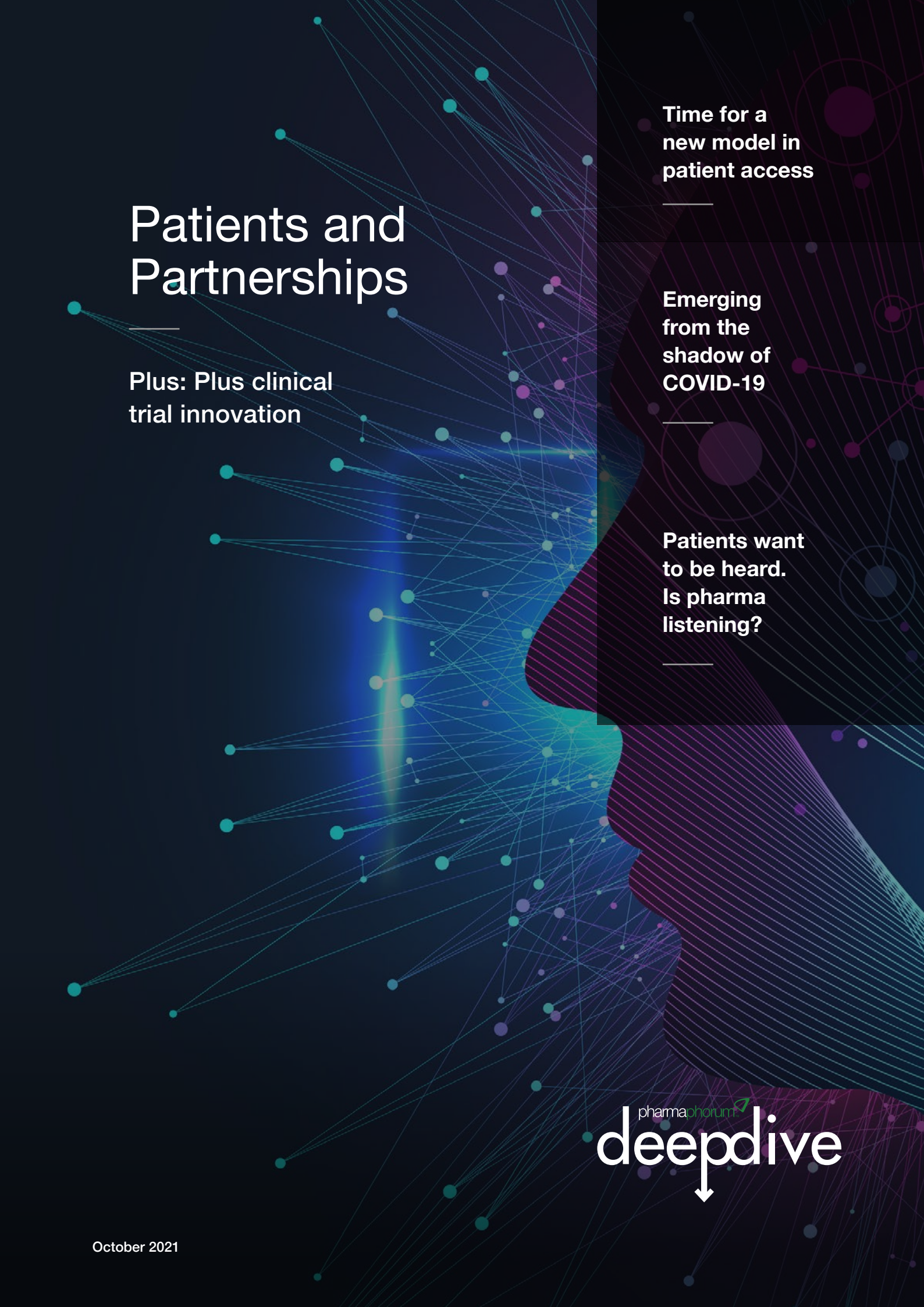

Patients and Partnerships

Plus: Plus clinical trial innovation

Time for a new model in patient access

Emerging from the shadow of COVID-19

Patients want to be heard. Is pharma listening?

pharmaphorum deepdive

October 2021

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pharmaphorum deepdive

Deep Dive: Patients and Partnerships

The world is slowly, gradually, hopefully emerging from the shadow of COVID-19 and as it does so there is, as Alnylam's Kasha Witkos notes in this issue, a unique opportunity to re-discover what matters most to patients.

"Patient centricity is continually evolving and as an industry, we must evolve with it if we are to leave a legacy where we can say we made the biggest difference we possibly could," she explains.

Patient access also features prominently in this issue of the magazine, with Astellas Pharma Europe vice president of global pricing Mig Sleeper explaining why it's time to rethink how value is defined.

Elsewhere in the issue there's informed commentary on health literacy, patients' role in the healthcare conversation and we also look at R&D innovation in areas such as biomarkers and real-world data.

Dominic Tyer
managing editor, Deep Dive

Next issue:
Digital Health Innovation (November 2021)
Plus:
- Pharma's evolving approach to oncology
- Healthcare transformation

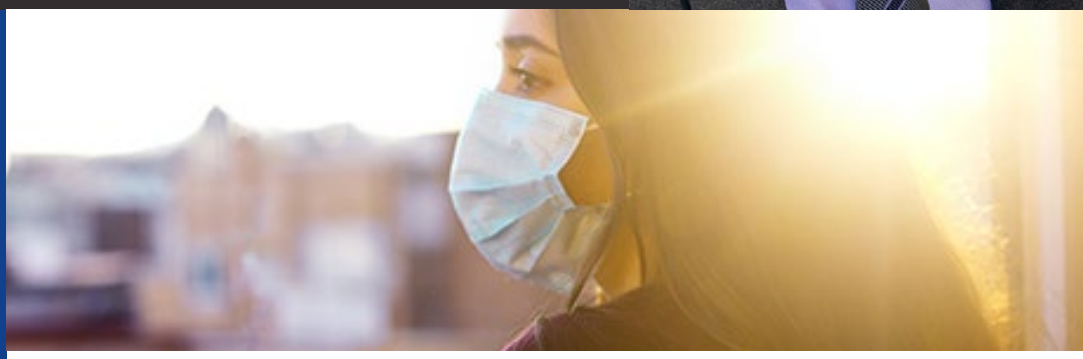
Catch up on recent issues:
Communications and Commercialisation – September 2021
Oncology – June 2021

Market Access – April 2021

Time for a new model in patient access

Mig Sleeper, vice president, Global Pricing for Astellas Pharma Europe, explains why we must rethink how value is defined as we enter a new technological era for medicines.

Why is a new access model so pressing, and has the pandemic accelerated the situation?



Yes, it has. Although the need for a change in the market access model predates the arrival of COVID-19, the pandemic has accelerated this process.

We have continued to see over the past 20 years, the nature of technological capability and company therapeutic focus shift from high-volume, low-cost therapy areas, such as diabetes and depression, towards high-value medicines targeted at niche patient populations. And, now, we see the arrival of even more specialised therapeutic technologies, which have enabled cell and gene therapies that can be tailored to an individual's genetic profile.

Industry's relatively recent shift towards rare diseases is symptomatic of this technological advance. The frequency of these 6,000-plus individual conditions – collectively affecting 30 million people in Europe alone – has pushed the pharmaceutical business model even closer towards lower volume, higher individual cost therapies.

But, within a customer environment that's always been cost conscious, this transformational shift has unsurprisingly made questions concerning budget and cost-effectiveness even more acute. On top of this, the pandemic has served to intensify existing healthcare system pressures, meaning we can anticipate an even sharper focus on cost savings in the future.

What does this mean for pharma?

It's too early to say fully but, in the past year, it's evident that medicines are being more frequently subjected to sales or budget caps. In this scenario, the marketing company pays a proportion or all the treatment's cost back to the payer over an agreed budget threshold that effectively 'caps' the cost to healthcare systems and governments.

I believe this reflects the beginnings of a fiscal response to the pandemic and will likely be followed by governmental and healthcare systems' need to follow up with further remedial cost management measures. It's also indicative of wanting a degree of financial certainty – and applying that cap ensures treatments can remain within a known, given budget envelope.

Is there a way forward that balances access to innovative medicines with the needs of financially constrained healthcare systems?

Cost-effectiveness as a measure of value is a very narrow one. We need measures that employ broader reflections on what value really is and, to do that, we must focus on what value means to all our customers.

This means identifying the critical questions we need to answer; the healthcare problems we need to solve; and then to orientate our technology towards those aims. Of course, this requires additional risk and investment on the part of industry if we are to generate the necessary evidence to answer those questions to our customers' satisfaction – as opposed to satisfying solely our own internal measures of value.

For example, we understand that to meet the expectations of oncologists and payers, trial data must show meaningful improvements in overall survival – generally acknowledged to be an extension of months to years depending on the tumour type and stage of disease. But, for these potentially curative, highly specialised technologies, what constitutes a "cure"? What is a representative study population? How do we define and agree these future outcomes? And over what timeframe?

There are many unanswered questions in terms of defining what that robust evidence base looks like and how we best meet the needs of patients, health professionals, payers and health technology assessors within reasonable cost and time constraints. But, I believe, to some extent, that these challenges are starting to be acknowledged and we are pushing against an open door.

Has this meant a change in approach for Astellas?

Where it is unrealistic to deliver against the customer's ideal definition of value, such as a definitive cure, we are, where appropriate, prepared to go the extra mile and gather additional evidence and data post-launch, with a view to accepting that reimbursement status and price will be contingent upon that evidence and data over the next, say, two to five years.

This is our approach to delivering Evidence-Based Pricing (EBP), whereby we commit to producing evidence to demonstrate value post-launch and we link the successful delivery of that data to price. Ultimately, the price – and therefore revenue – is contingent upon delivery of real value in the real world and with real patients over time, to the satisfaction of our customers and patients.

Ultimately, the price – and therefore revenue – is contingent upon delivery of real value, in the real world and with real patients over time, to the satisfaction of our customers and patients.

How does this work in practice?

It's hugely complex. Evidence generation would always ideally be able to deliver the endpoints and certainty the customer requires at the time of launch. But, if this is not possible – sometimes for ethical reasons, insufficient patient numbers or prohibitively lengthy studies – you need a plan B.

For us, this means preparing three to four years ahead of launch; co-creating with primary markets; anticipating unavoidable evidence gaps, post-launch issues or other uncertainties faced by customers; working with R&D to deliver a pivotal trial protocol that is acceptable to those stakeholders; and building a deep understanding of customers' needs to ensure our product is well received in the market and can fit within payers financial planning horizon.

Of course, collaborating and partnering closely with our many internal and external stakeholders is key if we are to achieve this critical objective. It's this early engagement or co-creation that determines the likelihood of success (or failure) in achieving reimbursement for our products. It also informs the level of product promise we commit to and the commercial sustainability of that promise.

Our industry has always acknowledged risk as being integral to the process of taking medicines from discovery through to market. The level of uncertainty has been amplified with these highly innovative therapies but, as we progress on the trajectory towards launch, we begin to understand whether the EBP approach will become more or less likely in individual markets.

Creating these bespoke value propositions is only possible by us engaging with our affiliates on a country by country basis, early enough in the development process.

Isn't value really about affordability?

Not at all. Value and affordability are not the same thing. A Rolls-Royce motor car, NASA's Saturn V lunar rocket and CRISPR gene editing technology are all undoubtedly high value, but unaffordable to most. The one area where pharma companies can really make a difference is on the question of value and this must be constantly redefined if we, as an industry, want this to be fairly reflected in our prices.

Don't misunderstand me, affordability is a vital part of the access story, as it's about doing everything we can to reduce or remove many of the barriers to patient access. These may be financial, logistical or systemic barriers or uncertainties at launch regarding the value that will be derived for the patient, health professional, payer or healthcare system. So really it's our duty to do everything we can to understand those barriers and ask ourselves what we may do to help patients overcome them?

Currently, a medicine's worth is still measured in the traditional way with reference to price per pill or vial, per day or month, but that's not good enough in the fast-changing pharmaceutical world of today. We must find ways to price based on what the medicine can do, recognising the technical difficulty, risk and investment in making that happen. Another example is that few markets have the wherewithal to price medicines coming to market with multiple indications with differing value points and cost-effectiveness outcomes.

For me, it comes back to redefining value: removing uncertainty and exercising flexibility around the pricing model benefits the value proposition and improves affordability.

What needs to happen next?

We know there is a sense of urgency – inflated and accelerated by the pandemic – to forge solutions that will address the question of value for the raft of technological advances that have already arrived or are coming closer to market. But healthcare systems and price and reimbursement processes are still very much rooted in pre-pandemic, traditional pricing and access models.

So, there's a lot of work to do to make positive inroads with new approaches in a way that builds trust with customers.

Ultimately, it's about value for money. If our products show minor incremental improvements against 10 – 15 year-old therapies, yet demand a substantially higher price, our customers have made it clear that's not good value for money. If we want to achieve prices that are sustainable for the industry and attractive enough to enable forward investment in difficult-to-treat conditions, then we must deliver significant value by showing a meaningful increment in patient-relevant and health economic endpoints.

Finding a sweet spot that's common to all stakeholders – with mutually agreed objectives and goals – will allow each stakeholder in the healthcare ecosystem to benefit. This value 'threshold' will vary from customer to customer and from market to market but, fundamentally, it represents value for money. It doesn't exist inside a snow globe, it's a real thing. And that's the Holy Grail for market access: timely access for patients, good value for healthcare systems and a sustainable, justifiable price for pharma.

Emerging from the shadow of COVID-19

We now have a unique opportunity to re-discover what matters most to patients, says Alnylam's Kasha Witkos.

In the biotech industry, if you are not motivated by helping patients and potentially transforming their lives, then you're in the wrong job. Yet understanding what people go through on their journey – particularly those living with a rare disease – is never straightforward.

We all hear daily talk of patient centricity and keeping patients at the heart of everything we do – but the reality is that what patients and caregivers need is often very different, even in single diseases. It also constantly changes, and COVID-19 is a clear example of where unexpected challenges have re-written much of what people prioritise as important.

As we come out of what has been a very dark year for many people, particularly those with a disease who have been extremely vulnerable to COVID-19 infection, we can reflect on what was done well and what could have been done better. Patient centricity is continually evolving and as an industry, we must evolve with it if we are to leave a legacy where we can say we made the biggest difference we possibly could.

