.

The last decade has seen some remarkable advances in medicine, with innovative new products like cell and gene therapies showing efficacy in diseases long thought almost untreatable.

But launching a disruptive product can be a double-edged sword. While the potential patient benefits are huge, these therapies face difficulties in passing health technology assessment (HTA) processes designed for more standard products, and once approved they face other barriers from putting new pressures on health systems.

Michalina Jenkins, who has assessed HTA systems in terms of associated barriers in a number of different countries in her role as a senior consultant at Executive Insight, says that one of the most common HTA barriers companies face is a lack of broad value recognition for innovative products.

"When considering the value of innovation, we know that it should drive benefits to patients – but also, broadly speaking, it should provide cost savings to the healthcare system and improve the wellbeing of society in general," she says.

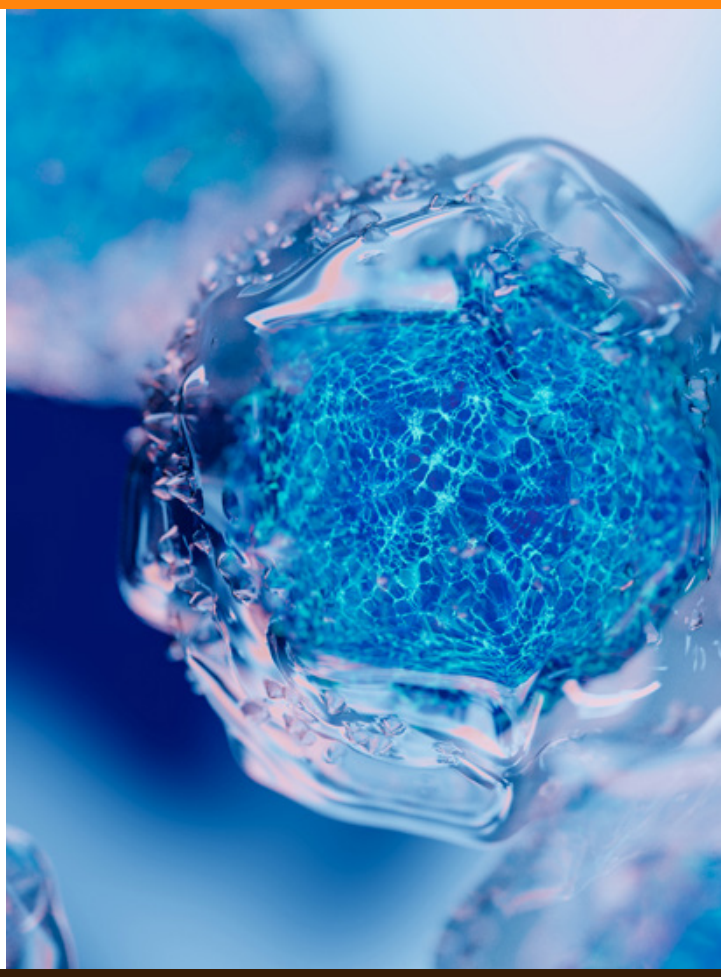
"However, that broader perception of value is often absent in HTA frameworks."

Likewise, she says that HTA processes can lack meaningful contribution from a wide range of stakeholders – especially in countries with newer systems.

A third barrier is that HTA processes, in terms of steps, timelines and assessment criteria, are often still not fully predictable. "This is an issue not only for patients, but also for healthcare providers, and we see it more commonly in countries with younger or emerging HTA systems.

"Meanwhile, access decisions are not always optimal in terms of their timeliness and the breadth of the funding. Often only limited populations of patients can benefit from an innovation when it is approved at the HTA level."

But Jenkins says it's important to remember that market access challenges don't vanish once a drug has been approved. After a product has launched there are often additional systemic barriers beyond the product level to overcome – and this is particularly true for advanced therapy medicinal products (ATMPs).



In the post-launch environment, the effects of not having a broad recognition of value become even sharper, says Luca Lorenzi, manager at Executive Insight.

He notes that, by their nature, these innovative products will often enter an environment that is not ready to support their access and adoption.

"Most importantly, there might be a lack of sufficient funding mechanisms available at launch – and if there is a high burden for healthcare professionals to obtain funding, that will be a major hurdle for adoption of the technology.

"It's important to look at the environment not only from a product angle, but also from an associated services angle – e.g. will the technology cause extra costs because of new procedure, diagnostics or administrative needs that might not receive sufficient funding at launch?"

Additionally, companies may find that there are no optimal care pathways to allow optimal integration of the innovation into the healthcare system.

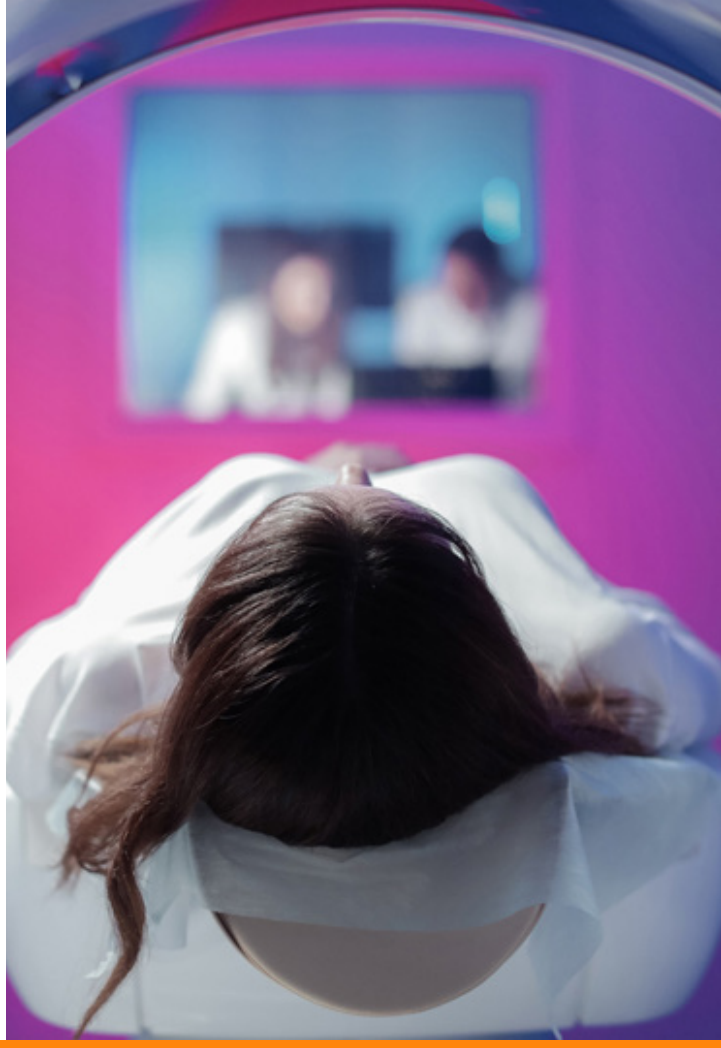
"This all comes down to whether the existing care pathways or process infrastructures are fit for purpose for your innovation, and whether your innovation has a specific complexity in the way it's delivered," says Lorenzi.

"This includes factors like the infrastructure and capacity of treatment sites. We saw with CAR-Ts, for example, that some healthcare systems intentionally limited the number of sites that could deliver this technology to better control its usage."

Similarly, a particularly disruptive product may require behaviour change from HCPs and patients, which can require additional time and cost investment.

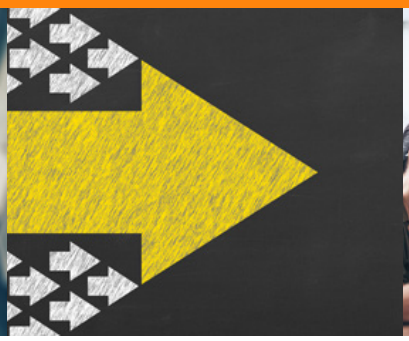
Lorenzi adds that successfully navigating these behaviour challenges again comes down to the broader clinical and economic value proposition of a product.

But often, he says, there can be a lack of awareness or belief in an innovative therapy's value from the healthcare system.



"Initially you might encounter a lack of awareness and understanding of the new therapy and where it fits in the therapeutic landscape. There could also be safety concerns from patients regarding completely new mechanisms of action."






Early planning for access

With such a wide range of factors to consider across the entire access landscape, pharma needs to start planning for potential barriers as early as possible in development – and that means working with key stakeholders systematically and repeatedly to shape the environment before launch.

"The environment won't evolve by itself – and if it does, it will evolve slower than you expect – so pharma needs to act as a catalyst," says Philippe Coune, director at Executive Insight.

"We've seen suboptimal launches of high-profile products that were clearly linked to the fact that the company did not consider potential barriers at an early stage.

"Companies who are specialists in innovative therapies are often okay because they're looking at the world through the prism of their innovation, but that mindset isn't ingrained in big pharma companies who usually work with more standard products."

Mary Rose Ropner, senior consultant at Executive Insight, adds that the process of environment shaping for a particular product can start as early as three to five years before it reaches the market, and can extend beyond launch.

