pharmaphorum deepdive

CRISPR: A new era for medicine

From concept to cure, explore key milestones in the ambitious journey of genetic editing

February 2024: R&D

Accelerating innovation in rare disease

How cell and gene therapies are infusing blood centres with new purpose

Heard on the pipeline: AI in R&D

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pharmaphorum deepdive

Deep Dive: R&D 2024

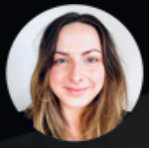
Just a few short months into the new year and already the crackle of innovation has begun to permeate across the pharma R&D landscape. Expectations for 2024 were already high, particularly in the gene editing space, after news broke that the first CRISPR-based therapy had received official regulatory approval, first in the UK and now in the US.

A decade in the making, CRISPR is a shining example of how scientific curiosity and collaboration can produce life-changing treatment options for patients around the world. And so, to celebrate this milestone achievement, it seems only fitting to kick off 2024 – the year of CRISPR-based therapeutics – with a look back at key points in the technology's development journey.

On the subject of patients urgently awaiting new therapeutic options, millions of people living with rare diseases desperately need innovation in diagnosis, treatment, and care, but how can we get new solutions to them faster? In this month's Deep Dive, join Alexion's Soraya Bekkali as she considers the possibilities.

Elsewhere in this issue, pharmaphorum editor-in-chief Jonah Comstock hits the floor at the 42nd annual JP Morgan Health conference to find out how attitudes towards AI in R&D are changing, blood donation centres infuse new life into the cell and gene therapy space, and we find out what it takes to navigate the complex and evolving landscape of clinical trial supply management.

For all this and more, read on.



Eloise

Eloise McLennan – editor, Deep Dive

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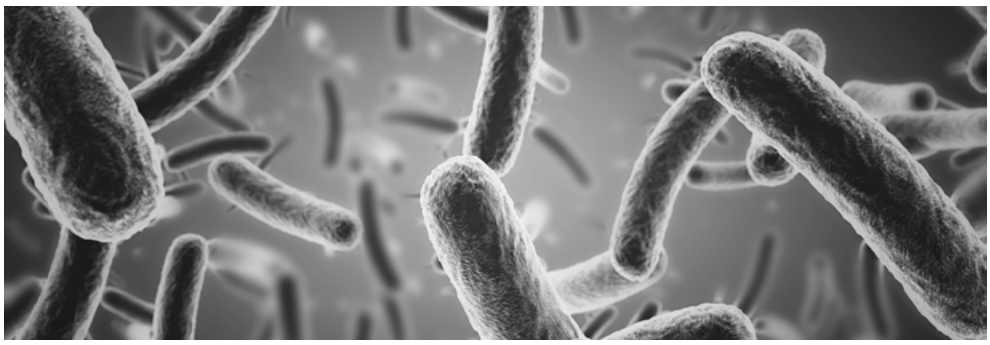
Decoding life: Key milestones in the CRISPR odyssey

The story of CRISPR's development, from its humble beginnings as a bacterial defence mechanism to its current status as a groundbreaking tool for gene editing and beyond, is a fascinating journey through scientific discovery and innovation, illustrating the collaborative and cumulative nature of research.

Initially observed as peculiar sequences in microbial genomes, CRISPR's true potential has been unlocked through decades of curiosity-driven exploration. Following landmark approvals in the UK and US, we reflect on the key milestones that helped to transform a biological curiosity into a powerful tool that promises to revolutionise medicine, agriculture, and beyond.