Creating stronger connections

Perhaps the most significant positive that has emerged for me from COVID-19 is that the whole experience has brought us closer to our patients and the communities that we serve. It has strengthened our connections and our ability to see what they go through. We have listened more intently, understood more fully, reacted with incredible speed, and innovated in exceptional ways. I'm certain that as a result, we know far better the experiences that patients and caregivers go through, and what really matters to them when life is at its most challenging.

Already we are capturing insight from a wider and more diverse set of people, including not just patients and immediate family members, but other caregivers and people impacted by rare diseases.

And we want to share the insights we gather. Partnering with patient groups we have been exploring how to do this. Our campaign One Step Further in the UK is a perfect example of this – realising that a disease like ATTR amyloidosis can have a far-reaching impact and that, if we take time to look, we will see the reality of this laid bare in the stories of loved ones, caregivers, children and friends. Creating this window lets us all learn more about what we can do to help. The challenge now will be to ensure that we continue to build these connections – not just with patients but with everyone impacted by the conditions we strive to improve.

Being patient centric at every step of our journey

From clinical trial design and coordinating regulatory approval through to managing reimbursement and care delivery processes, the moment we lose sight of who we are doing this for we decrease our impact. There is still much to learn here, and the reality is that patients and caregivers are – encouragingly so – becoming more and more involved at each step of a medicine's journey.

Our job must be to encourage this. To build this empowerment, educate and upskill those on the front lines of diseases – and trust that doing so will bring benefits for our industry as well as those in need.

At Alnylam we have looked to adopt this mentality, creating new platforms aimed at driving open and instructive interactions with patient advocacy leaders. This has ranged from facilitating business school training sessions to improve inclusion in health technology assessments to working with partners to remove industry jargon where it may be acting as a barrier to patients engaging with important processes along a medicine's journey.

Adapting to a new era of health technology

Even before the pandemic, it was apparent that technology was set to radically change the delivery and personalisation of healthcare. But it has acted as a catalyst in certain sectors – forcing our industry to bring forward technological innovations, moving communications and patient interactions online and reshaping healthcare in many other ways.

The recent launch of our digital symptom tracker app, STAR, to support people with ATTR amyloidosis and their families is an example where we have identified a need and acted with urgency, delivering a tool that can help patients from an early stage in their disease see, record and – if they choose to – share their symptom changes with their healthcare team. Beyond diagnosis and disease tracking, we have also built an interactive Family Dialogue Tool to help individuals have important (but often incredibly difficult) conversations about their conditions with a broader family network.

Health at home

Shielding patients have of course often had to, in many cases, receive care at home. As many of us across the industry know, implementing homecare in complex disease areas is no mean feat. But there have been many cases where we have achieved it in record time, and with great success. Yes, there are still important considerations around duty of care and appropriate management of patients from home; however, industry and health authorities have had their eyes open to new possibilities and have worked together to make homecare for many a reality. Having heard stories of people travelling hundreds of miles every few weeks for their appointments, the impact could be life-changing.

Diversity

COVID-19 has raised some serious questions around discrepancies in the way certain minority groups have suffered more than others. This is a longstanding issue that transcends many diseases, rare and otherwise. It is one that Alnylam – like many others – is keen to act on. We are now taking further action to explore every opportunity to inspire participation in our activities from a more diverse group of patient populations, so that everything we do represents a broader range of people, ethnicities and backgrounds. We are striving for more equity in the system, fuelled by an omni-channel approach that encourages fairness.

Inspiring healthcare professionals

The last but perhaps most important point I want to make about patient centricity and the industry's efforts to bring in the patient voice is recognition of the pivotal and inspirational role that healthcare professionals play in this process.

Reaching patients and getting them engaged and empowered almost always requires healthcare professionals who see the value in doing so. Time and again they go above and beyond to make sure that people have their voices heard. They hold us accountable for acting on the promises we make and without them much of the progress we have seen would not have been possible.

Many lessons have been learned from the last year but a central one is that we can always improve patient centricity. I believe we have an exceptional opportunity to do so and that, working alongside the clinical and patient community, we will create change in the coming years that many of us would not have thought possible.

About the author

Kasha Witkos is SVP, head of CEMEA at Alnylam Pharmaceuticals

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Acknowledgement
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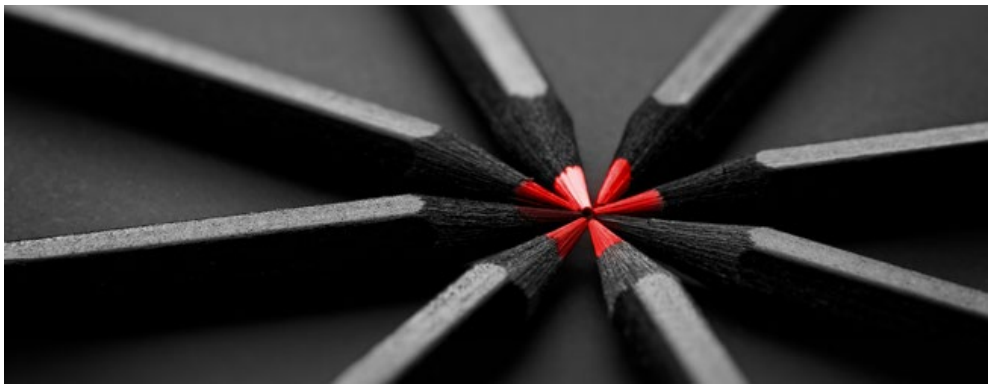
How the combination treatment challenge impacts patients and how we are working together to solve it

How the combination treatment challenge impacts patients

by Eric Low

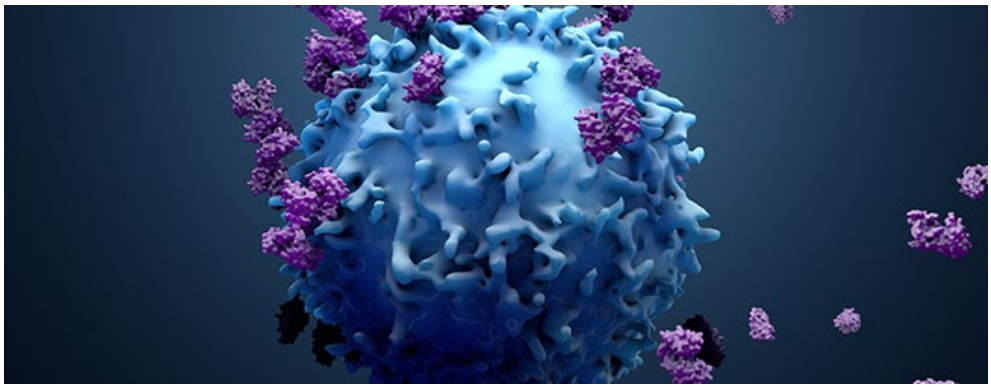
Access to medicines attracts a great deal of interest from patients, clinicians, and the public and there have been significant advances in drug development in recent years which looks set to continue.



Many new treatments can provide improved outcomes for patients, extending and increasing their quality of life. However, in some cases, funding new treatments can present a challenge to health systems around the world as budgets are finite. Therefore extensive consideration of the clinical and cost-effectiveness of treatments must be made, to justify their value for money and their value to the health system.

The combination treatment issue

Combination treatments combine two or more individual treatments, and have the potential to deliver significant clinical benefits to people with complex diseases such as cancer, HIV, rheumatoid arthritis, and Hepatitis C.



As understanding of disease increases, combination treatments are becoming increasingly common and critical to improving patient outcomes.

Combination treatments are often expensive, especially when multiple on-patent treatments are combined, which makes funding them a challenge for health systems. Often treatments included within a combination are developed by different companies and are priced independently. This poses a challenge as discussions between companies to agree on pricing and reimbursement are prohibited by strict competition law.

Increasingly, Health Technology Assessment (HTA) is used to determine which treatments health systems should fund and at what price. A fundamental requirement is that treatments must be seen to represent value. A treatment will therefore usually be priced at a level which the health system is willing to pay, based on its value.

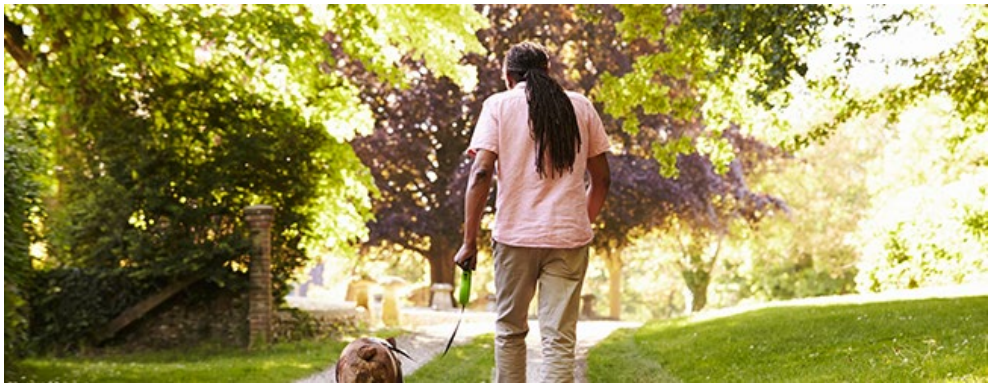
Combination treatments are usually developed by either combining two or more existing treatments, or adding a new treatment to an existing backbone treatment or treatment combination.

As the use of combination treatments can potentially extend the lives of patients, the combination, including the backbone treatment, is used for longer.

This alone can increase the cost of the combination treatment above the willingness to pay threshold, even before the cost of the new add-on treatment is considered. Consequently, the combination treatment would be deemed 'not cost effective', even if the add-on treatment was provided for free. This is problematic because combination treatments can provide value to patients and society but are unlikely to be made available to patients because they are not considered to offer value for money.

Impact on patients

A diagnosis of any rare or complex disease is likely to have an impact on both the quality and length of life of patients. For most, diagnosis or a relapse of disease will feel like a hard kick in the stomach and will be a terrifying confrontation with mortality.



Finding out that tolerable and effective treatments exist is an important step in helping patients come to terms with their diagnosis or a relapse. However, knowing that treatments exist but are not accessible due to cost-effectiveness issues is likely to cause emotional turmoil.

This is not a new issue; but it is one of many access to medicines issues that for too long has been seen as too hard to fix. With the steadily increasing number of potentially transformative combination treatments coming to market, it can no longer stay in the 'too hard to fix box'.

Let's also not forget that patients have taken part in drug trials to determine whether new combination treatments are better than the standard of care. Therefore, we have an ethical obligation to address any barriers standing in the way of future patients benefiting from their brave endeavours and involvement in clinical trials.

The importance of working together to solve this challenge

by Helen Taylor, Takeda

Sadly, one of the universal experiences of being human is that ourselves, families, and friends will be challenged during our lifetimes with ill-health and disease. So getting medicines to the right patient, at the right time, is critical.

Much of the HTA process is about gathering evidence from a multiplicity of different stakeholders to help make cost-effectiveness decisions about treatments. These decisions are not easily made by any of the stakeholders involved, so collaboration is key.

The 2014 report from the Decision Support Unit for NICE outlined the unique challenges and circumstances to assessing combination treatments and little progress had been made on a solution since.

At Takeda we recognised a need for change and in 2017, we held a parliamentary roundtable on this issue in conjunction with the Blood Cancer Alliance, to gain an understanding of the cost-effectiveness challenges for combination treatments from all angles. It was noted that access to combination treatments were either delayed, restricted or did not happen at all. There was strong consensus that there was a pressing need for a solution to be found.

In the years that followed, numerous stakeholders started to review the issue and propose approaches to address the challenge. In 2018, the Amyloidosis Research Group Consortium UK raised the concept of voluntary arbitration between pharmaceutical manufacturers.

In 2019, the Bellberry Group convened an international group of HTA experts to discuss the challenge and, also in 2019, the Voluntary Scheme for Pricing and Access (VPAS) UK acknowledged the challenge and suggested that industry take a lead role in the development of any potential solutions.

In the same year, Takeda UK established an Advisory Group of experts from academia, clinical and patient communities to clearly identify all the issues and design a transactable and implementable solution. We sought expert input from NICE and the NHS on key challenges and to align the solution with current NICE appraisal and NHS England commercial methods.

Takeda's proposed solution, detailed in two Whitepapers (An Attribution of Value Framework and the Voluntary Arbitration Framework), aims to address the economic and competition law hurdles Eric highlighted.

The economic methodology describes how you can fairly divide the value across the treatments within a combination, while the standard operating procedure supports compliant dialogue and agreement between pharmaceutical companies on the value attributed to each treatment within a combination. The solution is treatment and disease agnostic and, although it utilises NICE's methods and processes, we believe it can be adapted for multiple countries and market archetypes.

Next steps and call to action

Both myself and Eric Low have been working on this solution with a core team of passionate and dedicated individuals for the last few years and are delighted to be able to share it. Our proposed solution aims to contribute to the tapestry of options being explored by other stakeholders.

We now invite colleagues and senior leaders across the industry and wider healthcare community to consider the challenge, to review, critique and debate both Whitepapers, so we can ultimately work together to develop an actionable universally agreed solution.

The VPAS asked industry to take a lead role in developing potential solutions, and we hope that our collective brain power can deliver. Keeping patients at the centre of our focus is pivotal to our industry's raison d'être.

To download the Whitepapers, please see this link. https://www.takeda.com/en-gb/what-we-do/combination-treatments/

Thoughts and feedback can be directed by email to the Takeda team at combinationmedicinesUK@takeda.com

About the authors

Eric Low currently runs a consultancy business specialising primarily in strategic market access, life sciences and healthcare policy, patient, and patient group engagement. He has worked in the fields of medical research, market access and patient organisations for over 25 years and set up Myeloma UK in 1996, leading the organisation as Chief Executive until 2017. Eric holds several Board, honorary, advisory, and voluntary positions. He also advises numerous medical and health-focused charities on a pro bono basis. He was awarded an OBE for services to charity in the Queen's Birthday Honours 2012.

Helen Taylor is programme realisation manager at Takeda where, for the last three years, she is responsible for supporting the director of Market Access and head of Appraisals & Centre of Excellence involving optimal planning, early scientific advice projects and risk management. Helen has also provided consultancy guidance for over 13 years in various roles and finally as a Research Associate Partner at MMRG Ltd, a company of healthcare innovation specialists.

Acknowledgement

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Patients want to be heard. Is pharma listening?