"To start with, the companies can and should comprehensively assess any access barriers their products may face, both at the HTA and post-HTA level."

Jenkins says this begins with bringing together different internal functions within a company.

"Traditionally it was the access team that led a pharma company's efforts to address HTA barriers. But if you want to address issues on a more systemic level, you also need to bring in public affairs, patient affairs, as well as commercial teams.

"It really has to be a collaborative, multi-stakeholder effort on the internal side."

From there, Ropner recommends companies create a broad company position on what they see as the ideal access environment.

"That way, when different countries are trying to shape systems, they will all go in the same direction."

Stakeholder engagement

Companies then need to sit down with a broad group of stakeholders who share common needs and also want to be actors in this change.

All parties can then work together to identify and prioritise key barriers at all levels, and shape the access environment into that ideal position.

"That means you need to identify those stakeholders that have influence over shaping the system, and that may differ from country to country," says Jenkins.

"There are some regions or countries where pharma companies can create strong alliances within trade associations to tackle these barriers together, for example CAEME in Argentina, PhRMA in the US, or EFPIA in Europe. Meanwhile, there are other countries where policymakers are very keen to directly collaborate in shaping HTA systems."

Particularly in countries with emerging HTA environments, doing this means that pharma companies can position themselves as key partners in co-creating such systems, bringing the expertise they have developed in other markets.

"Saudi Arabia is a great example of a country where pharma companies were able to step in and shape an emerging HTA environment," says Jenkins. "The process was initiated by stakeholders building the system. They invited various representatives of access functions from pharma companies to roundtable discussions on what an ideal HTA system should look like, what the key challenges might be, and how they could make sure that the process is predictable from the outset."

Coune says that pharma will often need to widen the scope of the stakeholders it speaks to, so that they match the scope of the challenges a product is likely to face.

"That means going way beyond the usual payer/prescriber stakeholders and looking at all the actors in the system. If you're only interacting with the prescribers and the payers, you are going to miss out on the local level economic complexities associated with your product.

"This is also important because, with the complexities and novelty of some of these therapies, pharma will never be able to address everything themselves. It needs to involve the people who will be able to drive parts of the initiative independently."

Lorenzi adds that it's important to apply the patient perspective throughout all of these discussions.

"A clear advantage of doing that is it allows you to engage stakeholders more effectively. If you assume a patient perspective rather than a product perspective, you are talking to them in their language. That means attempts to shape the environment will be more successful and have more visibility."



Three categories

When it comes to preparing for post-launch barriers with other stakeholders, Ropner says companies need to look for problems across three specific categories.

"First of all, you need to be looking at whether the disease area is actually a priority for different stakeholders, particularly policymakers and payers. More barriers are likely to arise if it is not.

"Secondly, you want to look at the care pathways that are already established, as well as those that are not yet established, and ask how your patient will travel through the healthcare system. If it's a new treatment modality that requires a different administration or manufacturing process, that might impact how patients will receive their care."

US-based Spark Therapeutics, for example, solved this by creating a portal to help patients locate a specialist who can help with genetic testing and assess eligibility for its gene therapy Luxturna.

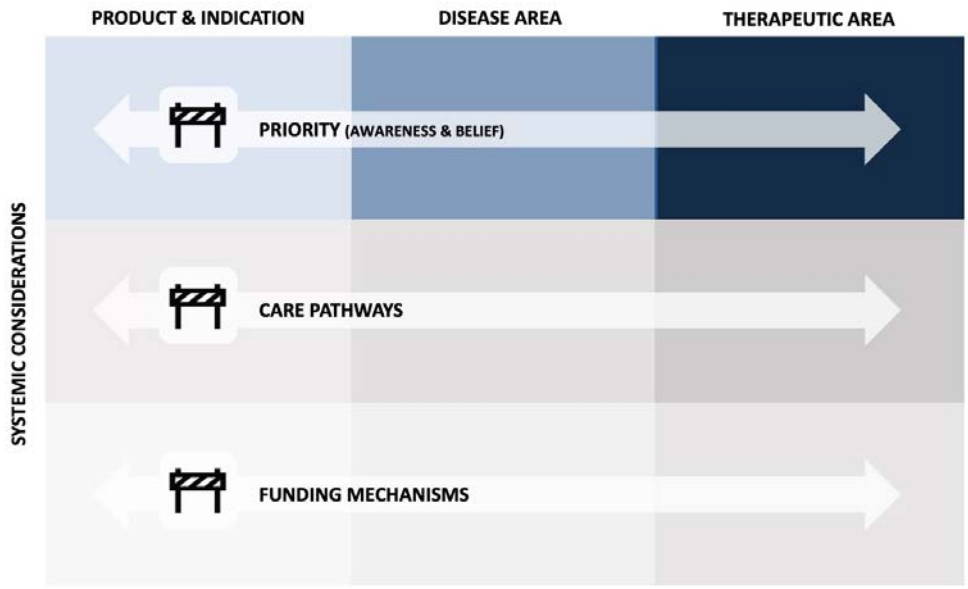
Ropner says that the final category to look at is funding mechanisms.

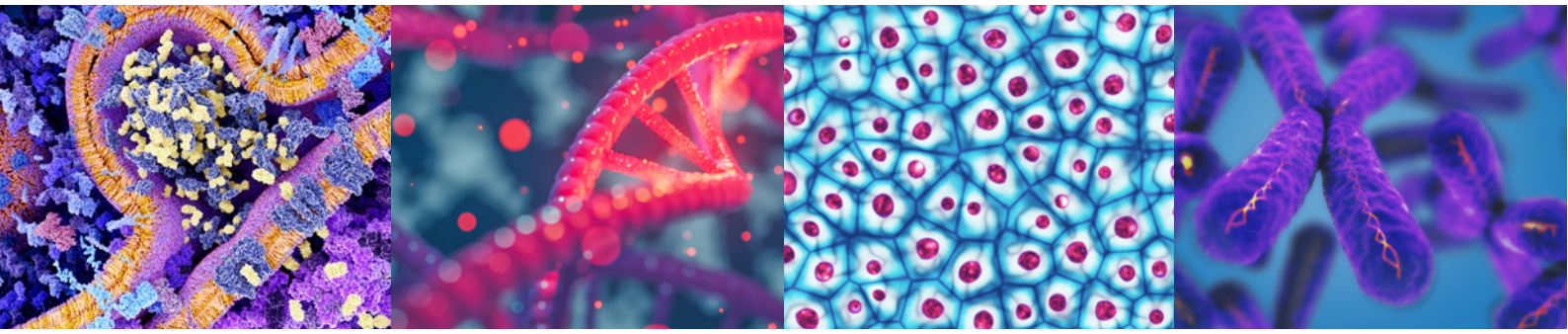
"Those are obviously important at the HTA level, but there might also be post-HTA economic issues – such as whether patients can afford to travel to receive treatment, or whether hospitals can afford wrap-around care and the costs of additional staff, adjuvant therapies etc."

She adds that it's important to consider barriers not just at a product level but also at an indication and disease area level.

"For example, if you have a product in diffuse large B-cell lymphoma (DLBCL) you might want to also look at access issues in lymphoma more generally.

"You essentially need to take a three-by-three matrix view. That means considering disease perceived priority, care pathways, and funding mechanisms, and looking at those three buckets at the product, indication, and disease and therapeutic area level."





Access for CAR-Ts

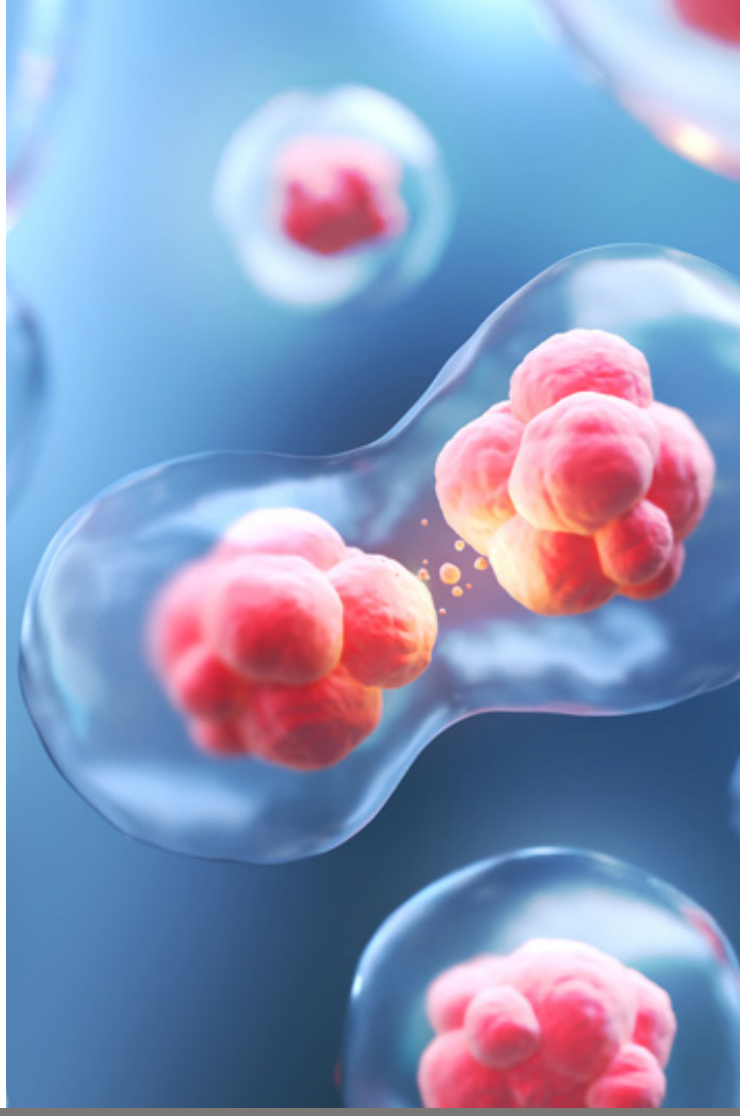
Ropner points out that there are many examples of these strategies paying off for companies – particularly in CAR-T therapies for cancer, which have been among the most anticipated ATMPs to launch over the last five years.