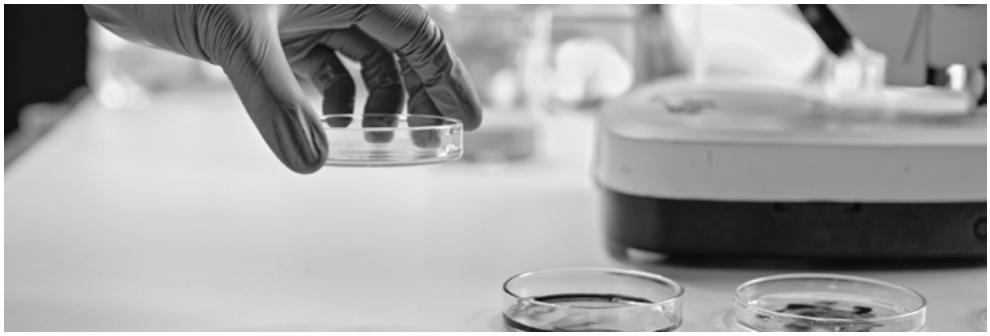
1987
A sign of things to come



The first sign of CRISPRs emerged in the late 80s, when, while studying a gene in E. coli, Japanese scientist Yoshizumi Ishino and his team stumbled across something unexpected – repeated sequences interspersed with shorter unique 'spacer' segments. These 'Clustered Regularly Interspaced Short Palindromic Repeats' had never been seen before. Unfortunately, due to a lack of DNA sequence information at the time, the actual biological function of these sequences would remain a mystery for more than a decade.

Of course, this unanswered question didn't deter scientists from exploring ways to use the information found in CRISPR loci. Just a few years after Ishino's initial discovery, in 1993, researchers in the Netherlands observed that different strains of Mycobacterium tuberculosis had different spacer sequences between the DNA repeats. Using a technique called 'spacer oligonucleotide', the team began to characterise strains based upon their spacer sequences.

2005
Identification of CRISPR's adaptive immune function



By the turn of the century, Spanish molecular biologist and microbiologist Francisco Mojica had already made quite a name for himself in the world of CRISPR, being the first to characterise what we now call a CRISPR locus (originally dubbed short regularly spaced repeats) in 1993. Throughout the 90s, Mojica devoted his work at the University of Alicante to uncovering the secrets of these sequences, and by 2000 he had observed CRISPR loci in 20 different microbes, identifying common features that are now recognised as CRISPR hallmarks.

Image: Distinction of the Generalitat Valenciana for Cultural Merit: Francisco Martínez Mójica [left]. (Photo: Alberto Sáiz)

Mojica's dedication paid off in 2005. After years of painstaking research, he discovered that CRISPR sequences matched parts of the bacteriophage genomes. This breakthrough led him to conclude that CRISPR serves as an adaptive immune system in bacteria, that protects microbes against specific infections; a notion confirmed by further research and parallel discoveries by others.

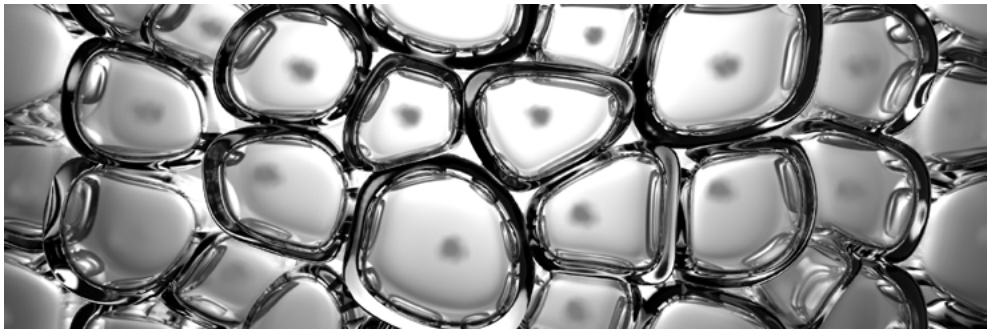
2012
Hijacking the system

A major breakthrough came in 2012 when Jennifer Doudna and Emmanuelle Charpentier demonstrated the ability to programme CRISPR to precisely target and edit specific DNA sequences. Simply put, the researchers identified a way to re-engineer the Cas9 endonuclease with single RNA molecules that could hunt down and cut the DNA target specified by the guide RNA.

Attribute "© Johan Jarnestad/The Royal Swedish Academy of Sciences"

Their landmark paper, published in 2012, demonstrated how CRISPR-Cas9, originally part of bacteria's immune system against viruses, could be harnessed to cut DNA at specific locations. This discovery provided scientists with a powerful tool to edit genomes with unprecedented precision, speed, and flexibility. However, despite the revolutionary implications of the results, when the study was published in June 2012, it wasn't met with the accolades one might expect for such a discovery, and it would take a long time before CRISPR-Cas9 would become headline news.

2013
The race to expand the CRISPR toolbox



In the scientific community, the race was on to push the boundaries of the technology. For months after her initial discovery, Doudna and her team worked tirelessly to find out if the test-tube success of CRISPR could also be translated to living cells. At the same time, four other research teams were also working towards the same goal.