A common theme heard from the advocacy groups AXON works with is "Nothing for us without us," says Natalie Turner, director at AXON, a global healthcare communications agency. Patients want to be part of the healthcare conversation and genuinely heard by those who make the final decisions, but pharma does not always understand how to connect with patients.

"A lot of pharmaceutical companies are trying to get their heads around how to include patients in the conversation," says Natalie. "The idea of collaboration and co-creation is at the heart of how AXON builds communities, and how we bring communities together."

AXON works with both the patient community and pharma to improve healthcare outcomes. They introduce companies to the advocacy groups where they've built valuable and meaningful relationships while focusing on putting patients first. They also help to build broader coalitions, garner insights about the patient and caregiver journey, deliver awareness campaigns and support access.

Natalie says companies should embrace collaboration and co-creation to understand the patient journey thoroughly and ensure patients are provided with what they need instead of what companies think they need.

But, from her experience, pharma often doesn't know how to connect with patients and caregivers to build those relationships, and missed opportunities for improving health outcomes can occur.

The strength of collaboration and co-creation

Developing an open line of communication with the patient community and hearing patients' stories can provide pharma with the guidance on how to truly put patients' needs first.

"We encourage our clients to think differently and understand the benefits of working collaboratively. That's where it was born for us, this idea of co-creation, but putting it into practice is quite challenging," says Natalie.

Creating those collaborative relationships can be tricky as some advocacy groups are not inclined to work with external partners.

"It's often about matching personalities and testing the comfort zone of an advocacy group and then matching that with the comfort zone of our clients and where they're at," Natalie states.

"Ultimately, we know collaboration leads to a positive impact on patient health outcomes. That's actually what a lot of people forget. There's a patient story. There's a real person."

Forming connections with patients is vital, but knowing how to talk to them is equally crucial. Patients do not respond well to what they see as industry jargon.

For example, the term patient-centricity has been a buzzword that many patient advocacy groups say is just a label.

"In a survey we conducted of 22 advocacy groups regarding the term patient-centricity, many groups said companies declare they are patient-centric, but in reality, many are not," says Natalie. "Pharma needs to be seen as being patient-centric, so they talk about it, but when pressed for examples, many pharmaceutical companies struggled to define it."

Being aware of patient needs and working collaboratively with advocacy groups to improve the patient experience will automatically generate "patient-centric" outcomes. Creating the perception of being "patient-centric" would, therefore, be unnecessary.

"We want to empower everyone we work with to consider the patient perspective. Ultimately, that leads to a deeper understanding of patients' needs, hopes for the future, and what their expectations are around the condition or disease they are living with."

Patients are the primary focus

Although both pharma companies and patient advocacy groups aim to improve patients' quality of life, achieving that complex objective can be difficult.

Natalie says AXON starts with what they internally call PATH – Patients at the Heart.

"It's an ideology, an ethos, and a process. We explain to clients that whilst we're happy to deliver a project or help them explore an idea, our position is always – it's about the patients."

Still, not every patient has the same needs. Gathering multiple patient perspectives allows companies to understand a common thread in patients' treatment needs and individual needs. In some instances, the caregiver insight is as important to consider.

"Regardless of which perspective we're coming from, the best place to start is by talking to patient communities," says Natalie. "Doing a lot of questioning and heavy-duty listening to immerse ourselves in their world."

When working with pharma clients, AXON may suggest the need for the company to engage with a specific patient community to gain valuable insights.

> "Ideas are brought forth, we share those ideas, and we run things by the patient community. When we do so, we often end up with the right result or the right approach because we listened and asked the right questions from the beginning."

AXON also uses scientific methods to collect analytical views. They combine face-to-face interactions with ethnographic research and work with sociologists and anthropologists who analyse the data.

"Our sister agency Madano has a superb insights and intelligence team, and they do the ethnographic socioeconomic deep dive to understand and analyse, for example, what's being said in many different patient community settings," Natalie states.

The analytics and insights help identify underserved and underrepresented populations and determines who we can help and where the most significant impact can occur in the quickest possible way.

AXON's work with communities and partners

Natalie says that working directly with underrepresented groups means a lot to AXON because it touches on so many parts of the work the agency team likes to get involved in.

For example, a leading African women-led organisation working to end violence against women and girls, approached AXON to assist with sharing the results of its latest research completed in partnership with an academic institution.

The report documented how the stringent, targeted, female genital mutilation (FGM) safeguarding measures introduced since the 2014 Girl Summit are causing distress and mistrust amongst African diaspora communities in Bristol.

"The research revealed first-hand experiences of how FGM safeguarding policies are leaving families feeling racially profiled, criminalised and stigmatised, sadly undermining some of the incredible efforts and great work in the community," Natalie states.

"We helped the charity tell their story and proposed a series of recommendations that supported an inclusive and passionate narrative around FGM. We planned and executed a social media campaign, working with influential individuals who are vocal in the areas aligned with the story. The campaign inspired a sense of urgency and action and generated great coverage and exposure for our client"

AXON was also approached by a pharmaceutical company that wished to better understand the day-to-day experiences of people living with a specific visual impairment, before embarking on any work to support this community.

Fifteen leaders in various roles from different advocacy groups were brought together – representing blind, partially sighted, normal sighted, and caregivers of the visually impaired – and asked to set the agenda.

"It was beautiful because the pharmaceutical company went in with no agenda; they just wanted to learn. They wanted to know what it was like living with a particular eye disease where no cure or current treatment exists," Natalie says.

AXON not only connects companies with patient advocacy groups they also work to raise awareness of detecting diseases.

Spearheaded by an annual awareness week, the Make Sense campaign, initiated by the European Head and Neck Society (EHNS), aims to raise awareness of head and neck cancer and ultimately improve outcomes for patients with the disease. As campaign secretariat, AXON drives activities that promote education on risk factors, disease prevention and disease signs and symptoms for both patients and healthcare professionals. The campaign has also evolved to support patients' needs and highlight their lived experiences as well as urge policy makers to support access to treatments.

The programmes AXON works on are a small part of the overall plan to ensure patients are always at the centre of the healthcare discussion.

Understanding what it's like to be a patient with a particular disease to ensure the availability of the best possible treatment options means including patients in the conversation.

"Talk to people how you would want people to speak to you. It's as simple as that." Natalie emphasises. "It's having respect and earning the right to ask the questions and then be absolutely sure you listen properly to the answer."

About the interviewee

Natalie Turner, director at AXON, has 22 years' experience in the pharmaceutical and healthcare industry in a variety of roles including: product launch and life-cycle management, media strategist, directing patient-focused communication programmes, internal training and patient advocacy group relationship development, and working with not-for-profit organisations.

About AXON

AXON is a global healthcare communications firm that ignites change in healthcare. In its 20th year of developing insights-led healthcare strategy and communications activities, AXON is passionate about making a difference. We believe that at the core of every complex scientific and medical challenge there is a simple, compelling story to be told. By harnessing insights and using storytelling approaches, we can drive behavioural change and improve healthcare outcomes.

The AXON team are a passionate, smart, and curious bunch, who love learning and delivering beyond expectations. As a firm, we shake things up, interrupt the norm and explore new ways of working for the best results.

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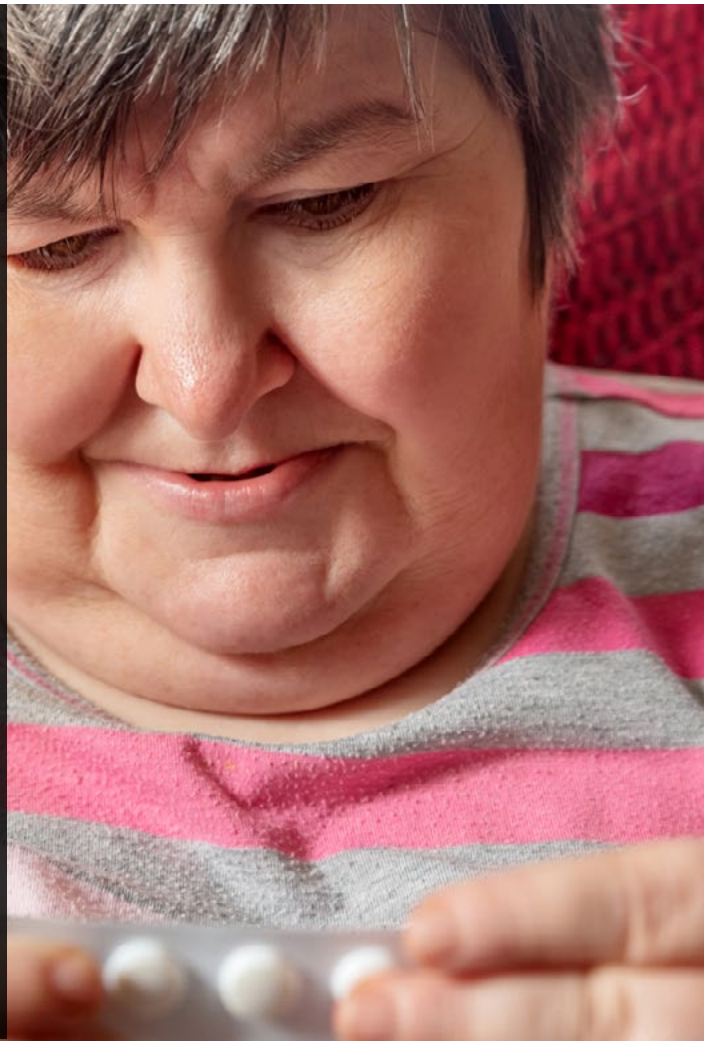
PRIME GLOBAL
The Medical Communications People

Health literacy for the intellectually disabled: is pharma doing enough?



What is health literacy and why is it important?

Health literacy (HL), defined by the CDC as the degree to which individuals have the ability to find, understand and use information and services to inform health-related decisions and actions for themselves and others, is a valuable tool against health inequalities and negative patient outcomes. Low HL is associated with poor patient activation and increases to morbidity, use of emergency services, premature death, and more.

Some patients are being left behind

We are in a HL crisis. Only 12% of US adults are health literate enough to understand and use health information effectively. HL is particularly lacking in vulnerable patient groups, thus contributing to health inequalities. Moreover, despite the concept of HL having rumbled around for decades, research and initiatives to date have largely neglected one of the groups who rely on healthcare services the most – that is, people with intellectual disabilities (IDs). ID patients are defined as those who have a significantly reduced ability to understand new or complex information and to learn and apply new skills, and the very nature of their disability can pose major challenges to expressing health needs or understanding how/why their medication should be taken.

Healthcare is a particularly pertinent matter for people with ID

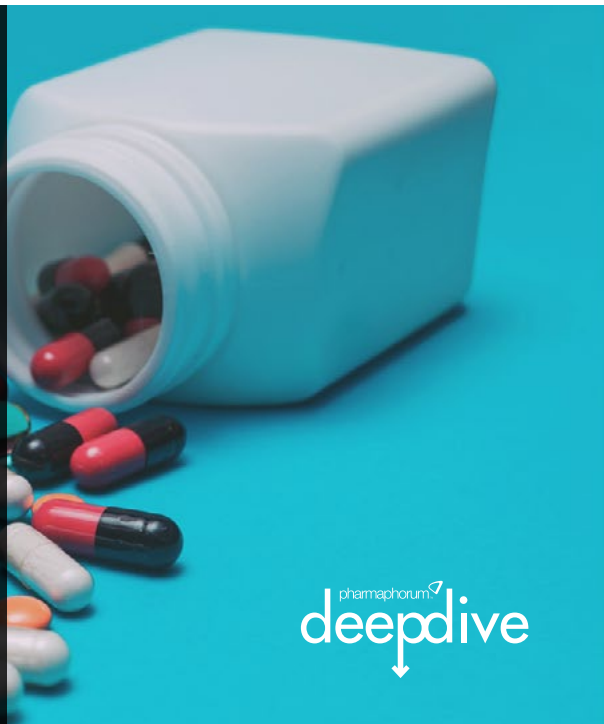
ID patients form one of the most heavily medicated groups in society. Although these patients face significant challenges due to their disability, ID presents across a spectrum, and many ID patients are capable of (and wish for) at least some degree of ability to manage and make their own choices regarding their healthcare. Thus, it is concerning that through the limited research conducted, ID patients have been shown to have insufficient understanding of their medication and be confused by or unaware of related side effects. This is despite legal requirements for all ID patients with mental capacity (the ability to make their own decisions) to be effectively informed about medication risks and also receive information in an accessible format (the latter of which 70% of GP surgeries in the UK fail to provide).

HL in ID (and how to improve it) exemplifies a blatant research and strategy gap. A specialised approach is vital, as generalised initiatives can perpetuate health inequalities rather than improve them.

As ID patients are underrepresented in HL initiatives, their HL is neglected and thus they are disproportionately affected by negative outcomes. For those with mental capacity, this is avoidable; a targeted strategic approach is urgently needed. Otherwise, compared to their non-ID counterparts, ID patients will continue to be more likely to develop preventable health problems, receive poorer care than they are entitled to, and die prematurely.

Why does this matter for pharma?

In 2018, Cathryn Gunther, vice president of Global Population Health at Merck at the time, described the importance of HL to pharma rather eloquently: “A new drug has no real value unless the person taking that drug understands what that medication is going to do for them so they can take ownership of their own care and adhere to the appropriate medication schedule. Health literacy is a big part of realising that value”.



Evidently, disregarding HL in any group, not just ID patients, would be foolish; what kind of pharma company would want their new drug to have no real value?

As ID is a diagnosis in itself, one may assume that ID patients would not be the target population for pharma companies unless said companies were developing products specifically relating to ID or the conditions of which ID is a symptom.

However, this is a fundamental misconception – *every single pharmaceutical company's work relates to intellectual disability*. This is because patients with ID, just like everyone else, are at risk of being born with or developing other conditions that these companies have a direct stake in – diabetes, cancer, etc. – therefore, to ignore ID-specific HL needs is to neglect their own target patients.

Furthermore, people with ID have a higher burden of multimorbidity and long-term conditions such as epilepsy and heart failure than the general population. It just does not make sense to ignore the need for ID-tailored HL support.