"The markets where CAR-Ts have seen more success are often those where HTA organisations engaged early with companies and healthcare systems and ensured that the system was ready to assess and approve these therapies," she says.

Meanwhile, CAR-T manufacturers across the world had to take broad, multi-stakeholder approaches when reacting to access barriers in different healthcare systems.

In the US, companies addressed financial and patient access barriers by providing a patient support system that covers transportation and hotel costs for CAR-T patients who need to travel to treatment centres. In Germany manufacturers had to devise ways to compensate hospitals for the costs associated with the apheresis required for CAR-T treatment, as the procedure has been excluded from funding by the G-BA.

To address administrative and care pathway burdens in the US and Japan, some of these companies have also provided an online system to help referring haemato-oncologists locate CAR-T treating centres that will assess the potential eligibility of their patients.



"There was a fear at the start that these new therapies would break certain healthcare systems," says Lorenzi. "But by companies engaging stakeholders pre-launch, communicating in a transparent way and helping run assessments, these systems were able to think about what changes were needed."

The overall message, then, is clear: a holistic approach to value and access is needed to drive sustainable adoption of innovation.

That means comprehensively assessing barriers at the product, therapy area, and system level – starting as early as possible – whilst also defining what an ideal environment should look like. To do all this, companies need to identify key stakeholders along the patient journey and build alliances so that together they can co-create solutions to address common needs.

From there, companies need to regularly reassess their approaches and develop corresponding shaping strategies.

"Stakeholders are usually keen to find solutions and build these structures upfront," says Lorenzi, "because in the end it's for the overall good of the patient, healthcare systems and society.

"This can only be a win-win situation for pharma, because ultimately it creates fertile ground for adoption while helping patients."

About the interviewees

Philippe Coune, PhD, is a director at Executive Insight. Phillipe has a background in market access and strategic pricing, with a focus on rare diseases and advanced therapies. His experience includes the assessment of funding options for cell therapies, as well as the development of market access strategies for innovative therapies in the orphan space.

Luca Lorenzi, PhD, is a manager at Executive Insight. He specialises in market access and healthcare policy with a focus on the intricacies of advanced therapies and specialty products. In his role he supports major biopharmaceutical companies in developing access and environment shaping strategies to successfully commercialise their innovations.

Mary Rose Ropner is a senior consultant at Executive Insight, where she specializes in developing pricing and market access strategies for early products, including for oncology therapies. She has provided consulting, market research and competitive intelligence services to major pharmaceutical companies for over five years and continues to support the development of early market access shaping strategies for different products within her current role.

Michalina Jenkins, PhD, is a senior consultant at Executive Insight. She is passionate about driving optimal patient access to innovative medicines. Her experience includes development of market access strategies, including access environment shaping and advocacy, for a variety of assets at different stages of market readiness.

About Executive Insight

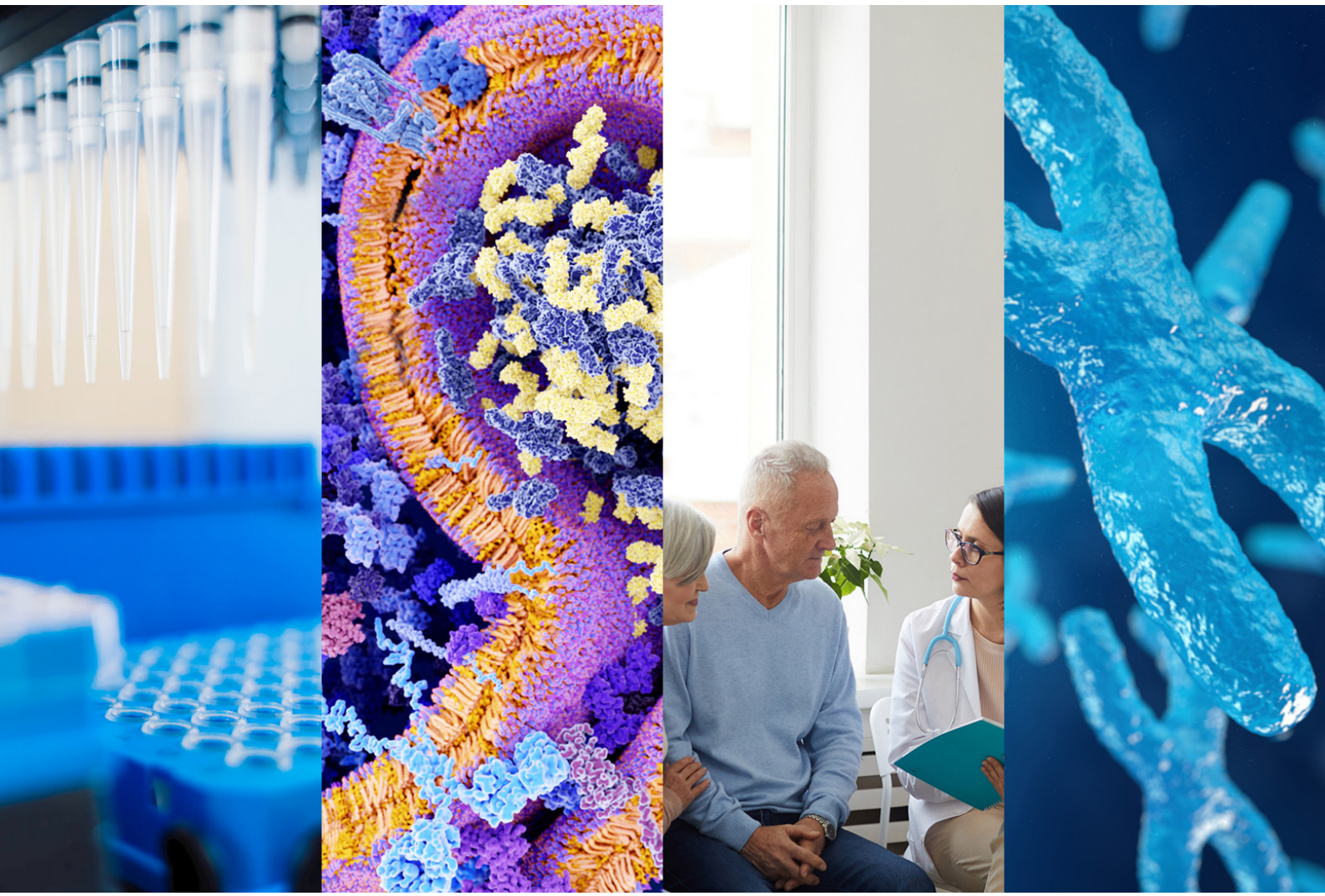
executive insight
HEALTHCARE CONSULTANTS

Executive Insight is a specialised healthcare consulting firm supporting biopharmaceutical companies in successfully preparing, launching and commercialising their products. The company was founded in 2000 by a group of industry professionals who recognized the need for specialised healthcare consulting. Today, Executive Insight proudly works for six of the top ten global pharma companies and has about 60 employees located throughout Europe and beyond. The head office is located in Switzerland with a subsidiary in London, UK.

You can contact the company on LinkedIn.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.



How to improve payer engagement with tailored value communication

Pharmaceutical drug success rests on being able to achieve market access. Brands are facing increasing pressure to demonstrate strong value propositions in increasingly crowded therapeutic areas. However, value can mean different things to different payers. In this article, Cecilie Alstad, senior analyst at Research Partnership, discusses how an approach established in the world of marketing and commercial strategy could be the key to significantly improving pharma's payer engagement.