In January 2013, five teams of scientists published studies demonstrating successful use of CRISPR in living animal or human cells. The first came from Feng Zhang and his team at the Broad Institute. The study, published in the journal *Science* saw CRISPR-Cas9 used as a tool for genome editing in eukaryotic cells of humans and mice. Around the same time, George Church, a pioneering geneticist at Harvard University, also published his research on genome editing in mammalian cells using CRISPR-Cas9.

The CRISPR toolbox expanded further with the discovery of additional CRISPR systems beyond Cas9, such as Cas12 and Cas13, each offering unique capabilities for gene editing and manipulation. This diversification enhanced the versatility and precision of CRISPR technology.

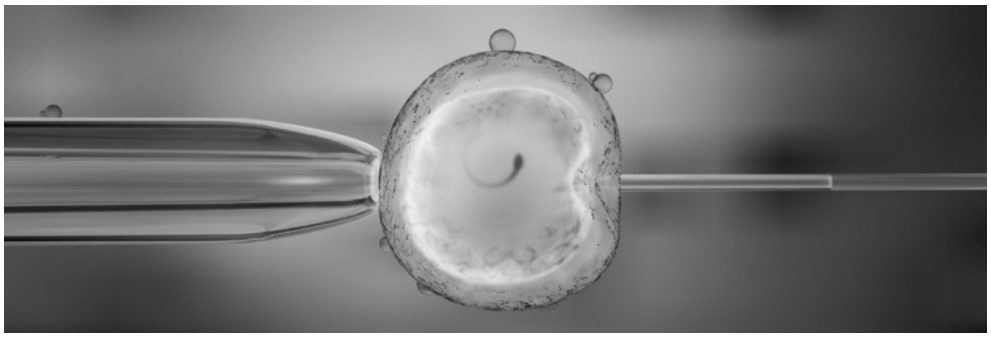
Later in the year, Doudna, Church, and Zhang joined forces with respected scientists David Liu and J Keith Joung from Havard university to explore ways to commercialise CRISPR-Cas9 technology. The result came in September 2013 in the form of a company – Gengine, Inc (which was swiftly renamed Editas two months later).

2016
First in-human trials commence

Naming issues firmly in the past, Editas soon began making its potential known. Just a few short years after the creation of CRISPR-Cas9, the company (alongside co-sponsor Allergan) was granted unanimous approval by a federal biosafety and ethics panel in the US to begin the first ever in-human trial of the technology.

The groundbreaking BRILLIANCE trial of EDIT-101 focused on gene editing treatment for patients with Leber congenital amaurosis, a genetic disorder causing severe vision impairment from birth. Patients would receive the treatment via a subretinal injection to reach and deliver the gene editing machinery directly to photoreceptor cells – marking a significant step towards in-vivo editing.

2018
The 'CRISPR babies' scandal



The meteoric rise of CRISPR technology brought with it a wave of possibilities and opportunities; concerns over the ethics of gene editing were also growing louder.

While the scientific community seemed to be united in the need to balance the excitement of innovation with cautious and careful consideration, one 'rogue' scientist had other ideas, ideas that would thrust CRISPR under a very public ethical microscope.

In 2018, Chinese scientist Dr He Jiankui made the groundbreaking, and highly controversial, claim of having created the world's first genetically edited babies using CRISPR-Cas9 technology – having altered the DNA of two human embryos to make them resistant to HIV.

Pictured: Dr He Jiankui speaking at the Second International Summit on Human Genome Editing.
Credit: Iris Tong, Public domain, via Wikimedia Commons

If Dr He had been anticipating his work to be met with global acclaim, he was in for a big shock. The technology was still in its infancy, with potential unforeseen consequences and safety risks still unknown. His actions triggered widespread and high-profile condemnation, fuelling ethical concerns as experts criticised the experiment's lack of transparency, proper oversight, and consent processes.

The incident prompted calls for stricter regulations and guidelines surrounding gene editing, particularly emphasising the importance of balancing scientific advancement with ethical responsibility. Moreover, for his actions, Dr He was found guilty of forging ethical review documents, misleading doctors into unknowingly implanting gene-edited embryos into two women, for which he was handed a three-year prison sentence.