If pharma companies don't step up, their profits and patients will pay the price

Ironically, despite the fact that ID patients are particularly highly medicated, the healthcare system fails to appropriately accommodate for the fact that their disability directly impacts their understanding of health, disease, and treatment. Furthermore, ID patients often must adhere to multiple medications, and this complexity exacerbates the risk of confusion surrounding treatments. Due to these comprehension barriers, medication non-adherence is a particular concern regarding this patient group. Non-adherence also has notable commercial implications; if medicines are not taken as often as instructed, prescription refills are reduced, which leads to revenue losses for pharma. This is no small matter; each year, the US pharmaceutical industry alone loses 250 billion USD in potential revenue due to non-adherence.

Another concern surrounding inadequate understanding of medications is the risk to patient safety, something that pharma of course have a duty to protect against, and one cannot simply assume caregivers will be available to protect against this. Appropriate care is sorely lacking for huge numbers of people with ID; indeed, social care inadequacies are one of, if not the, biggest factors impeding these patients' quality of life. Therefore, one cannot guarantee that all ID patients who need support with their healthcare are receiving said support.

If pharma companies wish to meaningfully honour their commitment to patient centricity (or if such a commitment has not been made, move with the times and enjoy the financial returns), they have a responsibility to support ID patients in understanding and applying knowledge surrounding their treatments. Fortunately, the starting points are clear.

What can pharma do to help?

Lay the foundations

The first step pharma must take is conducting research into the particularities of ID patients regarding how they make health decisions, how they interact with and derive meaning from information, what matters to them, and what they need help with the most.

Then, in collaboration with ID patients, caregivers, and transdisciplinary experts, pharma must work towards appropriate conceptualisation of (and design of measurement tools for) HL in ID. With this in place, ID-specific HL research may be performed to inform the development of effective strategies and interventions.

Educate and evolve

Another key shift pharma companies must enact is moving beyond the outdated assumption that information provision and patient knowledge expansion always translates into improvements to outcomes and critical engagement with health interaction. Systematic, company-wide training for many years will be necessary to effect this shift. While moving beyond standard lay language materials to provide health information in a manner accessible to varying levels of ID (for example, via more nuanced language adaptations, development of video resources, training of HCPs in 'Teach/Show Back' practice) would be of value, we cannot stop there; research and strategies must be specifically aimed at equipping ID patients with the skills to not just understand health information, but also take appropriate health actions.

Conclusion

By investing in HL in ID, we can achieve a triple-triple win for patients, pharma, and society. The first triple win comes from clarity in conceptualisation and measurement of HL in ID; patients will be more appropriately supported in self-determination, pharma will benefit across countless therapy areas from the clarity in researching and improving HL in ID, and society will benefit from a richer evidence base through which HL in ID can be promoted in an effective, transdisciplinary manner. The second triple win comes from the resulting improvements to adherence; better patient outcomes, recovery of otherwise lost revenue for pharma, and reduced health inequality in society. The third triple win comes from the added protection to patient safety; less risk of harm to patients, more meaningful patient centricity in pharma, and reduced healthcare costs for society. Evidently, we all stand to gain from investing in the HL of ID patients.

The bottom line

ID patients face unique challenges and inequalities in HL; however, it is possible to both study and improve HL in this group. Pharma can play a valuable and transformative role in addressing low HL in ID, modernising outdated approaches to research and care and making a meaningful difference to patient lives that will be reflected in pharmaceutical business success and advancements in society.

About the author

Olivia Kersey is a Patient Strategist at the Prime Patient Centre of Excellence. Through utilising her biomedical science background and professional history in medical communications, Olivia greatly enjoys exploring how industry can meaningfully improve patient lives. Stemming in part from close personal experience with neurodevelopmental disability, intellectual disability and appropriate treatment of disabled patients are of particular interest to Olivia.

Get in touch via @PrimePEPTalks on Twitter or PatientEngagement@primeglobalpeople.com

About Prime Global

PRIME GLOBAL
The Medical Communications People

Prime Global are experts in medical communications, market access, patient insights, engagement, and recruitment. They help the world's leading biotech, pharma and healthcare companies to transform global health and patient outcomes, now and for future generations

Three views on technology improving clinical trial efficiency

Marie Emms, vice president of site and patient participation at Syneos Health, tells us technology helped increase patient enrolment, improve diversity and inclusion, and boost retention rates for clinical trials after the pandemic. Still, she says a lot needs to be done to continue this upward trajectory.



"There's an acknowledgement among companies that the paradigm of running clinical trials needs to evolve. One of the challenges within the industry is that changes take a long time to happen, but there have been some innovative solutions we've seen come through," says Marie.

Keith Fern, vice president of corporate development at Elligo Health Research, and Matt Miller, CEO of StudyKIK, agree that the evolution of modern-day clinical trial operations need to continue, and data and technology is at the forefront of that progression.

Syneos Health, a fully integrated biopharmaceutical solutions organisation, was one of the companies helping adjust the clinical trial experience during the pandemic.

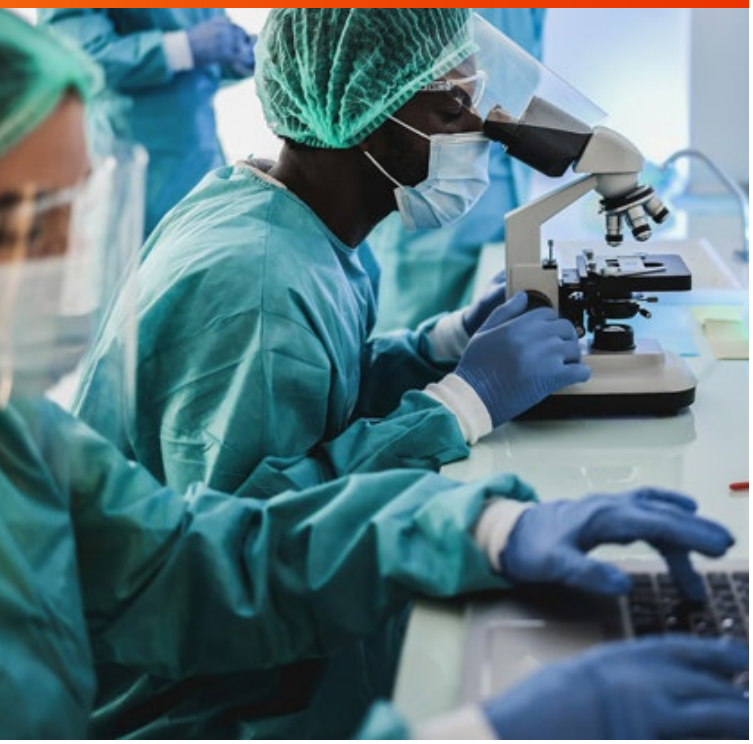
They recently acquired Illingworth and StudyKIK and partnered with Elligo Health Research intending to improve clinical research design and efficiency for patients, HCPs, and trial sites.

Each company presents solutions to enhance the complex clinical trial configuration, but the plight toward an effective trial model requires the industry to be open to feedback and necessary adjustments.

Changing the structure of clinical trials

Clinical trials typically engage 3% to 4% of the population year after year, and 85% of studies don't recruit on time or budget. Yet, the amount of time and money to bring a drug to market has grown.

"That's what has been challenging for pharma. We've utilised the same model that's broken, but it's the accepted approach," says Keith.



COVID forced companies to rethink clinical trial operations. Marie says Syneos Health looked at the commercial tools it could offer clinics to make enrolment easier. Thus, they partnered with Elligo.

Elligo's model engages healthcare clinics by using data to find patient-participants before a clinical research study begins, therefore, streamlining the recruitment process.

The company separates patient identification from enrolment and uses electronic health records to find patient-participants within its network of healthcare practices and partnerships.

"By finding patients first, the enrolment period activities are split and, therefore, the model is changed and risk within the overall study process is lessened," Keith notes.

Elligo then engages with physicians and provides the needed infrastructure to perform the study.

Using data to find participants in the clinical setting is quite valuable. Still, many patients seek information on how to volunteer for a clinical trial.

StudyKIK's platform provides patients with information on what clinical trials are, how they work, and how to join a study in their area.

The company created over 1,000 indication-specific communities and uses these platforms to distribute information.

"At first, a lot of content generated was focused on the disease states," Matt states. "We'd really try to bring value to those patients and introduce research as an option for them. One of the biggest barriers to patients joining a trial was the lack of knowledge on what a clinical trial is."

"We're now almost eight years in as a company, and we connect over 30,000 patients every month in clinical trials. Our communities reach over six million patients and growing, but there's still a lot more to do," says Matt.

Ensuring diversity and inclusion

Utilising the patient communities and clinics within those communities will ensure clinical trials better represent the diverse population of society or what the industry sometimes refers to as the "real-world population."

"We need to move research closer to healthcare and reflect companies in other industries that look to engage diverse groups of people; they don't consider anything but the real-world population," says Keith.

Marie agrees and says 10 to 15 years ago, the industry was more focused on diversity and inclusion, and different materials were created to encourage engagement.

"We would have more interaction with patients and focus groups to ensure we were using the language and the tone that they recognised and felt comfortable with," Marie says.

"Over the years patient recruitment became a commodity and wasn't seen as something that clients wanted to invest time and money. It was something that, often, clients felt they had to include, and it just dropped off the radar."

This is why the industry still has the same old issues with clients spending millions of dollars every day on studies that continue to lag in enrolment.

Including technology in the recruitment process is enormously beneficial as it can increase enrolment while improving diversity and inclusion since it expands the geographic availability of participants.

At Elligo, Keith says, the company has more diverse populations participating in trials because they practice research within healthcare in many rural and remote areas that never had access before.

"Utilising integrated technology is where we'll see significant improvements from enrolment, retention, and overall study timelines," Matt says. "We're already seeing it today."

The future of clinical trials

Technology has already changed the landscape of clinical trials, but Marie, Keith and Matt see areas that will progress and some that need refinement.

"Patient identification will be a primary focus before a trial gets the green light," Matt anticipates.

"The clinical trial design will change by introducing technology earlier from the patient access side and streamlining the process for sites to identify highly qualified patients within their database," says Matt.

Marie says improving protocol design is crucial to easing the process as well by holding patient focus groups and talking to patients ahead of protocol design.

"It's really about making sure the protocol is designed so numerous amendments don't have to be made before the first patient can be enrolled," states Marie.

Technology will continue to be an excellent asset in connecting with clinics and patients. Still, Marie says there's a tech burden that comes with using numerous platforms and having to keep track of logins.

"If a site is working on six or seven different studies, they could be working with six or seven different sponsors and CROs, each with their demands on platforms," says Marie.

To decrease the tech burden Matt recommends implementing a widely used technology already adopted by research sites to streamline the overall work process or allowing sites to recommend platforms that they prefer to use.

Unfortunately, with the use of technology the need for data security becomes ever more critical, Marie says.

"Data breaches are going to be particularly concerning as we continue to move forward. We need ensure that we maximise data security as much as we can," Marie says.

Navigating the elements that accompany increased use of technology will always be present. Still, each company's ultimate focus is easing the clinical trial experience to secure the treatments patients need to optimise their journey.

"The beauty of the industry today is the rapid progression. Without the expedited approach to research, many people would be waiting longer than needed for therapies that are currently being developed," says Matt.

"The motivation for us all is to streamline this process to reduce costs and timelines while ensuring patients get therapies that will best support them."

About the interviewees

Marie Emms, head of Patient Engagement at Syneos Health, has 25 years of experience in healthcare communications, spanning public relations, medical education and patient engagement. She has spent the past 20 years focused solely in patient engagement, supporting pharma and biotech clients. She specialises in the development and execution of strategic integrated communications programs to support sites and their patients through the clinical trial journey, from initial awareness, consent through to compliance and adherence. Marie has worked on a wide variety of therapeutic areas across neonatal, paediatric, adult and geriatric studies. Marie is responsible for developing engagement programs to support patient recruitment and retention globally across all Syneos Health studies.

Keith Fern joins Elligo with a decade of proven leadership in the CRO industry. As executive vice president and general manager of Worldwide Clinical Trials Early Phase Services, Fern managed all commercial and day-to-day operations of the company's 300-bed clinic and state-of-the-art bioanalytical laboratory, growing EBITDA from 1 percent to 13 percent. Fern invested in automation and expanded service offerings in both the lab and clinic, positioning the company for future growth. Before taking on the role as general manager, Fern was senior vice president of business development, expanding the sales team and establishing key partnerships to meet the needs of sponsors. He joined Worldwide Clinical Trials Early Phase Services as chief financial officer, leading the $110 million acquisition of the former company, CEDRA, by Worldwide. Fern began his career with Bank of America Securities, working in corporate and investment banking. He holds a Master of Business Administration from The University of Texas at Austin

Matt Miller is the Co-Founder and CEO of StudyKIK, a patient recruitment and retention technology company supporting clinical trial participants from start to finish of their trial journey. Matt has led the company to support thousands of clinical trials over the past 7.5 years in all areas of research across 32 countries. Prior to founding StudyKIK, Matt had spent time in the digital marketing and SEO industry. Additionally, he has spent time working at a contract research organization which owned early and late stage clinical units.

About Syneos Health

Syneos Health® (Nasdaq: SYNH) is the only fully integrated biopharmaceutical solutions organisation. The company, including a contract research organisation (CRO) and contract commercial organisation (CCO), is purpose-built to accelerate customer performance to address modern market realities. Created through the merger of two industry-leading companies – INC Research and inVentiv Health – it brings together approximately 27,000 clinical and commercial minds to help its biopharmaceutical customers shorten the distance from lab to life.

How biomarkers and bioanalytics are changing R&D

Biomarkers have been causing a paradigm shift in how HCPs treat patients for some time – now they are bringing the pharma industry a new gold standard for clinical research.

Thomas Turi, chief scientific officer at Nexelis, tells us how a biomarker-led R&D approach is transforming drug development in disease areas like non-alcoholic steatohepatitis (NASH) and cancer, and provides best-practice tips for companies looking to harness this approach.

Biomarkers have already guided our understanding of several cancers and led to the development of several precision medicines. Recent approvals in highly heterogenous tumours, such as non-small cell lung cancer, are now targeting smaller patient populations – representing just a single-digit percentage of patients with aggressive cancers and poor prognosis.

Unsurprisingly, some major pharmaceutical companies are in the process of revamping their entire R&D approach to focus on biomarkers, and see such technology as the key to discovering new, breakthrough treatment options that also have the potential to transform care by allowing patients to undergo less exhaustive testing.