Traditional payer archetyping is no longer enough

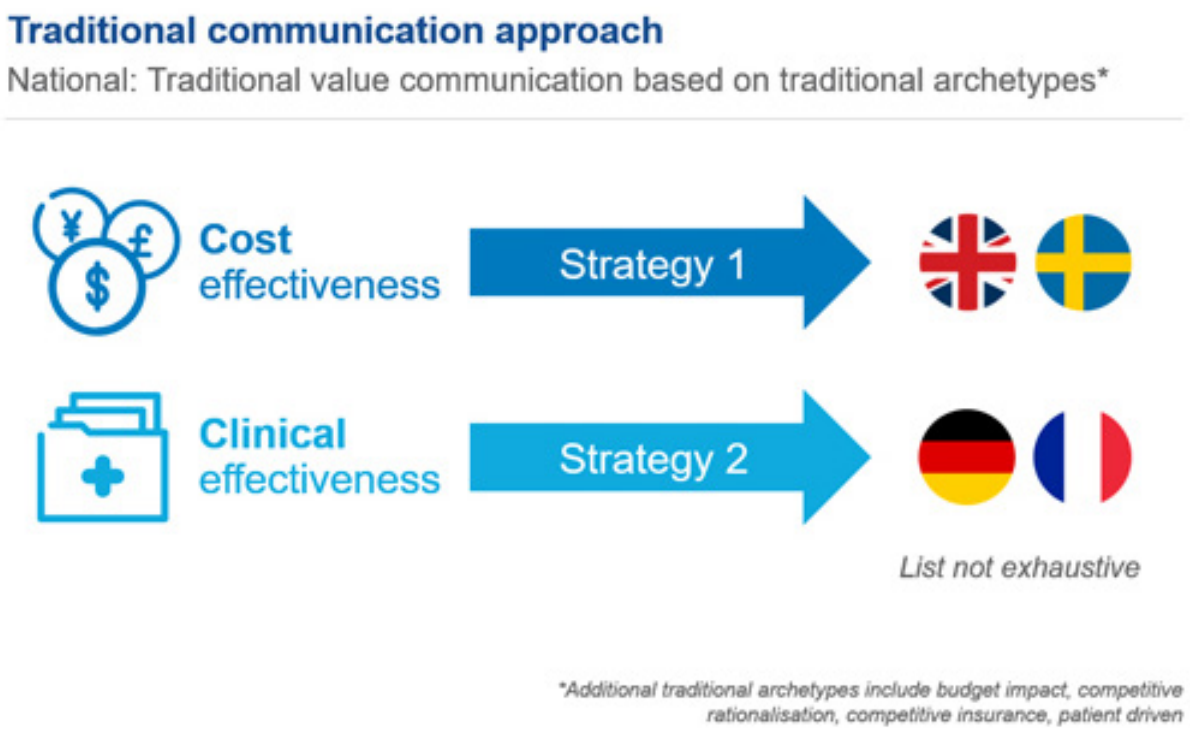
Evidence of a product's value is today's currency for gaining market access and value must be demonstrated to a wide audience including payers, healthcare professionals (HCPs) and patients.

While clinical trials are often designed with regulators in mind, and the efficacy and safety data they generate may be sufficient to obtain regulatory approval, this data alone is rarely sufficient to convince payers it is a product worth investing in. Payers do look for clinical arguments focused on efficacy and safety, similar to regulators. However, the relative importance of and preference for certain clinical endpoints and need for additional evidence, such as indirect comparisons, humanistic, societal and health-economic arguments, varies between different payers.

Variations in evidence requirements can be seen both across markets and HTA bodies, and between national and subnational payers. Their evidence requirements rarely align to those generated by the clinical trial itself, with choice of comparator and even primary endpoints subject to scrutiny.

Preference for certain evidence also evolves, which is the case with quality of life (QoL) data and other patient reported outcomes (PROs). We see variation across payer channels and therapeutic areas in terms of how payers are adapting to patient-centred data and the weight they give to QoL and PROs during decision-making.

To aid understanding of national payer management principles and capture differences in what they consider important in their value-assessments, pharma can apply the concept of traditional payer archetyping. Traditional archetypes are particularly useful to tailor health-economic evidence, e.g. is payer decision-making driven mainly by cost-effectiveness like the UK's NICE, therapeutic referencing like Germany's G-BA or budget impact like payers in Spain.



However, sub-national payer principles often deviate from the traditional archetypes. As we see such variation in payer management principles at national level, we can assume even bigger differences at sub-national level, where the quantity of payers and variation in payer roles are much greater.

This calls for a need to establish tools and frameworks that can support pharma in their transition towards value-based discussions with payers, particularly when it comes to access to treatments that are primarily driven by sub-national differentiation and decision-making.

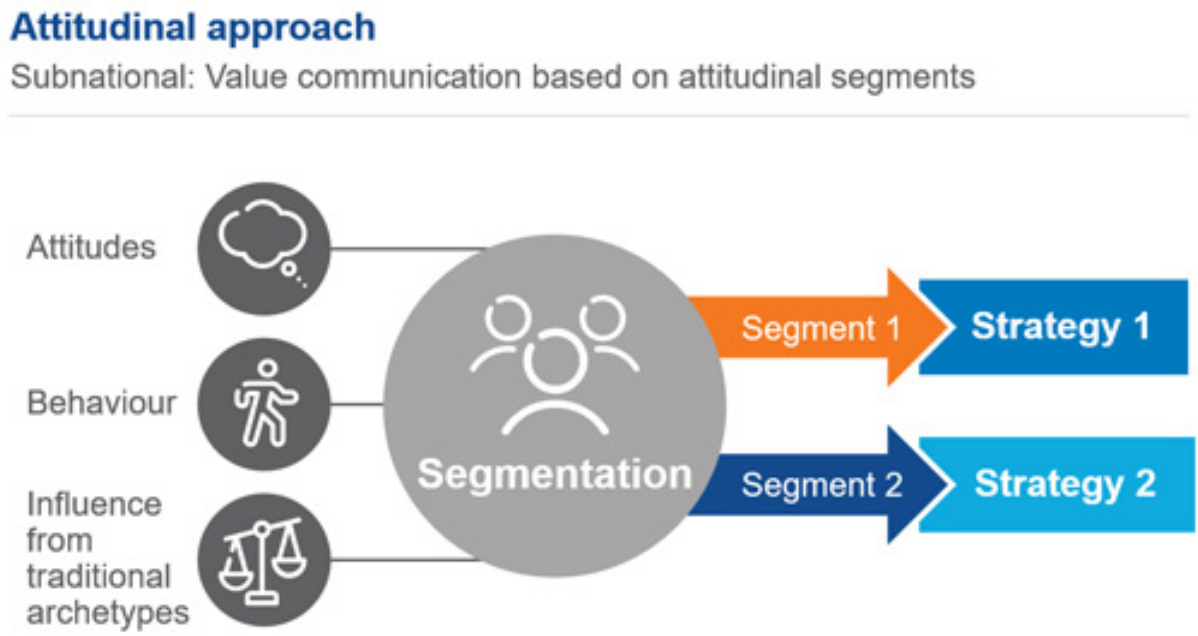
Tailoring the value proposition for more meaningful discussions

Segmentation of HCPs and patients based on their attitudes is already well established in healthcare commercial strategy and marketing. It allows commercial teams to identify inflection points that can be targeted to drive behaviour change during HCP and patient engagement.

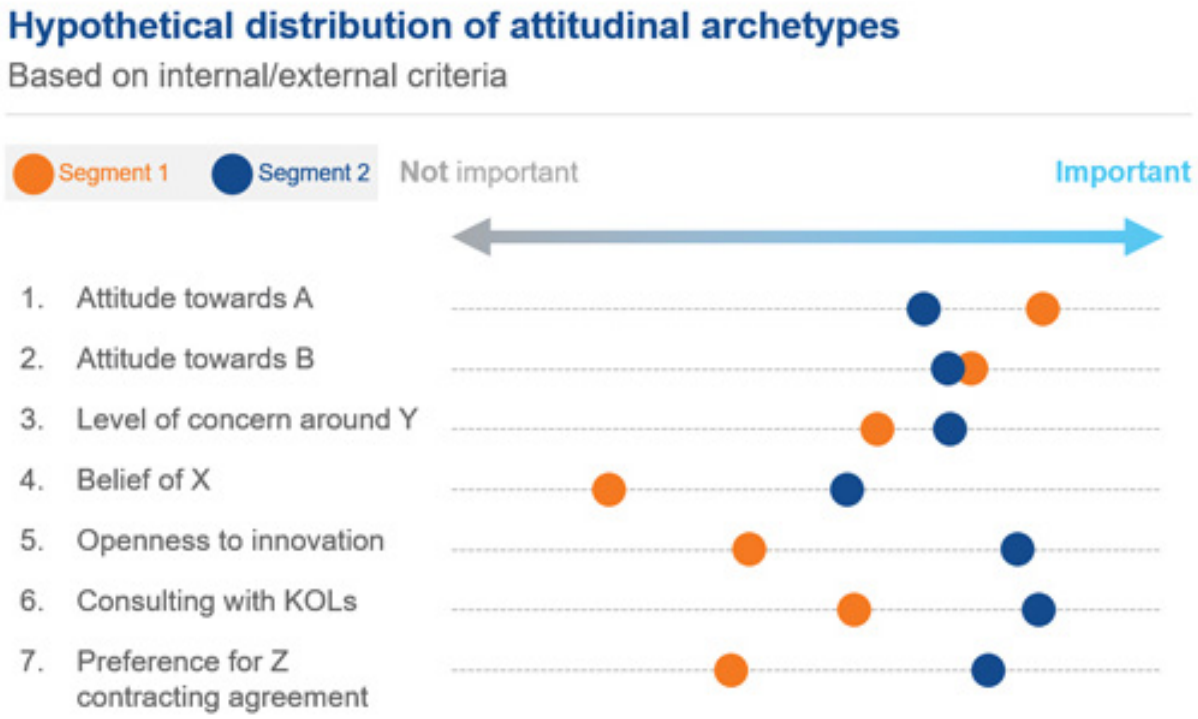
Understanding of education and awareness gaps, and individual's attitudes and needs, is the starting point for building a customer-centric strategy that focuses on optimising customer engagement and enhancing customer experience. Ultimately, it allows the commercial team to prioritise resources while efficiently communicating messages and delivering value added services that are tailored to HCPs' and patients' needs.