2019
Tightening the reins on CRISPR innovation

Rather unsurprisingly, amid fallout from the public 'CRISPR babies' scandal, the World Health Organization (WHO) convened an Expert Advisory Committee on Developing Global Standards for Governance and Oversight of Human Genome Editing. The 18-member committee approved the first phase of a new global registry to track research on human genome editing, as well as an online consultation on the governance of genome editing.

Addressing the second meeting of the committee, Dr Tedros Adhanom Ghebreyesus, WHO's Director-General, said: "New genome editing technologies hold great promise and hope for those who suffer from diseases we once thought untreatable. But some uses of these technologies also pose unique and unprecedented challenges – ethical, social, regulatory, and technical."

That same year, Chinese authorities also prepared gene-editing regulations, stressing that anyone found manipulating the human genome by gene editing techniques would be held responsible for any related adverse consequences.

2020
Recognising pioneers

While 2020 will likely be remembered for another significant development in the history book of global health, it was also a standout year in the story of CRISPR. One of the year's most notable achievements was the awarding of the Nobel Prize in Chemistry to Emmanuelle Charpentier and Jennifer Doudna for their development of the CRISPR-Cas9 gene editing technology, marking an historic recognition of CRISPR's transformative potential. This accolade cemented the two women as pioneers in the field and underscored the technology's profound implications for genetic research and its potential for therapeutic applications.

The year also saw advancements in CRISPR's therapeutic applications, with the first human clinical trials using CRISPR-Cas9 showing promising results in treating genetic disorders. Notably, a study reported in the *New England Journal of Medicine* demonstrated success in using CRISPR to edit the genes of patients with sickle cell anaemia and beta-thalassemia, offering hope for curing these hereditary blood disorders.

Elsewhere, in the phase I/II BRILLIANCE trial, researchers at Casey Eye Institute, Oregon Health & Science University, administered EDIT 101 to the first patient. This was the first time a CRISPR therapy had been successfully applied directly inside the human body.

Pictured: Emmanuelle Charpentier © Nobel Prize Outreach. Photo: Bernhard Ludewig

Pictured: Jennifer Doudna © Nobel Prize Outreach. Photo: Brittany Hosea-Small

2023/24
A new era of medicine

A decade after Doudna and Charpentier quietly showcased the potential of programming CRISPR to target and edit DNA sequence, in late 2023, CRISPR made mainstream media headlines once again. The first-ever CRISPR-based gene editing therapy had been approved for marketing in the UK.

The Medicines and Healthcare products Regulatory Agency (MHRA) cleared Vertex Pharma and CRISPR Therapeutics' Casgevy (exagamglogene autotemcel or exa-cel) in in the UK as a one-shot therapy that involves harvesting bone marrow stem cells from patients and using CRISPR to modify them outside the body (ex vivo) before reinfusing them to treat the diseases.

In the context of medical research, where treatments can take decades to reach patients, it was an extraordinary achievement.

Just a few weeks later, regulators in the US followed suit, making Vertex Pharmaceuticals' Casgevy the first every FDA-approved gene therapy to utilise the CRISPR method. With the ball now rolling, the FDA lost no time in clearing Casgevy for a second indication (transfusion-dependent beta thalassaemia) in January 2024, more than two months before its deadline.

The future of CRISPR

CRISPR technology is still in its early stages, but it has the potential to revolutionise medicine. Researchers are working on developing new CRISPR-based therapies for a wide range of diseases, including cancer, genetic diseases, and infectious diseases. The future of CRISPR is bright, and it is likely to play a major role in healthcare in the years to come.

About the author

Eloise McLennan is the editor for pharmaphorum's Deep Dive magazine. She has been a journalist and editor in the healthcare field for more than five years and has worked at several leading publications in the UK.

Heard on the pipeline: AI in R&D

As has become tradition in the pharma and biotech world, 2024 kicked off with a bang as experts from around the globe gathered in San Francisco for the 42nd annual JP Morgan Health Conference. The event gave attendees a chance to scope out the landscape of innovations, trends, and challenges for the upcoming year. And so, with pharmaphorum editor-in-chief Jonah Comstock reporting from the floor, it seemed the perfect setting to find out how attendees view one of the hottest topics in healthcare today.

How has the mindset within the industry evolved regarding the acceptance and adoption of AI technologies in R&D?