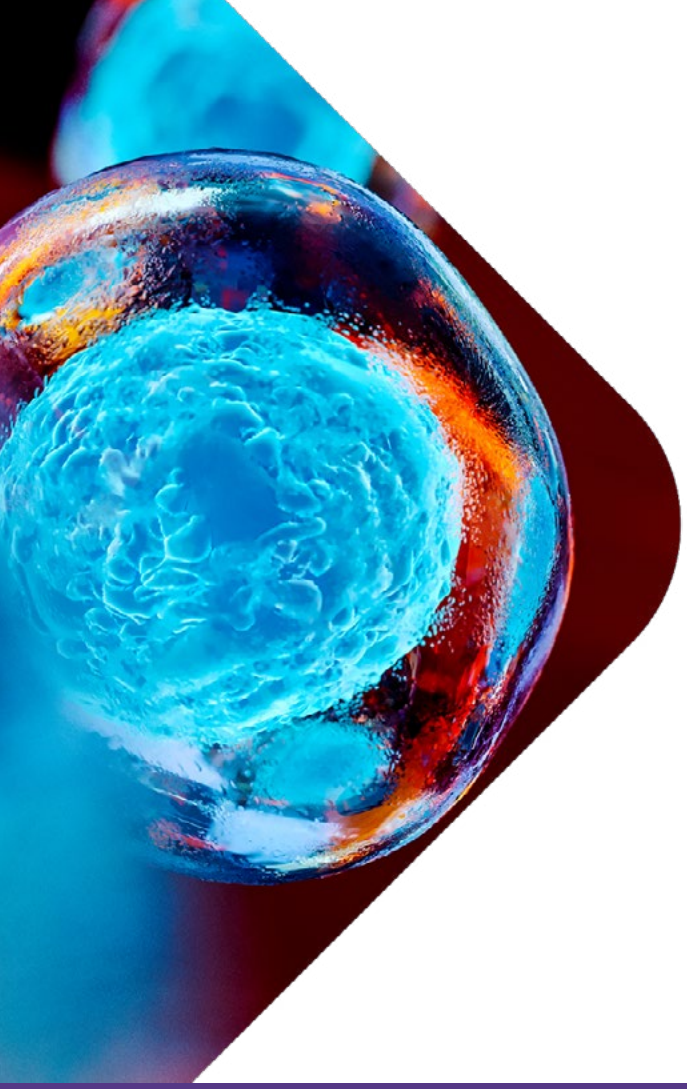
Developing a biomarker strategy for clinical trials

One key piece of advice for pharma companies moving in this direction is to involve CRO biomarker strategists early on, to help determine what the target is, and the potential mechanism associated with it, which will ultimately allow for a better trial experience for both companies and patients.

"As biomarker analysis becomes more complicated, it's important to find the balance between selecting appropriate endpoints to inform decision making and sparing patients the burden of a lot of repeated or invasive sampling procedures", Turi says. "Partnering with companies that specialise in biomarker testing at the early stages of trial design can greatly improve efficiency and efficacy, thus sparing patients extensive testing".

Biomarkers exist for different purposes, and strategists can identify the appropriate biomarkers that will help patients and HCPs choose beneficial treatment options.

"One type of biomarker can help stratify patient populations or determine which are the most appropriate patients to treat. This is especially helpful in a heterogeneous disease, where it allows clinicians the ability to better define the disease population", says Turi.



"Another type of biomarker can stage patients accordingly within their disease. While other biomarkers may indicate the likelihood of disease progression", Turi added.

"Despite the wealth of information and data on biomarkers, many disease indications lack informative markers. Thus, many sponsors undertake exploratory biomarker discovery studies. One of the big challenges of multiplex-type approaches and biomarker discovery or identification is overfitting of data and making sure you haven't biased your analysis", says Turi. "In addition, having a robust strategy where biomarker discovery, confirmation and validation are linked is essential to yield informative markers".

Identifying a set of biomarkers from a given study is quite easy and can help identify candidate biomarkers, but what's important is finding an informative biomarker by going through the qualification and validation of it.

Partnering with an experienced clinical research organisation (CRO) and clinical reference laboratory (CRL) is critical in biomarker testing as well. Turi states that it's beneficial to utilise CRO and CRL knowledge working in specific disease areas as well as their scientific and technical experts, who can provide insights that will inevitably advance programmes.

"A CRL partner or laboratory will be a thought partner as you continue to progress your development programme – someone who can engage early and has the resources and expertise in bioanalytics as well as the reach to support you throughout a small, focused phase 1 study to a global clinical trial."

All successful partnerships start with clear and open communication and transparency at the programmatic level as well as the scientific and technical levels. A fruitful research partnership is key to ensuring patients get the best results possible, which is why strategists are so important in biomarker-led R&D programmes.

"Biomarker strategy is essential when considering the idea of sparing patients from the trials and tribulations of multiplex, multivariant analyses throughout phase 1."

Taking a patient-centric approach to biomarkers

Some companies take a shotgun approach to analyses, which can overburden patients by trying to include many kinds of markers, but it's important to have the qualities, resources, and expertise needed for a focused biomarker-driven research programme to improve the overall experience for patients and researchers.

Turi states, "You need to be patient centric – the individual at the centre of the trial – and think about their journey through their disease. Study subjects try to help themselves and the broader patient population by volunteering for a clinical trial. We should never lose sight of who actually drives these trials – the patient".

Currently, researchers are thinking of how biomarker-led approaches to clinical research can support the development of new treatment options, especially in heterogenous diseases such as NASH and non-alcoholic fatty liver disease (NAFLD).

Non-alcoholic steatohepatitis, better known as NASH, is a type of non-alcoholic fatty liver disease and a condition that causes inflammation and accumulation of fat and fibrous (scar) tissue in the liver. About 3% to 12% of adults in the United States have NASH. In such a prevalent disease, having prognostic markers or risk stratification markers would be useful.

"We don't have a true understanding of the underlying molecular mechanisms that contribute to NAFLD and subsequently NASH. So, we don't have a true aetiology of it".

"Currently, the best recognised markers for identifying NAFLD and NASH is through liver biopsy, a highly invasive procedure that really is based on a pathologist's interpretation," Turi states. "In order to stage one's disease, serial biopsy may be necessary, which attributes greater burden and potential risk to patients".

"There are some blood-based biomarkers used for staging or identifying NASH that require analysing multiple biomarkers and then translation into a score. However, these assay may not always provide a good indication of where a patient truly is in their progression of the disease or whether a patient will progress in their disease", Turi adds.

Therefore, creation of therapies for NASH remains a challenge because the current understanding of the pathogenesis of NASH, while much improved, remains unclear. A biomarker-led research approach could be critical for the potential discovery and development of a NASH treatment.

Supporting the future development of biomarkers

Although there's no general database of informative biomarkers at the moment, many companies are working to analyse them with appropriate tools and technologies that identify potential candidates.

"The NIH, the Critical Path Initiative, and many others have provided guidance on how one can take steps not only to identify biomarkers, but also to provide appropriate context on their use. It's a long process to generate the evidence to demonstrate that it truly is an informative biomarker that can be used for decision making".

Turi notes that, ultimately, more biomarker-led research will be incorporated within trial settings in the future to improve the overall clinical strategy. Biomarkers will be used to select patients who are most likely to respond to treatment, and more researchers will make biomarker-guided decisions and use biomarker selection, and biomarkers will become broader and more prevalent in treating disease areas outside of oncology.

"Biomarker scientists and translational scientists are becoming more critical and vital to the success of drug development programmes. As people come up with more novel trial designs – adaptive-type trials, where you can change approaches and treatments within a given trial, move patients from one arm of the trial to the next – it will become more evidence-based. Biomarkers will be central in those decision-making processes."

About the interviewee

Thomas Turi, Ph.D. is part of the executive leadership at Nexelis, where he is chief scientific officer. He has 25 years' pharmaceutical and contract research leadership experience, during which two of Dr Turi's accomplishments were establishing the Biomarker Center of Excellence for Covance and serving as senior director of translation biomarkers and mechanistic biology at Pfizer. He has also previously served on the Board of Directors for Cellcarta (formerly Caprion Proteomics) and led several external partnerships, including those with Rules Based Medicine, Celera, Incyte and Affymetrix. He has also served on grant and programme project review boards for NASA's Section for Biotechnology and Tissue Engineering.

About Nexelis

nexelis

With unrivaled expertise in immunology, five operating sites in North America and Europe, and a translational offer of services covering the needs of the pharmaceutical industry from the lead selection to the late clinical stage, Nexelis is a leading provider of assay development and advanced laboratory testing services in the infectious, metabolic and oncologic fields. Its versatile team of scientists, working with state-of-the-art technology platforms, were instrumental in the development, qualification, validation, and large-scale sample testing of assays that supported the FDA filing of almost 100 new molecular entities, including blockbuster vaccines and biologics, anti-viral drugs, immunotherapy, gene and cell therapy products.

CERTARA®

Leveraging real-world data to meet payers' needs

Payers are becoming savvier in accepting real-world data. Certara's Lee Stern and Shawn Bates discuss the challenges and how pharma can use real-world data to meet payers' needs.

For years, clinical trial data was king. But as healthcare costs continue to be squeezed, there is pressure on pharmaceutical companies to actually deliver on clinical trial efficacy in the real world. It's no small feat in the tightly controlled and "biased" environment of randomised controlled trials. This is where a growing plethora of real-world effectiveness data comes in.

From electronic health records to medical claims information, real-world data (RWD) is increasingly being used to supplement the data generated by clinical trials. Just in September, the US Food and Drug Administration released guidance as part of a framework for evaluating real-world evidence to support the approval of a new indication for an already-approved drug. This comes as more pharma companies include RWD in their regulatory applications.

But it's not just regulatory applications where RWD can have an impact. As companies are increasingly forced to persuade budget-holders of a drug's cost-effectiveness to ensure post-approval market access, RWD can play a central role.

Lee Stern, VP and head of global operations of the Evidence and Access Division of Certara, says the industry is noticing the shortcomings of traditional sources of clinical trial data.

"We'll never have years and years and years of clinical trial data to show overall survival in, say, oncology. So, we need to leverage real-world data to extrapolate those findings from a trial into the real world and into a longer-term survival situation to reduce payer's uncertainties about their eventual spend."

It's a similar position with rare diseases, she says, where there are often not enough patients to even recruit into clinical trials.

The rise of RWD

Enter RWD to fill critical evidence gaps. Increasingly, information from electronic health records, claims data, patient registries, inpatient or outpatient data, scientific literature and even patient surveys are being accepted by both regulatory and health technology assessment agencies as valuable sources of RWD to supplement clinical trial findings.

On top of that, different countries have different data sets. For instance, German sickness funds, the National Cancer Registration and Analysis Service (NCRAS) run by Public Health England in the UK, and the French administrative healthcare database SNDS, which covers around 99% of the population, can be specifically analyzed to understand practice patterns, resource utilisation and outcomes.

"Now I can do an analysis of a patient population even before my client's drug has hit the market to demonstrate actual resource use and treatment patterns," says Stern. "[If I leverage SNDS in France], I think that's a pretty powerful bit of evidence to show when doing a French reimbursement submission."

The benefits of RWD are vast, including establishing unmet need, particularly for rare diseases, how drugs are used, how a disease develops and what the costs associated with the patient journey are. RWD can also provide a comparative analysis of outcomes and costs between different drugs for the same indication.

Ultimately, explains Stern, RWD can provide a better understanding of a disease and its progression, and the healthcare and costs around it, particularly over the long-term. That's something payers are becoming more interested in, which makes it all the more relevant for pharma to include in reimbursement packages, she says.

Oncology is a case in point. "I think payers think that the standards of care or generic treatments or basic chemotherapies are what's being used, but in reality, when you look at the RWD, there's a lot of very expensive off-label drugs being used because there's just no other option," Stern says. "Actually, when you start to compare what a new drug could be in terms of cost to the treatment pattern that exists, which can be very expensive, I think that's a bit of an eye opener for some payers."

Or take, for instance, Novartis' CAR-T therapy Kymriah, used to treat a type of lymphoma and leukaemia. In post-marketing studies, the drugmaker found that in the real world the drug not only had comparable efficacy but a better safety profile than in clinical trials. According to the data, this resulted in reduced use of medications to treat the side effects reported in clinical trials, meaning better outcomes for patients and reduced costs for the associated healthcare. Given the high costs and high competition in this drug class, any associated cost-savings is a market access plus.

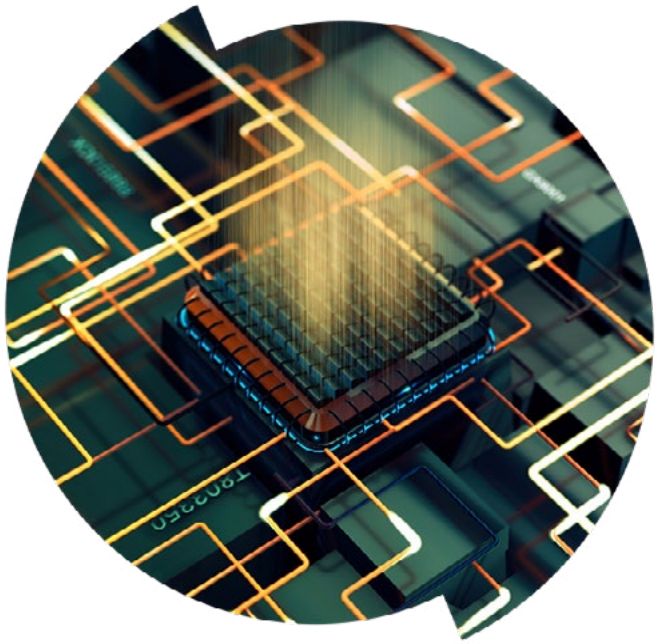
It's for these outcomes and cost-savings reasons that now puts RWD on payers' radars, says Stern. "They're becoming more up to speed on the types of data out there and how they can be applied to their benefit in terms of reducing uncertainty around a disease and a drug and the risk when approving products."

Shawn Bates, Certara's VP of US sales and business development, agrees, adding that payers increasingly want outcomes data and comparative analysis based on what is happening in the real world to help them make reimbursement decisions. "Industry really is starting to have to go in that direction, because payers have substantial influence in the market space."

The challenges of RWD

But there are challenges, Bates notes. For starters, RWD is complex and uncontrolled data. "It's a mix of different data from varying sources and the inconsistency in how that data is reported makes it difficult to consolidate and place value around it to say this is what outcomes look like in the real world," he says.