Attitudinal segmentation of payers adapts and evolves the approach used by commercial strategy teams to target HCPs. When seeking to understand attitudinal differences between payer segments, it is important to capture both internal and external factors influencing their decision-making, rather than solely considering external factors as in traditional archetyping.

Internal drivers such as payers' beliefs, attitudes and concerns can provide a deeper understanding of their behaviour beyond their organisational roles and responsibilities. For example, how do payers like to be engaged with by pharmaceutical companies, and what are their attitudes towards the industry? Are their concerns relating to new products driven mainly by budget, patient-outcomes or other challenges in their local environment?



In order to capture a holistic view of payer attitudes it is also important to understand how payers prefer to engage with other stakeholders, their openness to innovation and contracting, and where they look for support during decision-making. For example, when do they prefer to engage with pharma, and how willing are they to give access to innovative treatments? Furthermore, do they consult with Key Opinion Leaders, rely on national guidelines, etc. in order to make informed decisions? Additionally, as payers rarely make decisions in isolation, how do they interact with their formulary colleagues?

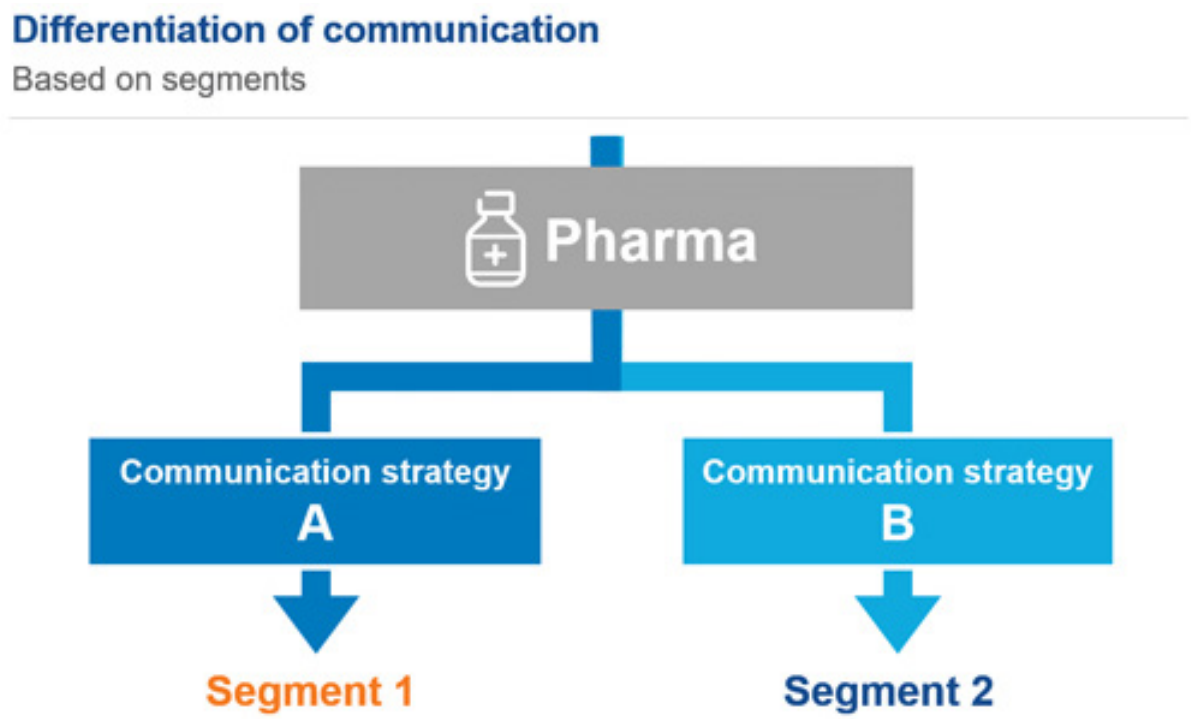


Understanding how payers vary across these parameters allows for the identification of leverage points that can be differentiated for each payer segment. Payers can then be targeted via the channels through which they are most likely to be receptive to engagement, and the content of a product's value proposition can be tailored to their preferences. For example, when evaluating a new entrant to a crowded market, one payer segment may be more open to considering PROs than others who remain more focused on traditional endpoints. In that case, the value proposition can be differentiated by highlighting PRO data in the core value story to add value for payers in the first segment but excluding it for the second segment to avoid unnecessary data load.

In this way, payer segmentation facilitates a shift towards more effective value-based discussions between pharma companies and payers by putting customer relationships at the centre of the communication strategy.

Segmentation can also be beneficial when it comes to negotiation tactics. One payer segment may only be interested in financial-based agreements, while another may see value in additional services. Understanding this allows the manner of entering negotiations with each segment to be differentiated and limited resources to be prioritised by only offering additional value to the second segment.

A key difference to segmentation in the HCP and patient context is the feasibility of large samples for robust analysis, given the limited universe of payers. Effective attitudinal payer segmentation requires advanced analytical techniques combined with informed interpretation. This must be built upon a thorough framework that captures the full range of potential internal and external factors that may potentially influence payers' decision-making, rooted in an in-depth understanding of the market access landscape. For the segmentation to be actionable, it then needs to be possible for each payer segment to be described and identified based on a minimum number of highly predictive parameters.



Better payer engagement leads to improved market access

Attitudinal payer segmentation can support pharmaceutical market access strategy by establishing a logic of heterogeneity among payers and providing a framework that enables pharma companies to navigate that diversity. Effective application can support an access strategy that meets the requirements of a broad payer audience while strengthening the customer relationship with each payer.

Although efficacy, safety and cost will inevitably remain at the forefront of value communication to payers, today's increasingly challenging access environments call for a need to re-think how the nuances of this communication can be tailored to attitudinal preferences in order to secure access opportunities and ultimately better meet patients' needs.

To learn more about attitudinal segmentation with payers and how Research Partnership can potentially support you in this area, visit our website.

About the author

Cecilie Alstad is a senior analyst in Research Partnership's dedicated Market Access team. Much of her work focuses on Europe where she has specific experience in HTA evaluation, pricing & reimbursement strategies and value communication across a variety of therapy areas including orphan diseases, NCDs and infectious diseases (incl. COVID-19). Her experience spans different stages of the product lifecycle, and covers key markets, including North America, Scandinavia and emerging markets (e.g. LATAM, MENA and APAC). She holds a 1st class MSc from Karolinska Institute/ Imperial College London, and a 1st class BSc from Queen Mary University of London, which collectively covered biochemistry, nutrition science and public health.

About Research Partnership

Research Partnership is the largest independent healthcare market research and consulting agency in the world. We collaborate with clients from the global pharmaceutical, medtech and biotech industries, providing research intelligence and strategic recommendations that elevate healthcare brands and power their success. Our specialist market access service supports the world's leading manufacturers in market access, pricing, and reimbursement. To find out more please visit:

researchpartnership.com/marketaccess

Building honest dialogue with patient advocates

How can the industry listen to patients in a way that puts the person behind the disease first? Blogger Chris Aldred and Joe Delahunty from Ascensia have been working together to answer that question for five years. They tell us what they've learned and discuss what the industry often gets wrong in its patient engagement.

It's not often I speak to a patient advocate and an industry representative on the same call – usually it seems the two parties keep a respectful-if-wary distance from one another. But Joe Delahunty, global head of communications for Ascensia Diabetes Care, and diabetes blogger Chris Aldred have a long-running partnership in building up the company's patient engagement efforts – and so when they discuss these activities it makes sense for them to do it as a duo.

Chris leads the editorial board for Ascensia's patient blog, and has worked closely with Joe on many of the company's other patient engagement projects. They are both keen to discuss what they've learned from years of witnessing patient-industry interactions and finding ways to make these efforts work better for people with diabetes.

Chris, better known by his online pseudonym 'The Grumpy Pumper', was a well-established diabetes blogger before Joe first got in contact with him. He says it was a "total accident" he became an advocate, after he joined the online community to keep in touch with other patients he'd met and found that people liked his "honest" thoughts on diabetes care.