Jen Nwankwo

CEO, 1910 Genetics

"Last year was certainly the year of AI, fuelled by ChatGPT, which OpenAI and Microsoft launched in November 2022. I think that launch brought AI to the consciousness of the layman. People who hadn't heard about AI, people like my mum, people at the grocery store had the opportunity to play with ChatGPT, a consumer-facing conversational AI interface. It really captured the imagination and continues to capture the imagination with the opportunity to do a variety of things and just this idea that there's this sort of friend that you've got in the form of ChatGPT that you can ask questions and get answers to.

"The release of ChatGPT brought AI into the consciousness of the average American. For those of us who are in what you'd call a verticalised solution space, which means we are using AI to build solutions in a specific vertical like biotech and pharma, we certainly are excited to see AI in the mainstream consciousness, but for us not a lot has changed, both in terms of the immense opportunity for AI to improve R&D productivity in pharma, which has declined consistently since the 1950s, as well as some of the barriers that have prevented AI from delivering on its potential in pharma and biotech. So, we're excited to see everybody coming along for the ride and just forging ahead with trying to bring the potential of this tool and this incredibly powerful technology to fruition in the form of actual medicines for patients."

Katherine Stueland

CEO, GeneDx

"It's rapidly impacting the way that we're conducting our business, both in terms of how we are scaling our interpretation platform, how we're taking the knowledge base that we have and insuring that, at scale, we're able to deliver the highest confidence level results. But we're also looking at how it applies to business operations. An important element of access for patients is making sure patients have insurance coverage and that we can then actually have a smooth billing process. So, AI can actually help inform the way we are able to procure medical necessity documents that support a claim that may be going to a payer. I think there's a whole host of ways we are today deploying AI and will continue to deploy AI from a clinical perspective, from a scientific perspective, but also just in terms of the nuts and bolts of how we operate the business day to day."

Simon Arkell

CEO and co-founder, Ryght

"One thing we've never heard anyone say is 'You know this AI thing, I'm not interested.' Everyone's interested. The question is, for a company that's in the business of selling SaaS software to help solve problems in the industry, what are the problems that have a cheque book attached to them? Where do you have a painkiller and not a vitamin? Last year there was just a lot of playing around. There were pilot budgets available with some of the bigger companies and just a lot of intrigue and playing around with it themselves in the smaller companies.

"But we're now seeing that companies have failed at doing it themselves because they realised how much complexity was involved on the backend, and we're seeing that they're coming to us and saying why don't we just get the initial application live, start seeing value, and then grow from there. So, we're seeing real traction now, but it's been five or six months of people just dipping a toe in the water. Where we're really going to start seeing value is when there's true, demonstrable ROI and then the industry will start to pick up."

Gene Kinney

CEO, Prothena

"Certainly, there are a number of companies that have started around AI technology in terms of target selection, thinking about how to better model and approach the way we select targets and target biology; how we might model what certain proteins do when they go awry and when they start to become aberrant in the context of disease. I think that that's an important understanding of things we can leverage in our understanding of how normal biology becomes abnormal.

"But I think, on an operational level, of course AI can be very helpful just in terms of keeping the operational wheels spinning in a more efficient way. Incorporating that in a responsible way into an organisation, thinking about databases, collating data, making sense of certain data sets that are rich in information are also important ways one can incorporate AI into your normal everyday work life."