Pharma would ideally prefer a one-stop-shop data source incorporating claims, outcomes, laboratory data – the works, says Stern, "[but] what I have to tell our clients time and time again is there is no holy grail data set. If there was, you'd know of it already". She says a generic large database can sometimes be the equivalent of looking for a needle in a haystack – trying to find the right patients. The better option is to identify the exact research question and market access issue and then design a study using the right source or even sources of data.



But that, of course, presents its own challenges. Sometimes the data just isn't detailed enough to answer the questions pharma is asking. "I feel our pharma clients get disappointed that when they look under the hood, that data is missing," Stern says. "I think it takes a lot of very specific understanding of what is in the data and what's not in the data in order to be sure you're going to answer your research question."

Furthermore, companies should keep in mind the difference between efficacy and effectiveness when interpreting the outcomes seen in RWD – they might be quite different from those results found in the trials given the totality of the patient populations that are actually being given the product. Stern says this can be especially common in the first year of a drug's release when it's often being prescribed to, for example, the sickest patients. "This isn't a function of the drug not working. It's just a factor of the different patient populations being treated."

She suggests one way to hedge this is to use a simulation model, combining some RWD and some clinical trial data, to estimate a range of possible outcomes on how the drug might react in the real world.

Perhaps one of the most frustrating challenges is making the RWD relevant for the particular payer pharma is targeting. In many cases, says Bates, this is at the local level. For instance, what's the local patient population? What are the local payer needs? "If you don't have localized real-world data specific to that payer, and you try to utilise national or global level data, then your value message will not resonate as powerfully as you might need it to with the payer."

Sometimes, says Bates, that local data isn't even there but using RWD that's similar to the target patient population ensures the message is as tailored as it can be for the payer and will resonate more.

Leveraging RWD

Challenges aside, the key to leveraging RWD to create impact with payers is to communicate its value. But keep the message simple, says Stern. "It starts with a very simple table with the value proposition being the clinical, economic and humanistic unmet needs in the disease and then aligning that in the next column with how the drug is going to fulfil those unmet needs and then the data to support what you're trying to say becomes more clear and focused.

Stern gives the example of diabetes, saying that often the message highlighting unmet need will be: 'There are 64 million people with type 2 diabetes'. But a new drug won't solve that direct problem, she notes. "A more appropriate thing to say is: 'There are X number of people with diabetes who have tried two therapies who remain uncontrolled and are costing the healthcare system X amount of dollars'. With that level of specificity, I can then design a RWD study to answer that unmet need... and that's going to help my value story... I want to make it very crystal clear and not just throw all the data at the payer and see what sticks."

This is where technology can help communicate the value. In the past, value messages were presented as simple Word or PowerPoint documents, but Stern says app-based formats are now changing how pharma can communicate with payers and other stakeholders.

With app-based formats, you can choose the audience you're talking to and the question you want to ask, with the added ability to dig deeper into the RWD, she says. "I think these digital platforms are helping us to combine all the evidence into not just a blob of information that's unruly but something that's very navigational and very easy to support what you're trying to say."

"What this means, is the message is no longer getting lost in a 140-slide presentation, an unwieldy excel calculation model, or a number of pdf documents that you are trying to use with the stakeholder all at the same time," says Bates.

"These digital engagement platforms and apps can also provide clarity and an opportunity for remote engagement, which is particularly important when reaching out to local payers who won't necessarily have a field force representative walking them through the complex information and data."

In addition, there is the ability to customise and localise the data instantly with each interaction, while two-way engagement and analytics highlight what those payers find impactful. "That engagement with payers probably only occurs once or maybe twice a year and the outcomes of those limited interactions are worth millions if not hundreds of millions of dollars. Being able to clearly deliver that message but tailor it specifically to their needs and their specific patient population is what will make the engagement resonate and ultimately impact a therapy's access."

Communicating value in this way can be done now but, moving forward, Stern says the industry needs to consider leveraging RWD from the get-go. She believes integrated drug development – where pharma integrates RWD, market access and the payer point of view into the clinical trials process – strengthens both the drug and the value story. "If that information can be brought to the clinical team in the very early stages, then at the end of the day, we'll have information the payers want to see; we'll have end points that might include resource use, rehospitalisations, disease progression." It makes communicating value for market access that much easier.

The RWD opportunity

The opportunity then to leverage RWD is huge. Indeed, just using RWD to help provide payers with what they want to see from pharma companies, has the potential to have a substantial impact on the market access prospects for new drugs and indications.

Yet, as Bates notes, many pharma companies are still missing an opportunity when it comes to targeting and impacting payers. "Less than 9% of digital and traditional marketing budgets go towards payers. It's very small … most marketing is still targeted primarily at healthcare professionals."

While some companies are starting to up their game, particularly those in the US and Japan, Bates says there needs to be a paradigm shift. He suggests companies triple or even increase fivefold the proportion of marketing budgets directed at payers.

"Payers have significant influence and impact on HCP decisions," he explains. "They control your access, which controls your revenue. You want them to understand how to prescribe the therapy and what outcomes to expect and why your product is comparatively more valuable to a particular patient population than somebody else's."

RWD is the magic ingredient. The opportunity is to communicate the information in the right way.

If not, the consequences are stark, as Bates notes: "If companies can not do that effectively, a lack of access will significantly stunt a product's utilization and as a result a manufacturer's revenues."

In the shifting pharma landscape, RWD is not only king – it's becoming a game-changer.

About the interviewees

Lee Stern is vice president and the Global Head of Consulting Operations at Certara. She is involved in all global operations out of the Certara Evidence and Access Division. In her 20+ years of experience, she has been involved in hundreds of health economic models, dossier submissions, evidence generation projects and strategic product launches. Prior to joining Certara, Lee earned a B.A. degree in Neuroscience from The University of Pennsylvania and an MS in Clinical Nutrition from New York University. After obtaining her graduate degree, Lee served as the primary nutritionist in a 28-physician practice, closely affiliated with the NYU Langone Medical Center.

Shawn Bates joined Certara in summer 2020 as vice president of US Sales & Business Development for our BaseCase stakeholder engagement platform after gaining over 20 years' experience working across the pharmaceutical and medical device industries. Having held a variety of senior leadership positions, his expertise lies in stakeholder engagement and management in the market access and medical affairs spaces.

About Certara

Certara accelerates medicines using biosimulation software and technology to transform traditional drug discovery and development. Its clients include more than 1,650 global biopharmaceutical companies, leading academic institutions, and key regulatory agencies across 61 countries

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ENVISION PHARMA GROUP

Time to take patient engagement to the next level

Patient engagement in pharma is evolving, with some companies doing it better than others. Envision Pharma Group's Dawn Lobban and Sarah Avent discuss the need to engage with patients at every level for holistic inclusion of the patient voice in order for pharma to become truly patient-centric.

The Covid pandemic has sparked people's interest in science and medicine and empowered the public to make important health choices. It's also shone a light on the need to provide accurate and timely information and to engage the public at every point in the medicine development lifecycle.

More than anything, however, it highlights the importance of patient-engagement more generally in medicine development, a concept the pharmaceutical industry has been talking about for years. Says Dawn Lobban: "We know that clearly patients are experts at living with the disease, and if we want to understand what it's like to live with a disease, then they are the people who can provide those insights."

Indeed, there is growing recognition of the importance and value of patient engagement in medicine development. Not only can it benefit patients by better addressing unmet needs and improving outcomes, but it makes financial sense. For instance, a 2017 study by a team, including researchers from Janssen, Duke University and the Tufts Center for the Study of Drug Development, among others, found that compared with an investment of $100,000 in patient engagement, the increases in net present value and expected net present value (ENPV) could exceed 500-fold the investment, with the ENPV increase equivalent to accelerating a pre-phase 2 product launch by two and a half years[1].

Regulatory changes are also shifting the needle, with companies needing to demonstrate they are addressing the real unmet needs of patients. Meanwhile, various initiatives are pushing the cause, such as the not-for-profit Patient Focused Medicines Development (PFMD), which brings stakeholders together and provides processes and toolkits to support pharma. Training courses for patient advocates, like EUPATI and cancer network WECAN, have also played a role.

For these reasons, Sarah Avent says the industry is in the process of a massive sea change where the patient voice is becoming prioritised. Many companies have gone beyond talking and there are now numerous examples of effective patient engagement initiatives. But some companies are engaging better than others, she adds, and those that do it well look across all levels, from a cultural level through to an operational and patient-engagement level. "Everyone's got a willingness to put patients at the heart of what they're doing, and seeing others do this through the publication of their experiences is really going to help internal learning and drive best practice across the industry."

This is just the start of the sea change. Now it's time for the whole industry to really embrace patient-centricity and patient engagement at all levels, and make medicines development really work for all stakeholders. Many companies recognise the value of patient engagement, says Lobban. "Now they're asking themselves more questions about the how."

Engagement at a cultural level

The first step is a cultural shift. That is, developing a patient-centric culture embedded within the fabric of the organisation, driven from the top down and bottom up. And that means leadership buy-in to a patient-centric approach. Avent says more pharma leaders are putting patient-centricity at the heart of the company. "We're hearing from some pharma that patient engagement and voice is within their corporate strategic priorities for the year coming up... People have talked about it a lot but now we're really seeing people walking the talk."

But Avent warns, this is not a tick-box exercise nor is it about developing materials internally and then getting them rubber stamped by a patient group. "People need to be doing this for the right reasons. It's not just one individual's responsibility to be more patient-centric, it's everybody's."

And that means having the resources, the processes, infrastructure and internal communication in place. "It's making sure you've got your advocates at the top," explains Avent, who works with pharma using 90TEN's EMBED model which combines the agency's Feel-Think-DoTM behavioural science approach with organisational change principles from experts such as Dr John Kotter. "Then you make sure you've got champions throughout the organisation understanding what it is that people want to be moving towards and measuring it and then not just seeing it as a one-off initiative but an approach that is integral to the fabric of the organisation."

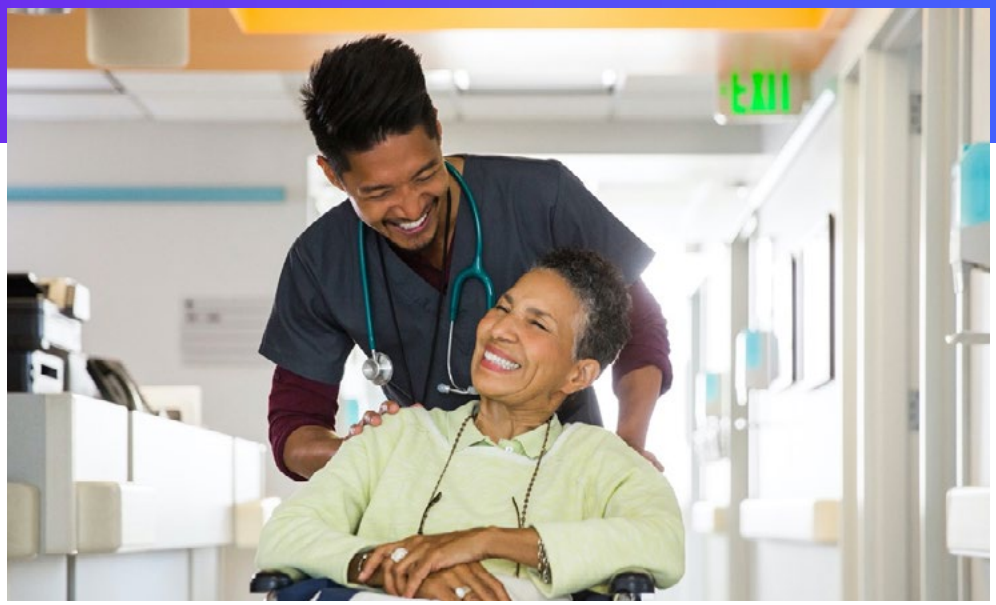
It's also about ensuring people within the company feel confident they can engage with patients compliantly, she adds. That might involve training and learning from other companies that are doing patient-engagement well. It's entirely possible, she says, but the support needs to come from the top down across the whole organisation.

This change-management-style approach results in new ways of operating that intrinsically have the patient in mind. For instance, inviting compliance teams to meetings with patients to hear first-hand the patient voice, finance teams making sure patients and patient groups get paid quickly, HR including value-based questions around patient-centricity in recruitment packs, manufacturing teams understanding how to put patient information leaflets together, and annual reports that balance commercial drivers with patient access to medicines and how many lives have been changed.

"For too long, patient engagement has sat in a silo in pharma companies driven by a separate team," says Lobban. "When we think about true patient engagement throughout the medicine lifecycle, this actually needs to be an integral part of every function and every person's role within that function. The best way to make this happen is combing a top-down and bottom-up approach – both leadership and followership are critical to success".

Engagement at an operational level

Furthermore, a patient-centric culture is about treating patients as partners over the long-term, Lobban says. “It needs to be a partnership based on mutual goals and mutual benefits. There needs to be tools and guidance developed both for patients to get involved but also for pharma in terms of how to involve patients.” PFMD, EUPATI and WECAN are already spearheading this, she adds.



The strategy for long-term patient engagement and partnership, Lobban believes, is to tap into the patient voice early in medicine development. “Too often we see pharma companies thinking about the patient as they get towards launch and thinking about patients more like an audience than a partner.”

That said, there are companies already involving patients in various elements such as how to make clinical trial protocols more patient friendly or how to improve recruitment. But Lobban says getting patients involved even earlier than this, during the designing of the research question ahead of developing the medicine, is a relatively untapped area for pharma. Doing this can more likely lead to meeting a real need, an important consideration for drug approval and market access. Although, she adds, it’s imperative patients are then involved as partners all the way through the development process.

Another untapped area of patient engagement is within scientific literature and publications. “For too long, the patient voice has just not been heard in the published peer-reviewed literature,” says Lobban. “We see a lot of market research, qualitative research, focus groups, but then too often those insights are taken and actually published by scientists and healthcare professionals and that patient voice, which was obviously present in the insight, is lost in the publication.”

Encouraging patients to author publications ensures the patient perspective is accurately reflected in the scientific literature, unmet needs are prioritised in research, and publications are more relevant for and accessible to a patient audience. Given the public's growing interest in their own health and in science since the pandemic, having a patient voice in publications can bridge the gap between scientific literature and the public, Lobban explains. "The more we can bring the two together, the better to empower people to take responsibility and to encourage shared decision making."