"There aren't many people who wake up one morning and go, 'I'm going to be an advocate'," he says. "I think everyone in the diabetes community advocates for something because they're passionate about it.

"I don't know at what point I became an advocate, but I think in a way I always was."

He highlights this to counteract the idea that patient advocacy is "dirty work" that involves "selling out" to the industry.

"Some people think we sell our souls to the industry – but every advocate I know does this because of their own passion, often at great expense to themselves."

Nonetheless, Chris is vocal about what will and will not do for a pharma or medtech company – and when Joe first reached out to Chris in 2016 to get his thoughts on a new meter, Chris was clear that he wouldn't treat it as a product review and blog about his thoughts.

"Joe said that was fine, and that Ascensia just wanted my honest opinion. It's always nice to see someone that isn't just contacting you to push their product to sell.

"He called me back a couple of weeks later and I gave my opinion – and he learned that I can be very honest about these things."

Chris also told Joe that he would never engage with a company unless they gave something back to the community.

"We started talking about a campaign that had been community-run for a few years – Spare a Rose, which encourages people to buy one less rose on Valentine's day and instead donate the money to Life for a Child, which will provide insulin to a child in a less advantaged country.

"Joe said that Ascensia's employees were also keen to find opportunities to give back to the diabetes community, and because of this interest and the simplicity of the message it would be an easy sell – so we got talking and planned for the next year's campaign. Ascensia ended up doing a great job for us, and have run it every year since.

"I know there's always a corporate advantage to these relationships, but I don't care if it's also being done to stop kids dying of type 1 diabetes."

Joe later hired Chris to go to various medical conferences and write reports from a patient perspective.

"It's very rare that patients actually get to go to HCP conferences," Joe notes. "We would publish Chris' reports on our website and promote them with patients so they could see some of the developments that were happening at these meetings. Then Chris would give his own perspective on how they were going to affect care."

Chris adds: "They wanted something in my own words, so that it would be something that people with diabetes would actually be interested in reading, instead of the medical, technical data that usually comes out in the reports. Personally, whenever I read reports like that I get bored."

These writeups proved to be popular with the community, and over the years have developed into the Ascensia.com blog, for which Chris leads an editorial board that meets once a month to decide on topics to cover.

Joe says the aim for the company is to have a "hands-off approach" to these kinds of patient engagement.

"Chris and the editorial board drive the content. That's important for us because it helps us understand the needs of the diabetes community, and how we need to work and build out products to address them."

Chris and other online advocates have also worked with Ascensia on Diabetes Social Media Summits.

"We bring together a group of advocates for a few hours," Joe explains. "We sit down and talk about the topics that they feel are important. We then look at those topics and ask whether there are any where there could be a mutual agenda with the company. If there are, we come up with campaigns or initiatives that try to address them.

"Some are things that the community wants to address and work on themselves, and for others they want Ascensia's support and partnership. We look at the best ways to approach each one."

Part of the appeal of the summits for Chris was as an alternative to other meetings that had become "too big and too commercialised".

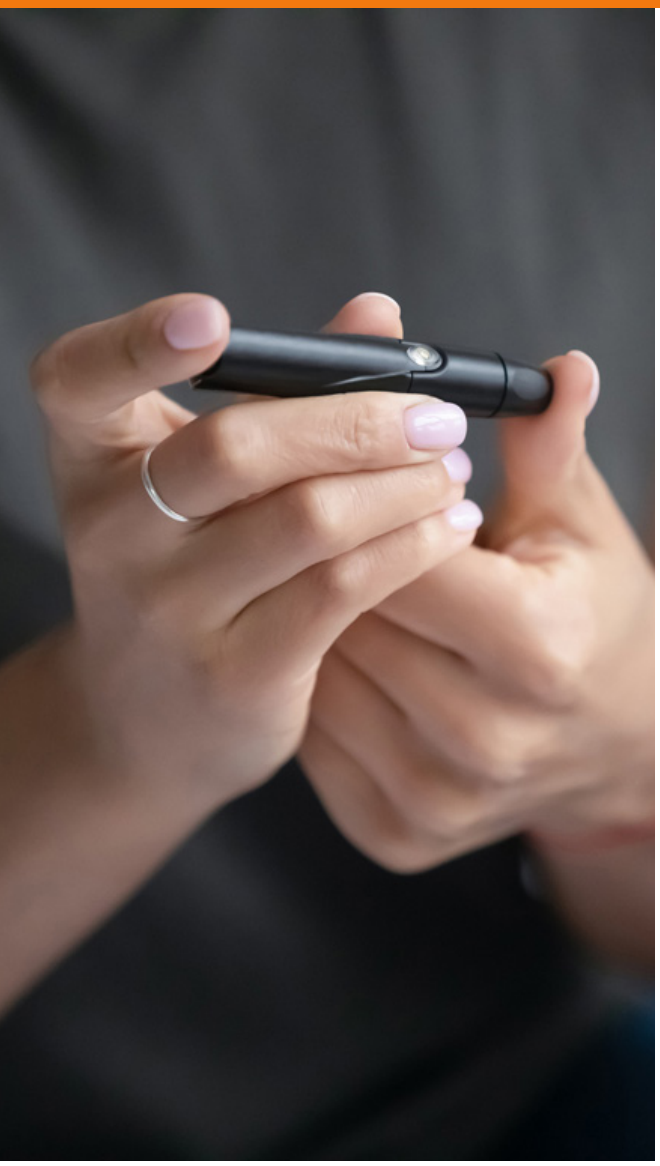
"I've been at a lot of events where you never see any tangible outputs come out of them – then when you return the next year it's like everyone is just redoing the same thing.

"When Joe and I were talking about the summits, I said I didn't want the same people in a room, doing the same thing, with no output, every time. So we try and invite different people to every meeting we've done and come up with tangible goals from them."

Start with the person

Chris' key message is that companies engaging with patient advocates should "start with the person", not come to them from a product point of view.

"Companies will often tell you how exciting their products are, how they're going to make your life great. The thing is, I know how lucky I am to have the technology I have, but I don't actually want any of it, because I don't want diabetes. I didn't ask for this.



"I see a lot of ads for these technologies that have the implied message of, 'Wouldn't it be great if people with diabetes didn't have diabetes?'. These adverts come off as very patronising and stigmatising to the people that don't have access to the technology. And that's still one of the biggest challenges in this area – access to standard technologies, healthcare and education."

Joe echoes this.

"Rather going to advocate like Chris and saying, 'This is what we want to do, can you be involved?', we want to go to him with a blank page," he says. "If we want to do an event, for example, we can ask him what topics are important to the diabetes community at the moment, then try and come up with a shared purpose and see where we can work together.

"The industry doesn't see enough of that."

He adds: "If you do want a group of advocates to look at a product for you, it's best to be upfront about it. We've had good feedback in the past when we've been clear that we want people's honest opinions on a product, and we've not asked them to write about it or anything afterwards. If those discussions happen as part of another patient advocacy meeting, we try to keep them separate from the rest of the discussion.

"That allows people to maintain their independence. If they don't want to be involved in that part, they can leave."

Both of them note, though, that it's important to recognise that each person with diabetes will have different experiences and different opinions.

"I go to Chris if I want to know what a white male based in the UK thinks about certain aspects of diabetes care," Joe says. "If I need wider opinions than that, I ask him who should I speak to in India, or Japan, or the US, and expand the network that way.

"Even when you're doing that you have to keep in mind that there are millions of people with diabetes across the world. Each one of them has a different experience and different challenges, and you're only ever going to get to a very small portion of it.

"You've got to try and take the advice that you've got and look at where there are common things that will be useful for a large group of people. Remember that different people are going to have different challenges."

This is particularly important when it comes to product design, says Chris.

"Sometimes you look at a device or an app and think, 'No one that lives with diabetes has touched this until now, because all the features are just wrong.' Or it hasn't taken into account the wide range of individual needs in the community.

"A good example of that is a meter that beeped loudly when it receives the blood. That didn't bother me, and someone with vision issues really liked it. But a young woman I knew who was in school at the time said she would never use it, because everyone in class would look at her when it beeped.

"Even the kind of device that people want is going to vary. I've been at talks with healthcare professionals that look after people in very deprived areas in America, who won't have a smartphone because it will probably get stolen.

"It's not just about coming to patients with a blank page, but also thinking about who your target audience actually is."

Insights into actions

The hope from Ascensia's side, of course, is that insights gathered from these activities can help inform more patient-centric ways of working and designing products.

Joe gives a few examples of things the company has learnt from its patient engagement, including realising the impact of diabetes-related complications for the community.

"One of the things we identified was that there was not enough conversation about this – in fact it was almost a taboo subject within diabetes. People tend to either talk about complications in a way that scares people or they just don't talk about them at all.

"With the advice of a social media summit group and the editorial board, we came up with an unbranded campaign to raise awareness of these complications and the fact that they need to be talked about. We launched that on Facebook last year."

Conversations with patients have also identified the fact that people with diabetes often don't have a good "back-up plan" in case the technology they use every day fails.