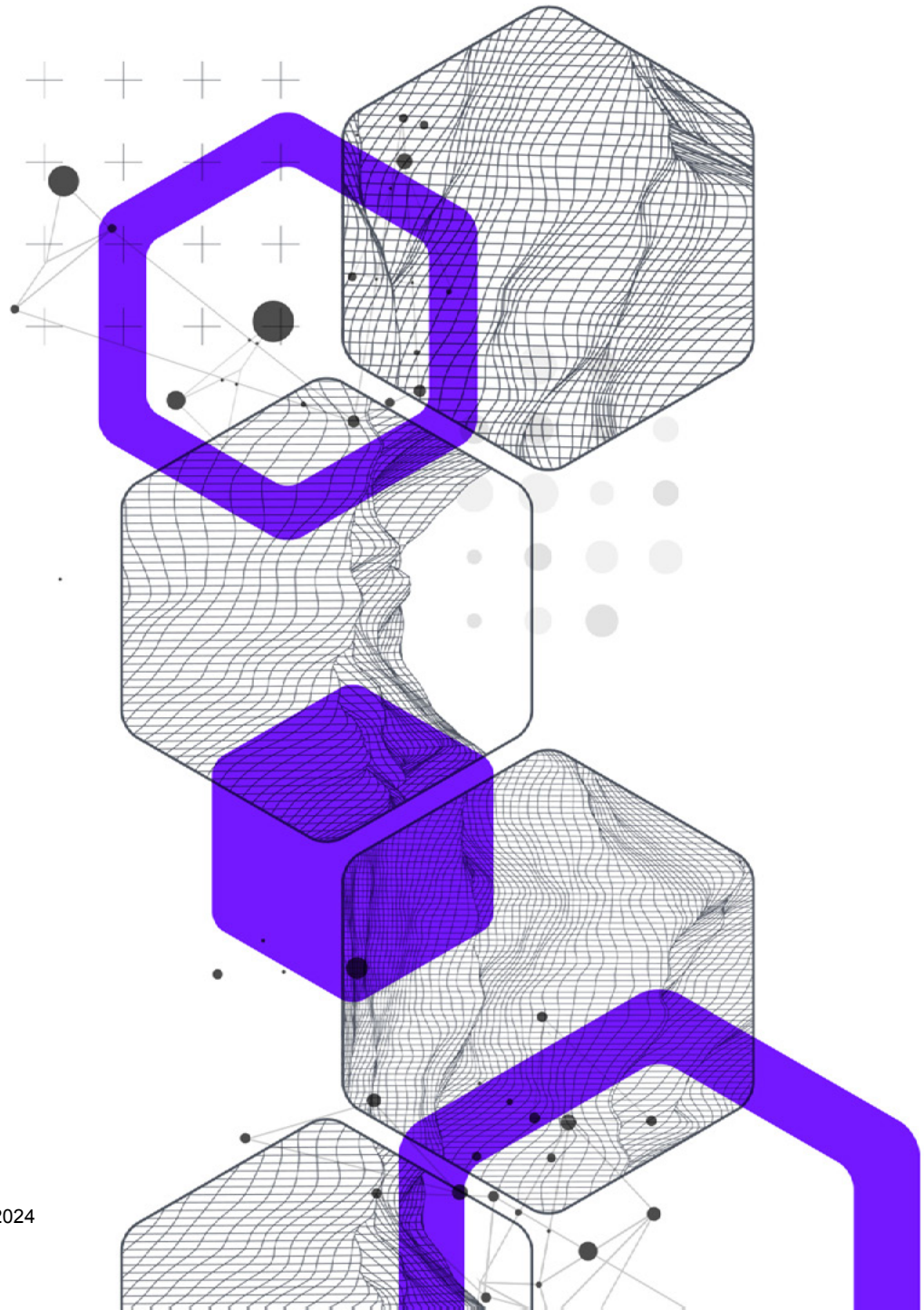
Trevor Martin

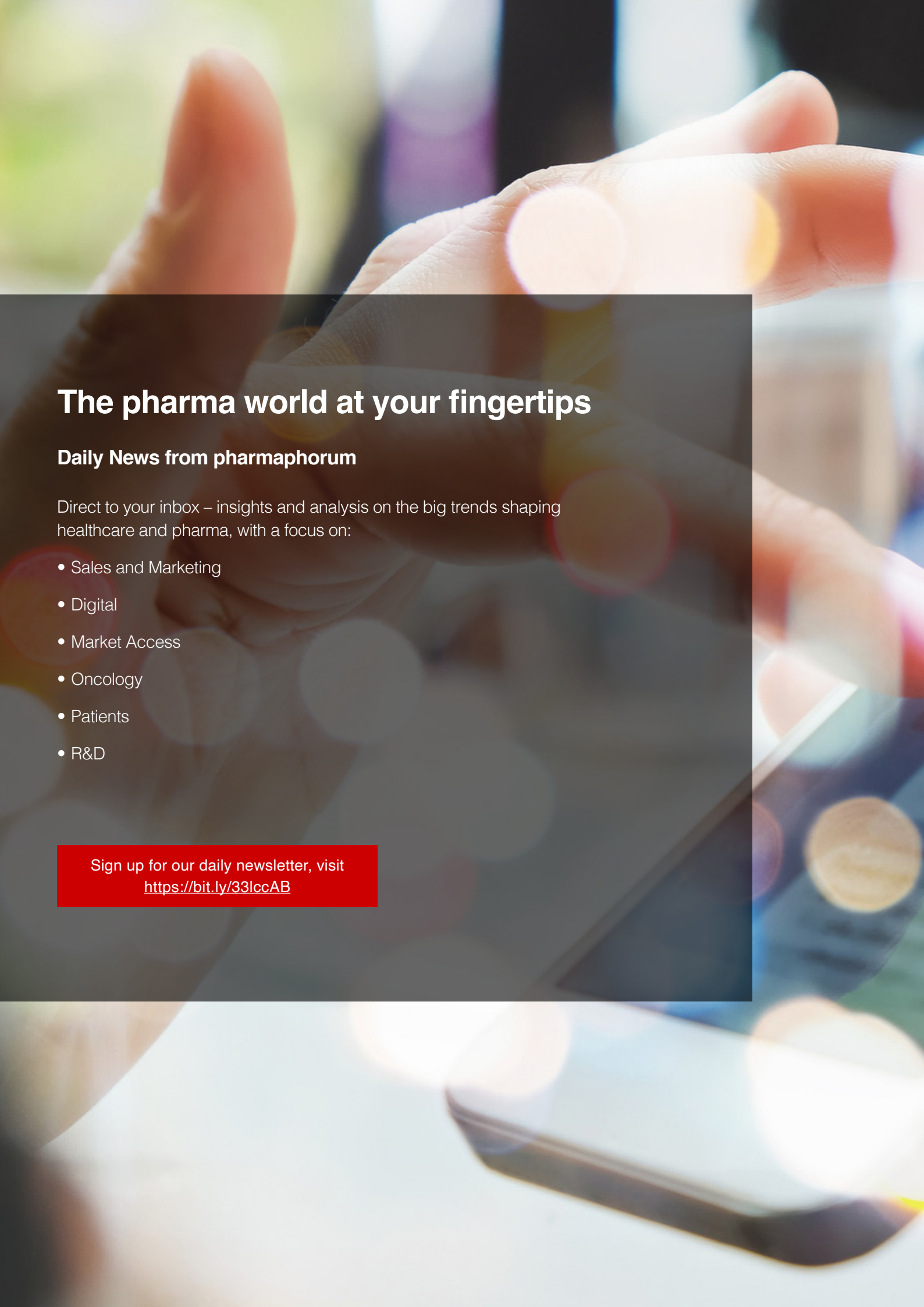
Founder and CEO, Mammoth Biosciences

"Things always go through some cycles. First, it's like 'What is this?', then, it's this race to find different applications. And now I think we're getting to the part of the cycle that's most exciting to us, because I think it's what matters the most, which is, what does this actually make a difference in? You don't just want to slap AI on different things. How are you going to use it that's actually going to make a difference for building a drug, for finding patients, whatever it is, but what is the AI actually going to enable that wouldn't be possible without it? For example, for us for the last decade leveraging these new AI techniques to find and characterise new CRISPR systems, seeing which work and which don't, and getting better and better at it over time."

About the author

Jonah Comstock is a veteran health tech and digital health reporter. In addition to covering the industry for nearly a decade through articles and podcasts, he is also an oft-seen face at digital health events and on digital health Twitter.





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Accelerating innovation in rare diseases

Millions of people living with rare diseases are urgently waiting for diagnosis, care, and life-changing treatment options. Alexion's Soraya Bekkali considers how to get new solutions to them faster.

Rare diseases are a global health concern. They affect an estimated 400 million people worldwide and are often severe, progressive, and life-limiting. For patients living with rare diseases, getting a diagnosis – then accessing treatment if it even exists – is a challenging journey, which, on average, takes over five years. And, despite significant R&D progress in some areas, there are still no meaningful treatment options for 90% of the estimated 10,000 rare diseases.

People living with rare diseases desperately need innovation in diagnosis, treatment, and care – and they need it fast. So, how can innovation in rare diseases be accelerated?

Expertise and innovation: Tackling the challenges of rare disease R&D

The complex biological nature of rare diseases and their infrequency in the general population pose a myriad of obstacles to R&D that can slow – or even stall – innovation.