More patient authorship can be achieved through education and training – highlighting what it means to be an author and how to get published – and encouraging more patients to become authors, as well as ensuring the opportunity exists for them. The interest is there and Lobban says Envision is advocating tracking of the patient author to follow the increase of this trend.

She dismisses the compliance objection, saying there is no compliance reason why patients shouldn't be authors if they, like healthcare professionals, fulfil the authorship criteria. "I think there also needs to be education within pharma companies around the fact that patients can and should be authors where it's appropriate... The more this happens, the more it will happen because people will see other colleagues doing it."

Another way to bridge the gap between scientific literature and the public is the introduction of plain language summaries, which makes the complex scientific literature understandable and accessible to the broader lay audience, Lobban says. Most pharma companies are keen to develop these for their publications and various recommendations and guidelines are in development, which will help address the 'how's of doing this. It's an important move forward, she adds, as this will help empower patients and the public to become part of the conversation.

Engagement at the patient level

Indeed, incorporating patients into the conversation and tapping into the patient voice is key for better communications. When patients are engaged and their insights are fed into the development lifecycle and materials are co-created, it won't be just the medicines that are better but the resulting communications will also resonate better with patients.

But it's important the right patients – that is, the target audience – are engaged and listened to, and that companies are objective, says Avent. "It's making sure that even if people have got different views to you, or different values, or different belief systems, that they are all listened to and respected. Then we work with these communities to co-create a programme that's going to resonate and address their specific needs."

But for true patient-centricity it is also recognising that there is no one-size-fits all, she adds. Consider the differences between older people and the younger generation, for instance. The former will be more of the mindset that the doctor knows best while the latter are more proactive with their own health, more information seeking, and want that information at their fingertips. These two audiences are going to engage in different ways.

It's therefore important, says Avent, that pharma engages with a wide range of different audiences, including different ages, religions, cultures, disabilities, and socio-economic situations. "Diversity of patient voice is really important – just making sure we're engaging with real patients and finding them in different ways and making sure there's a lot of diversity in the views and opinions that are being brought into the organisation."

Ultimately, engaging patients means that patients are more engaged with their own health. Avent gives the example of HIV. Thirty years ago, an appointment with a GP would be a discussion about viral load and CD4 count and treatments. "Whereas now, because you can live a very long and normal and healthy life with HIV, the appointment now should be less focused on these things and more on how HIV might impact your long-term health, mental health, co-morbidities, side effects of HIV or the treatments."

She says Envision's Hep C, Ki? campaign with Gilead is a case in point and is a good example of shared decision making. Here it's not just about a tool, handout, or resource but about encouraging patients and healthcare professionals to have the right conversations in a language and way that resonates with the patients. "This is about pharma making sure they are supporting people to have these better conversations."

Engaging patients for better healthcare

Pharma knows it can't design a product for patients without involving them. But moving forward, how pharma engages with patients and incorporates the patient voice needs to be richer and deeper.

It's about engaging with patients in a way where they are central to the medicines-development process so that patient needs are met and outcomes improved. It's about empowering patients, making them proactive participants in their healthcare, from the early stage of drug development right through to the healthcare appointment in the doctor's office.

Many companies are making headway, focusing on engagement across the cultural, operational and patient levels. But a more holistic approach across the industry, and learning from each other, will transform healthcare and its delivery and make pharma truly patient-centric.

This is where healthcare is moving to and it's a job that shouldn't just be left to patient groups and healthcare providers, says Avent. "I do think there is a role to play with everyone together collectively supporting patients."

For pharma moving forward, full patient engagement is everything. Pharma has the power and potential to help drive this change and, if harnessed in the right way, the industry can bring much to the future of patient health.

About the interviewees

Dawn Lobban, global lead, Patient Partnerships, Envision the Patient Part of the Envision Pharma Group. With a strong background in medical affairs, Dawn is particularly interested in optimal patient involvement in medicine development. Through active participation in key working groups, including ISMPP (International Society for Medical Publication Professionals) and PFMD (Patients Focused Medicine development), Dawn works hard to explore effective ways to ensure effective patient engagement. Her research and opinions are published in a number of abstracts and articles.

Sarah Avent, associate director at 90TEN, part of the Envision Pharma Group. Sarah has more than 15 years' experience working in healthcare communications across the NHS and within communications agencies. 90TEN's patient engagement lead, Sarah has extensive experience in managing patient organisation relations and communication to patient audiences. Having led award-winning campaigns co-created with community group organisations, healthcare professionals, and pharma, she is passionate about integrating the patient voice into industry work wherever feasibly possible and always puts the needs of patients first.

About the company

Founded in 2001, Envision Pharma Group is a global, innovative technology and scientific communications company serving pharmaceutical, biotechnology, and medical device companies. Envision is a leading provider of evidence-based communication services and industry-leading technology solutions (iEnvision) that have applicability across many areas of medical affairs and related functional responsibility. Envision Pharma Group provides services and technology solutions to more than 90 companies, including all of the top 20 pharmaceutical companies.

Envision has 20 offices: six in the United Kingdom – Bishop's Stortford, Glasgow, Horsham, London, Wilmslow, and Alderley Edge; one in Serbia – Subotica; one in Hungary – Szeged; one in Coimbra, Portugal, nine in the United States – Fairfield and Glastonbury, CT, Philadelphia and Wyomissing, PA, Warren, NJ, Boston and Melrose, MA, Powell, OH, Pasadena, CA; and two in the Asia-Pacific region – Tokyo and Sydney. The company employs 1100+ team members, including over 250 highly qualified and experienced in-house medical writers, and 200 technology solutions team members who provide software development and customer support. To find out more, visit www.envisionpharmagroup.com.

bruntwood SciTech

The booming life sciences sector needs dynamic real estate support

Bruntwood SciTech is the UK's leading property provider dedicated to driving the growth of the science and technology sector.

UK life sciences are buoyant with the success of the COVID-19 vaccine programme and a stream of innovation and investment marking it out for a starring role in the UK's economic future.

The government has pledged £1 billion of funding for promising initiatives that tackle the biggest healthcare challenges of our generation and private investment is running at record levels.

The growth is accelerating across the spectrum with start-ups and established businesses both prospering, generating a huge demand for high-quality laboratory and office space. Companies are seeking environments where collective dynamics can energise the sector even further.

Life sciences have a collaborative element with open scientific innovation providing a springboard for discovery and development across drugs, diagnostics and digital. The spirit of co-operation that shone through the pandemic is the essence of healthcare's day-to-day and will drive future prosperity and patient benefit.

Bruntwood SciTech, which is at the spearhead of progressive life sciences real estate, is in tune with the ethos and has developed a major, national strategy to fulfil the needs of an industry central to the government's recovery plan and levelling-up agenda.

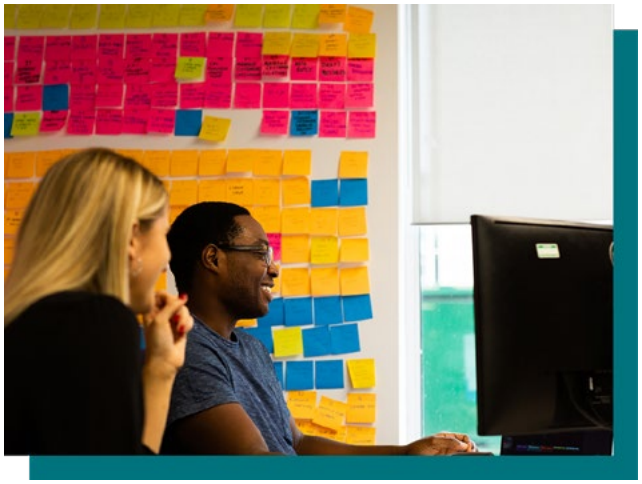
A pipeline of 40,000 high value jobs

The company's ambitious strategy, built on 40 years' experience, will invest £2 billion and create 40,000 high value jobs in science and technology over the next decade with its portfolio of 500 companies is expected to expand to 1,500 over the next five years.

Exciting developments are on track across the UK, in Manchester, Cheshire, Liverpool, Birmingham, Cambridge and Oxford to develop and enhance clusters of expertise.

"The events of the last 18 months have put the life sciences industry in the spotlight and it is clear how strong the sector has become," says Dr Kath Mackay, director of Life Sciences at Bruntwood SciTech. "It is reaping the rewards of investment in the development of advanced therapies, vaccines and associated supply chains while we are traditionally strong in drug discovery and clinical trials.

"Through the expansion of our SciTech portfolio and development of our campuses, we are aiming to create 40,000 jobs and these are high value science and technology roles – ranging from laboratory assistants to senior leaders – that will be of huge benefit to the UK."



The BioIndustry Association, which represents more than 300 start-ups, emerging and established bioscience companies, academic and research and organisations, reported a £3 billion raised by UK biotech in the first three quarters of 2021, with £576 million raised between June and the end of August.

The fast pace of growth needs to be matched with the swift expansion of laboratory and office space and the campus and cluster opportunities. Identifying the right space for the right place and designing it to evolving needs is critical to the national drive for life sciences success.

Life Sciences Real Estate Comes of Age

Bruntwood SciTech is a joint venture between established commercial property specialists Bruntwood and Legal & General to create the UK's largest property company dedicated to life science and technology.

It is building on a strong heritage to create life sciences and technology based innovation districts that have the perfect features for companies to form, scale and grow.

"Life sciences real estate is becoming more widely recognised as an asset class," says Dr Mackay. "Bruntwood SciTech is the most networked property company working in the science and technology space through our joint ventures with universities and hospital trusts, and a number of large corporates.

"It is a dynamic and exciting sector to be a part of as there's a huge need for laboratory space across the UK.

"There is a race to develop laboratory space in the UK but it is important to recognise that this is a very technical area; while there is a lot of discussion at the moment about repurposing office space – it isn't always possible."

Bruntwood SciTech closely monitors evolving needs driven by advances in technological processes, automation and robotics that require bespoke responses.

"Bruntwood SciTech is home to expert developers with great experience at meeting demanding technical criteria and understanding the needs of clients," she adds. "This expertise is vital to the growth of our clusters around the country and the success of companies that choose us."

Sector knowledge is critical to growth

The Urban Land Institute identifies the life science property market as a top three emerging trend and predicted that ageing populations and technical advances would combine to increase healthcare economies and that science parks and clusters would need first class facilities and amenities to attract talent in a highly competitive market.

But it also pinpoints challenges around lack of knowledge and operational expertise.

"This is where Bruntwood SciTech's experience is so important," adds Dr Mackay. "We have the sector knowledge and expertise that is critical to establishing and growing a life sciences business in the right ecosystem with the right support.

"For instance, you need an experienced developer who really understands what a drug discovery company needs to make its drugs, to comprehend the heavy chemistry requirements and the need for specialised space. It is very difficult to operate in this field without true life sciences real estate expertise.

"One of our key USPs is that we support our businesses with deep knowledge around the technical areas they operate in."

The ULI Report, Understanding the Life Sciences Sector: The Case for Real Estate Investment, published earlier this year, predicts continued growth across life sciences property to meet the needs of a healthcare industry innovating to treat an ageing demographic living with multiple co-morbidities and tackle major health challenges.

A National Vision

The UK's life sciences sector is crucial to the economy and the government's Life Sciences Vision – a programme devised to make the UK a Science Superpower – calls for a connected national response to make it happen.

Its Foreword states: "The human Life Sciences sector is among the most valuable and strategically important in the UK economy, and critical to the country's health, wealth and resilience. In recent decades, advances in the Life Sciences have fundamentally improved the length and quality of life in the UK and globally, and we stand on the cusp of an era of cures, in which new technologies make previously terminal disease treatable or curable."

The triple power of academia, local government and private companies have been identified as a spur for innovation and industry investment.

The Life Sciences Vision report highlights a need to unite talent and opportunity by supporting clusters of excellence.

"International evidence suggests the development of clusters is important for sustaining and attracting manufacturing investments in particular geographies," it states.

"Government will therefore work with UK-based manufacturers and local partners to support cluster formation around existing sites, as well as interventions, such as Freeports and access to shared technical facilities and advice, that will support cluster formation in new areas of the country."

Bruntwood SciTech's strategy synchronises perfectly with the vision by operating on a national scale and working as a facilitator and accelerator of regional clusters that generate growth and collaboration locally and nationally.

Stronger Together

When global molecular testing company QIAGEN chose to locate its new HQ at the £25 million Citylabs 2.0 development in Manchester, it was a clear indication of the value of being at the heart of the action.

"They decided to put their Global Centre for Precision Medicine at our Citylabs campus because it is one of the leading innovation campuses in the country, if not the world, with direct access to the world-leading research and talent availability at the University of Manchester and immediately surrounded by the hospitals they want to work with. They saw the benefit in being in that rapidly expanding clinical and precision medicine campus."

The Citylabs campus is a hive of innovation, located within the UK's largest Foundation Trust, Manchester University NHS Foundation Trust, where it is surrounded by five of the country's major hospitals including the Manchester Royal Eye Hospital and Royal Manchester Children's Hospital. Opened in 2014, the campus now includes Citylabs 1.0 & 2.0 which are fully let with Citylabs 4.0 imminently due to being construction given the overwhelming demand from precision medicine, diagnostics, genomics and digital health companies – all wanting to be located on the campus where they can access joint clinical trials, sample supply, diagnostic collaborations, medtech adoption and research.

Its evolution, located within Manchester's Innovation District, the Oxford Road Corridor, is part of an ambition by Bruntwood SciTech to grow the Citylabs Campus and neighbouring Manchester Science Park to 1 million sq ft of space for life sciences and tech businesses which is need to accommodate the flood of investment and companies attracted by the proximity of world-class institutions such as the University of Manchester, Manchester Metropolitan University, the National Graphene Institute, the Biomedical Research Centre, Manchester Centre for Genomic Medicine and The University of Manchester's Health Technology Research Hub – the Pankhurst Institute.

Citylabs has attracted the Stoller Biomarker Discovery Centre, Yourgene Health, MAC Clinical Research, Apis Assay Technologies, Nuffield Health and Takagi, to name a few while it is also home to the city's unique singular umbrella health science and innovation system – Health Innovation Manchester – which enables products and services to be accelerated into the health system at pace and scale.

"There are many benefits from being in a cluster; whether you're a small company or a large company. You can tap into a specialised talent pool and collaboration is much easier. We see businesses on our campuses working together at all levels on existing projects and innovation programmes," says Dr Mackay.