"We worked with the community to develop a checklist of what they should be carrying with them in case their usual technology breaks, including the items they should carry on a day-to-day basis and what they should take with them when they're away on holiday."

Joe says the company also found that patients felt that the language used to describe diabetes can often be demotivating, alienating and cause stigma.

"There is a strong movement within the diabetes community to ensure that when people are talking about diabetes, they talk about it in a way that is encouraging, not stigmatising, and does not cause anxiety.

"We decided to create a language education module for our employees. When someone starts at the company, they have to go through that and learn how best to interact with people with diabetes."

As for why companies should encourage patient advocacy, Joe says that it allows companies to contribute to the "greater good" for communities in a way the industry has not traditionally done.

"It comes back to that idea of having a shared agenda, finding the goals that are of mutual interest and making sure you're working on them together."

"The more pharma and medtech employees that understand the challenges of living with diabetes, the less they'll make assumptions on what people want," says Chris. "It's important to be able to be able to have open conversations with them and be able to say what you want to say."

"I think that honesty that people like Chris bring to the table is incredibly important," Joe adds. "People like hearing someone talk about issues that aren't usually discussed. They feel like a problem shared is a problem halved."

Chris sees this as another reason why 'advocate' is not just an official title, but something any patient can do, even if it's through small acts.

"If all you do in your advocacy work – or whatever you want to call it – is make someone feel less alone, less like they're walking in the dark, that's a job well done."

About the interviewees

Chris Aldred was diagnosed with Type 1 Diabetes at the age of 25. Fifteen years later, Chris started to blog as The Grumpy Pumper and has been an active member of the Diabetes Online Community ever since. He is widely considered to be a global diabetes advocate and is a regular speaker at global diabetes conferences. Chris is a member of the Council of People with Diabetes for Diabetes UK and a board member of IDF Europe.

Joseph Delahunty is global head of communications for the PHC Group and Ascensia Diabetes Care, overseeing all internal and external communications activities, including patient advocacy. Prior to Ascensia, Joseph worked for FTI Consulting for more than 12 years, in their London and New York offices, leading global communications programs for healthcare clients.

About the author

George Underwood is the editor for pharmaphorum's Deep Dive digital magazine. He has been reporting on the pharma industry since 2014 and has worked at a number of leading publications in the UK.

PTC THERAPEUTICS™

measured by moments

Q&A: PTC Therapeutics' Alberto Vicent on listening to ultra-rare disease communities

When it comes to ultra-rare diseases, the smaller the patient population, the bigger the challenges.

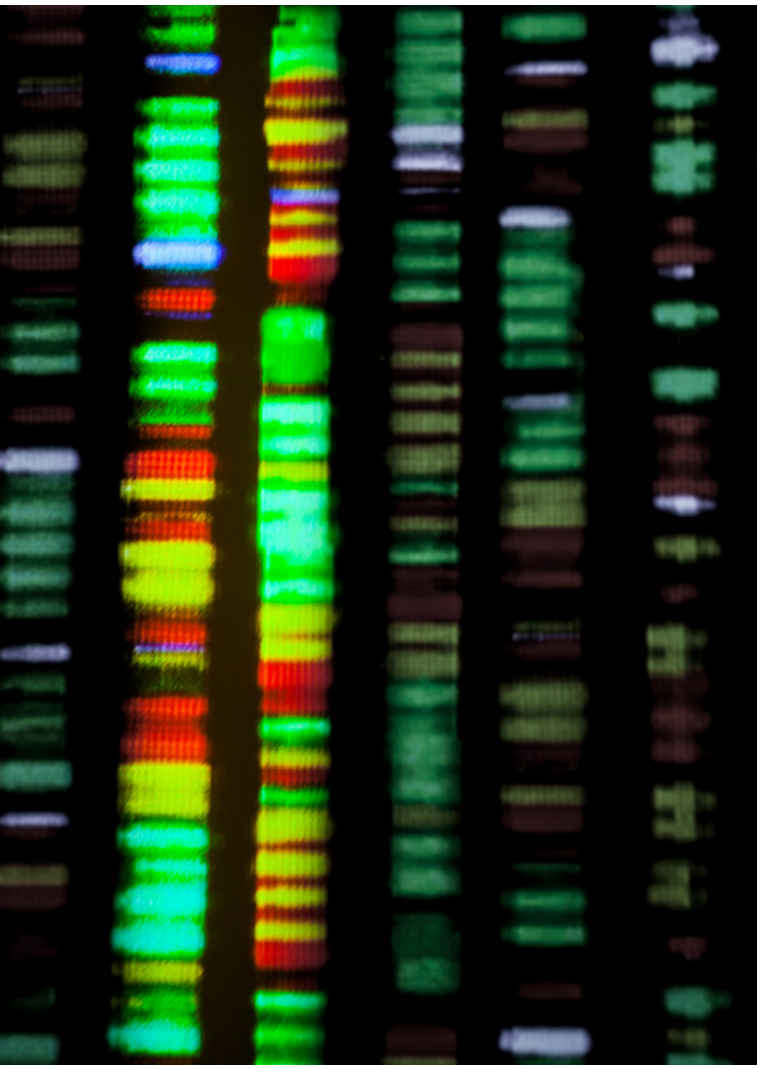
As a global commercial biopharmaceutical company pioneering therapies for ultra-rare diseases, PTC Therapeutics is familiar with the challenges. The company prides itself on a patient-centric approach to treatment but there can be significant hurdles in patients accessing potentially life-changing treatments. Alberto Vicent, vice president and general manager Southern Europe, tells pharmaphorum why we need to view ultra-rare populations differently, so they can also access treatments.

What are some of the challenges of developing innovative therapies for ultra-rare patient populations?

There are challenges at every stage of the journey from lab to patient. First and foremost, it's extremely challenging to develop a deep understanding of ultra-rare diseases due to small and disperse patient populations, limited understanding of the natural history of the disorder, commonly no established clinical endpoints to be able to conduct clinical trials and the fact that many patients are misdiagnosed and there is often no standard of care.

Once all of that is addressed and a treatment is discovered, it goes through years and sometimes decades of clinical trials and final regulatory review. The challenge is for the patient to be able to access the treatment. Payers and manufactures must come together in order to ensure that a treatment can reach the patient. This is especially relevant for the new era of gene therapies that are disease modifying, offering hope for some of the most intractable and devastating diseases, many of which affect children. As these therapies are intended for one-time use and bring tremendous transformative value over a lifetime, it is imperative to consider the long-term value and benefit to quality-of-life improvements these treatments can offer, not only for the patient but also for the families.

How can we ensure pharma companies continue to innovate if there are these hurdles?



I believe many would agree that patients with rare disorders deserve access to treatments. Regulatory pathways have been established in order to aid companies willing to take the challenge to develop treatments for patients with rare diseases. However, in order for treatments to be developed, companies need to be able to fund the discovery and development of those treatments.

Manufacturers and payers have been working together in order to ensure that patients have access to these treatments, however these arrangements have not been designed to address the new wave of disease-modifying, one-time gene therapies. They offer groundbreaking opportunities for severely debilitating or life-threatening conditions.

The focus must be on the value associated with the long-term benefit. Evaluation needs to capture what lifetime benefit means to patients and caregivers on the one hand, and value to payers and society on the other.

This is novel, unchartered territory and it's vital that we work together to adapt reimbursement pathways and mechanisms and learn from the early experiences. A reliable and predictable pathway is vitally important for patients. Once we can provide a framework that continues to drive the science and development of innovative treatments, only then can patients access the transformative value that gene therapies offer.

How do you identify and find these ultra-rare disease patients and raise awareness about what is going on?

At PTC, we undertake physician education and patient screening initiatives to identify patients and encourage earlier or correct diagnosis of ultra-rare diseases. For example, we are supporting access to free-of-charge genetic testing for patients with suspected AADC deficiency, an ultra-rare disease, as well as initiatives to educate physicians to incorporate AADC deficiency diagnostic tests into their work up of patients, and biobank screening initiatives that allow physicians who are undertaking patient screening to quickly screen databases.

How do ultra-rare diseases impact decisions about access?

Ultra-rare diseases significantly complicate access, but it is vital that all stakeholders work hard to ensure ultra-rare disease populations have the same rights to access treatment as those with conditions that affect more people.

The challenges are greater with novel technologies, like gene therapies, which offer particular hope to these populations.

The rarity of a condition, the limited number of specialists and the complexity of the technology mean these treatments cannot be administered in a community hospital setting. Instead, they need highly specialised and accredited treatment centers staffed by specialists, doctors, and nurses who need to have dedicated training.

Generally, there is a need for consistency and alignment around patient access – the manufacturer, the patient, and the hospital. It is important that the patient journey is clear across all these aspects.

What are some of the post-approval challenges of developing global innovative therapies for these small patient populations?

We need to improve physician awareness and understanding of an ultra-rare disease, and work with clinical experts to improve and many times establish diagnostic pathways. We need to ensure the right patients can access therapy wherever they live. That starts with facilitating diagnosis right through the setting up of treatment centers and engaging payers.

Why do you think it is so important that the needs of ultra-rare disease populations are heard?