"We recognise the value of collaboration and design our campuses with key breakout, meeting and event spaces to get people out of their laboratories and workspaces and talking to each other. We also provide a community events programme alongside bespoke business support to help our customers thrive, network and, ultimately, grow."

Bruntwood SciTech aims to be an extension of a company's team with deep understanding of their needs for specific lab and office space and quick access to talent and expertise.

It also re-invests surplus operating profit and revaluation surpluses arising from increased occupancy back into Manchester Science Park, which has enabled the site to expand and become self-sustaining. It has a rolling programme of campus masterplans and building developments along with the creation of enhanced collaborations space, meeting and conference rooms, amenities and the flexibility for companies to grow organically.

Cluster benefits promote scientific discovery

The connectivity includes easy exchanges and collaboration with companies at Alderley Park, which is now a world leading science and innovation campus. Offering 1m sq ft of labs and office space, it is home to national institutions such as Medicines Discovery Catapult, the CRUK Manchester Institute, alongside global leaders such as Sai Life Sciences and Evotec.

"I don't believe you get that benefit of collaboration if you're not physically in a cluster. Being co-located and connected with institutes, academic groups, hospitals and university infrastructures brings a wealth of advantages," adds Dr Mackay. "Alderley Park is now thriving. It's home to over 3500 people working in many areas of drug discovery and development, with particular expertise in oncology and infectious diseases, but also with a growing tech cluster and it is only 30 minutes from Manchester which has Europe's largest clinical academic campus.

The government's Life Sciences Vision picks up the mantra of driving economic growth and self-sustaining clusters of excellence across the UK, with its intention of supporting Manchester as a world leading centre for genomics and data, developing Liverpool's reputation for infection and immunology and enhancing med-tech heritage in Yorkshire and Humber.

Bruntwood SciTech is underpinning that with a strong presence at the Liverpool Science Park where its five-year masterplan is attracting science and digital tech campus companies. It follows the cluster template, bringing innovative enterprises close to the Liverpool School of Tropical Medicine, the University of Liverpool's Institute for Infection and the Manufacturing Technology Centre's northern base and other NHS centres.

"We're already working closely across the regions to advance discoveries – at Alderley Park we're working in collaboration with the Medicines Discovery Catapult to build a Translational Research Facility in Infectious Disease which actively seeks to build collaborations with SMEs and academics in infectious disease research."

The new facility will provide critical infrastructure for future infection readiness, including a new UK-leading category 3 biosafety (BSL3) facility to grow the capacity and capability of organisations working to validate diagnostics and treatments in infectious diseases.

Bruntwood SciTech's national reach extends to Begbroke Science Park, in Oxford, and Melbourn Science Park, just outside Cambridge. Acquired in 2020, Melbourn Science Park offers office, lab and engineering space as well as meeting and events space and further underscores the national reach.

Networks of opportunity

Bruntwood SciTech's ambition is to grow and energise existing campuses and create new centres which provide a nationwide network of opportunity.

It has used its 20 years' experience of the Birmingham property landscape to acquire and develop the Innovation Birmingham Campus which is occupied with more than 150 digital start-ups that enjoy 113,000 sq ft of co-working, serviced and leased offices and a community hub.

The campus, which is expected to expand to 800,000 sq ft over the next decade, is close to the University of Birmingham, Aston University and Birmingham City University in the city's Knowledge Quarter. The first phase of this expansion will be Enterprise Wharf, a 120,000 sq ft smart-enabled building which is due to complete in 2022.

Bruntwood SciTech has big ambitions for Birmingham which extend beyond the Innovation Birmingham Campus. The company has recently unveiled plans to develop Birmingham Health Innovation Campus, in partnership with the University of Birmingham. The development, in Selly Oak, in the south of the city, will link closely with the Innovation Birmingham Campus and galvanise the life science sector in the region.

"It is the first development of its kind in the region and Birmingham has this huge, untapped potential where we can really build upon the clinical and academic strengths in health and life sciences in the area to create a focal point for life sciences," says Dr Mackay.

"We're looking to deliver a 10-year master plan. Phase one of that we'll complete in 2023 and offer about 68,000 sq ft of commercial laboratory space, alongside the University's Precision Health Technology Accelerator. This ambition is possible because we have unparalleled experience in the sector and there is a real understanding of the businesses in our portfolio.

"Our innovation services team, the heads of innovation, venture managers and business growth managers are in tune with the businesses they serve and help. Regular on-on-one advice sessions are available, particularly for emerging businesses, helping them write grant applications, raise funds and a whole network of support we put round them to ensure they are successful.

"We also run specialist accelerator programs and workshops and boot camps and access to longer-term, structured programmes."

Amazing Space and more

Bruntwood SciTech offers a full range of products including coworking, serviced office, managed office, conventional leased space and laboratory space. It comes with the traditional benefits of facilities and property and project management but its life sciences heritage elevates the offering to a transformative level. Alongside the property offering, the company also provides a range of highly specialised scientific services such as partnerships with Cytiva and Waters which allow customers to access high quality equipment without having to invest capital when they are starting out.

"We do create amazing space and working environments but it is much more than that," adds Dr Mackay. "Being in partnership with institutions such as hospital trusts brings huge opportunities for the businesses in our network. They get access to clinicians. They are able to have discussions around getting their products adopted, initiating clinical trials and getting some real-world evidence as they develop their products and their services."

"We understand the impact of co-location. Our accelerator programmes provide unique opportunities to co-locate and collaborate with established businesses in a challenge-led environment. This means they can access everything from expert advice, to help with pitching to investors, PR and profile opportunities, HR and recruitment support and a range of networking events."

Bruntwood SciTech was selected as the chosen partner earlier this year by the University of Manchester to create the final, albeit extremely large, piece of the puzzle for a world-leading Innovation District in Manchester called ID Manchester. The £1.5 bn ID Manchester will be home to the most exciting new ideas and ventures, addressing some of humanity's greatest challenges and opportunities with many opportunities for life science companies to make it their home.

"Our JV partnership with The University of Manchester has got huge ambition for ID Manchester and we're extremely excited to have been selected to work with them to deliver its vision and masterplan, which will be on of inclusive innovation shaped by creativity and invention, and working to deliver a healthier, happier, greener future for all," adds Dr Mackay. "We haven't just been selected as a developer. What they really value from us is the specialist support we offer to our customers and our in depth understanding of what life sciences and tech businesses need."

Deep understanding

"They've seen that as incredibly attractive when they were looking for a partner to develop the site. Taking Citylabs in Manchester, which we are developing jointly with the University of Manchester NHS Foundation Trust, as a further example it brings a wealth of experience and innovation within touching distance of each other.

"The hospital and the clinicians within those groups are very keen and hungry to work with industry. Being in a joint venture, creating a campus with a hospital trust is very powerful in facilitating that. It's an incredible opportunity for organizations looking to join us.

"We help facilitate that too through introductions and through that deep understanding of the businesses that we have in our networks.

"Our UK expansion marks a significant advance for life sciences."

"Our UK expansion marks a significant advance for life sciences."

About the interviewee

Dr Kath Mackay is director of Life Sciences for Bruntwood SciTech. She has a keen interest in growing the life science industry, and businesses and infrastructure within the sector, ensuring the UK is the best place to establish and scale a life science organisation.

Kath oversees Alderley Park – the UK's largest single site life science campus – the new Birmingham Health Innovation Campus and Citylabs. Kath is also non-executive director of the Northern Health Science Alliance, the North of England's health partnership, Infex Therapeutics, a clinical development company which acquires, develops and licenses innovative drugs to treat pandemic infections, UKRI's National Biofilms Innovation Centre, and Cheshire and Warrington Local Enterprise Partnership.

About Bruntwood SciTech

Bruntwood SciTech (a 50:50 joint venture between Bruntwood and Legal & General) is creating a network of innovation districts, connecting the UK's leading regional cities and science and technology communities through opportunities for collaboration and an extensive partner network.

Bruntwood SciTech owns and operates the UK's largest portfolio of science and technology assets. Creating the environments and ecosystems needed for companies in the science and technology sector to form, collaborate, scale and grow. In addition to high quality office and laboratory space, scientific services and tailored business support, Bruntwood SciTech provides unrivalled access to finance, talent, markets and mentorship.

Our locations provide inspirational, innovative communities in which start-ups, scale-ups and global headquarters thrive.

www.bruntwood.co.uk/scitech

Recruiting patients to increase diversity & inclusion in clinical trials: one size does not fit all

More so now than ever, the inclusion of more diverse, and often more relevant, study populations is gathering momentum within the clinical trial community.

From calls for more diversity in age, gender, and ethnic groups, to more consideration for lifestyle and cultural differences, trials need to ensure they represent the key population of interest and identify a greater range of opportunities to maximise benefits for both patients and pharma.

Seth Nelson, senior director, Patient Recruitment Solutions at ICON, stresses that pharma needs to proactively encourage inclusion and diversity across clinical trials, from trial planning and protocol development to patient recruitment and education.

Effectively, the detail and the differences matter. "Across the spectrum, optimum outcomes will only be achieved by drawing on patients' cultural, geographical and everyday influences," he points out.

Challenges to the design, planning and implementation of clinical trials

The past 18 months of the pandemic and the respective lockdowns has had a marked impact across both healthcare and medical research. Clinical trials are no exception and have been on the receiving end of both challenges and opportunities.

The identification and recruitment of patient participants in trials became more difficult, as non-COVID patients steered clear of hospitals and GP surgeries for fear of infection and/or over-burdening an already overwhelmed NHS. On the plus side, this limited the spread of infection, but at the same time, opportunities to recruit and retain potential trial participants were also impacted.

However, he adds, that on a positive note, the pandemic created a captive audience and generated an interest and increased understanding in health and research. “Now is the time to capitalise on that to improve diversity and inclusion.”

Greater diversity and more tailored participant subgroups in clinical research

For some years, patients have had an increasingly more central role in how clinical trials are designed and conducted. Nelson notes that pharma and clinical trial organisations are listening to the patient voice and developing a protocol with the patient foremost to plans. “This is vice versa to what was done historically, where at the most basic level the sponsor identified the unmet need, proposed a potential treatment, developed a protocol based on this and then searched for potential participants,” he explains.

He believes a departure from the traditional approach to conducting trials will be truly patient-centric, leading to greater participant diversity, as well as improved education that is more tailored to the needs of the study population. Together these will ensure patients are more engaged because the trial has greater relevance to them. "I think this is going to make a significant impact on trial success moving forward."

Nelson explains that one of the changes in medicine overall is the move towards more personal medicine. "We're narrowing down the overarching therapeutic area into smaller groups." A similar approach is being adopted by including greater diversity in clinical trials, he says, explaining that there will be a higher number of subgroups, such that any potential treatments, as well as the trial process, can be made more honed to unmet treatment needs.

Oncology is typical of this approach with not only multiple different cancers, but within one cancer type, there are often many specialist subgroups. "In oncology, especially in lung cancer, there's been a lot of advancements driving interest in new therapies. Human nature draws us to those we see provide value, so when you see success coming from something, you're more interested in being part of it – both as a patient, trial participant and as a physician."

Speaking to diversity beyond that of the disease aetiology alone, Nelson points out that trial planning needs to take into account a wide range of social and lifestyle factors. "We need to understand how a new therapy interacts with everybody differently," he says. "Being able to bring a more diverse data set and understanding of how that therapy is going to impact and help a more diverse population, provides better insight on where potential success of the therapy or product lies."

"If you aren't including a diverse population, you're missing a large part of what you're really trying to do and understand," he stresses. "It's time to understand who this is going to benefit and how it will benefit them."

ICON is placing diversity and inclusion central to its trial work

Diversity and inclusion are high on the list of ICON's priorities in trial planning and implementation. Nelson notes that the design and planning process needs to shift physically and mentally to accommodate the needs of the most relevant patients. "If a disease has high prevalence in a subgroup, then take the trial to them – geographically, culturally, and educationally. Trial design needs to incorporate that patient population at the table from the word go."

He outlines ways that ICON is adapting to meet these needs and insists that the solution is far from a one-size-fits-all approach. "Instead of really going after just interest with outreach and social media, we're utilising census data again, and materials that address a larger population to identify subgroups that have the greatest potential to be positively impacted by a certain treatment or study."

"It matters who you're identifying and reaching out to, because that changes your strategy and your outcome," Nelson emphasises. He reflects that progress has been made but there remains a long way to go. Patient advocacy groups are being better engaged, he adds, but much more education of all stakeholders is still needed. "The status quo is starting to change and is moving in the right direction, but there are a lot of pieces in this puzzle."

Diversity and inclusion need to be embedded in planning if the desire is to truly transform clinical trials. "We need to move on from 'here's a trial… now you need to identify a more diverse population'," Nelson points out, "to something that more proactive and integrated into protocol planning, patient voice insight, patient recruitment strategies, and patient advocacy engagement."

And it needs to be a consideration in the selection of study sites, training and educating site study teams and investigators, and patients. “Driving the overall strategy that we’re all part of diversity and inclusion within the trial, needs to be a major focus from the start.”

Engaging patients and encouraging study participation requires a carefully considered value proposition. “It’s providing that kind of value statement of what it is about the study such that participating can hopefully change the outcome of your disease. We need to present the value proposition to patients,” says Nelson.

Essentially, trials need to be tailored to meet the real-world needs of patients with different backgrounds including ethnicity, gender, geography, lifestyle factors, and different ages, among others. “We’re really diving into that and honing into which populations these therapeutic areas impact the most. We are then creating the trial around that – sites are selected within the regions where that population lives. The site staff benefit from localised education on how to recruit, and how to get in contact with that population,” adds Nelson.

He reinforces that this is a long-term goal, and that there are no shortcuts. “We’re taking the right steps, but there’s this big turn that we need to make, and we need to make it every single time we put a study and a protocol together.”

About the interviewee

Seth Nelson has over 15 years of clinical research experience, focused on clinical operations and patient recruitment. In his current role, Seth leads Patient Recruitment & Retention Services. Prior to his current role, Seth held multiple high level positions within the clinical research space including heading a multi-site research network, VP operations in clinical research recruitment and retention. He holds a BS Biology and Chemistry and a MS in Medical Physics.

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's 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 Organisationsthrough a number of high-profile industry awards. With headquarters in Dublin, Ireland, ICON employs approximately 38,000 employees in 151 locations in 46 countries. Further information is available at www.iconplc.com.

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