It is vital to give a voice to those who otherwise have limited access to treatment. The nature of ultra-rare diseases is such that families living with some of the most devastating and debilitating diseases have no organised voice to advocate for better care or access to innovative therapies.

Many of these families are facing battles at every stage of their journey, on top of caring for a severely ill child, simply because of a lack of knowledge of the disease. They may face misdiagnosis or no diagnosis, failed attempts at treatment or no treatment. Typically, there are no guidelines, minimal disease knowledge and limited or no effective treatments.

Little understanding of a disease and no established patient organisations can lead to social isolation and suffering. Families often also face significant financial problems as there is an extremely strong caregiver and financial burden in neurologic and childhood diseases.

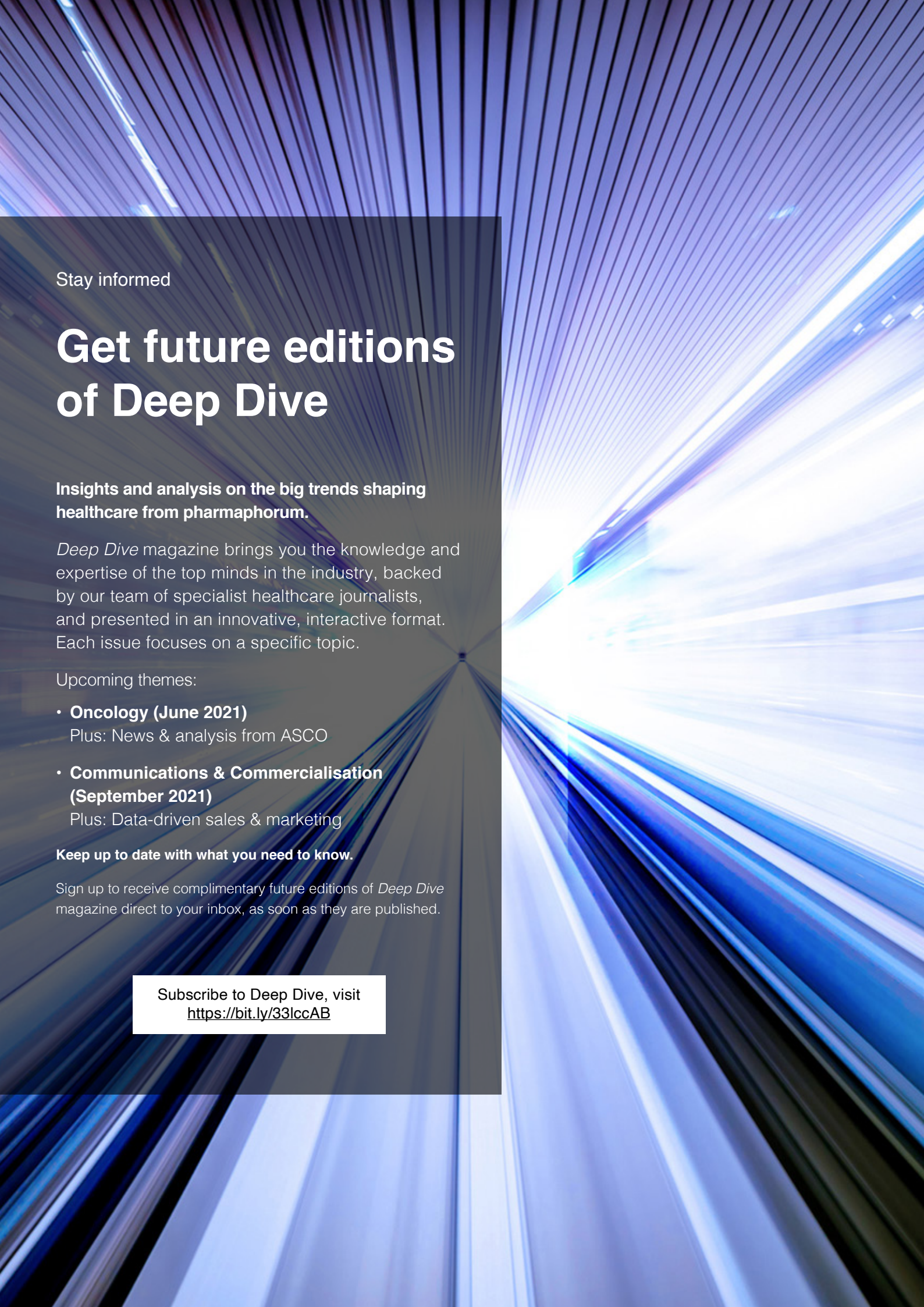
Sadly, the caregiver and family financial burden is often not recognised in some reimbursement pathways and can also lead to poor appreciation of the true impact of a disease.

About the interviewee

Alberto Vicent is VP and general manager Southern Europe at PTC Therapeutics. Alberto, a trained pharmacist, has spent most of his career focused uniquely on rare diseases for a number of pharmaceutical companies, including Shire Pharmaceuticals, Synageva Biopharma and Kyowa Kirin. He is a past president of AELMHU (Spanish Association of Orphan & Ultra Orphan Manufacturers).

About PTC Therapeutics, Inc.

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialisation of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialise products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximise value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.



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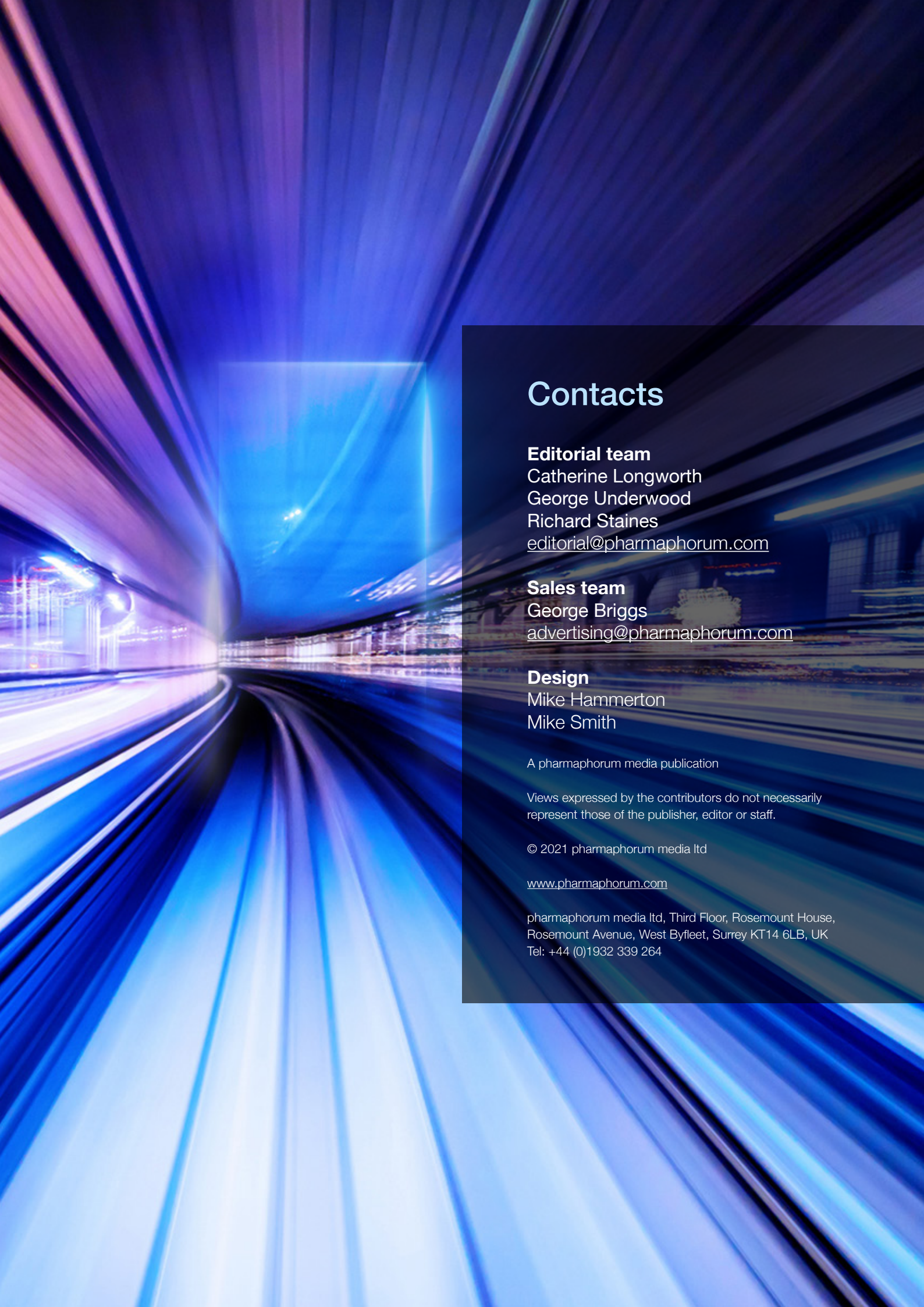

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A pharmaphorum media publication

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