Identifying patients and defining unmet needs are complicated by small, heterogeneous populations, poor underlying disease awareness, limited diagnostic capability, and gaps in knowledge about the natural history and progression of the disease.

Designing and populating trials is more difficult because of the characteristics of the patients involved (e.g. paediatric patients) and the fact that small, geographically dispersed patient populations often require multicentre, multinational trials. Participation can be restricted by rigid inclusion and exclusion criteria and, for many rare diseases, established endpoints or defined clinical measures are frequently unvalidated or simply don't exist.

Generating evidence to demonstrate the value of innovative therapies is particularly complex because orphan drugs often don't fit into traditional value assessments due to the nature of evidence that is possible and ethical to produce.

New technologies and partnerships: Advancing our understanding of rare diseases

Developing a deep understanding of rare diseases and combining this with the agility to innovate and adopt new R&D approaches is essential to drive innovation forward. Around 80% of rare diseases have a genetic origin, and gene and cell therapies are rapidly emerging as a promising new route for delivering the breakthroughs patients so urgently need.

In addition to a pipeline of investigational assets across multiple disease areas, over the last year, Alexion has acquired a portfolio of preclinical rare disease gene therapies, which show considerable promise, and has collaborated with Cellectis to accelerate our cell therapy and genomic medicine ambitions. Our goal is to develop new therapies with improved safety and efficacy profiles and to expedite these innovations into the clinic.

We are also harnessing artificial intelligence (AI) and machine learning to increase rare disease understanding. These powerful tools can potentially help us shorten the time to diagnosis and identify and validate potential therapeutic targets more efficiently. Partnering with Verge Genomics, we are able to leverage their AI-enabled ConVERGE platform, which applies machine learning to human tissue data to identify novel disease targets that may have a higher chance of success.

The future of medicine development can also be accelerated with digital solutions. Alexion, combined with AstraZeneca, has the opportunity through our health tech business, Evinova, to implement digital health technology in clinical trials that may improve the patient experience and drive healthcare transformation with accelerated timelines and reduced costs.

These new technologies are creating exciting research opportunities and cementing a new era of collaboration as rare disease experts, medical pioneers, patients, and other members of the rare disease community partner to accelerate scientific progress. We have great optimism about the potential to deliver real change for patients in the coming years, but it is equally important to ensure that drug discovery is coupled with an empathetic and inclusive understanding of patients and their experiences.

New perspectives: Patient-centricity fuels R&D progress

Being deeply connected to patients enables us to lead the way in innovating clinical trial design. Alexion was the first company to adapt the patient friction coefficient to rare diseases, evaluating the burden of clinical trial participation on patients and caregivers, informing protocol changes, mitigation plans and enhancing support services. In addition, our Solutions To Accelerate Results for Patients (STAR) process gathers insights from patients and other stakeholders along the disease journey, enabling us to design clinical programmes and development strategies that are centred around patient needs.

No matter how small the patient community, we need to listen to as many voices as we can. We must focus on enhancing equity and do everything in our power to ensure that all patients are able to benefit from medical progress. But, drug discovery will ultimately be meaningless if the value of innovation is not appropriately recognised and realised for patients. A regulatory environment that encourages continued innovation and investment in research will be crucial to achieving long-term positive change.

New policy approaches: Shaping the innovation landscape

In terms of policy, this is a critical moment for rare disease innovation in the EU. Alongside the European health data space, which has the potential to have far-reaching effects on how data – including genomic data – can be used to accelerate R&D, and a potential EU 'action plan' on rare diseases, the European Commission is also updating the EU's orphan medicinal products (OMP) regulation. The OMP has been a catalyst for innovation in rare diseases, providing important incentives to drug manufacturers to undertake 'high-risk' research for small patient populations where considerable need exists.

Concerns have been raised that the proposed updates to the OMP could impact the clinical and economic viability of researching and developing rare disease treatments. By reducing incentives offered to drug manufacturers, including shortening data and market exclusivity periods, there is a risk that the OMP could fall short of achieving the Commission's aims – and may even hinder progress.

It is essential that rare disease policies continue to support earlier, faster, and more accurate diagnoses, promoting the use of novel technologies to accelerate innovation within a framework that enhances equitable access across countries, regions, and hospitals.

Partnership across the rare disease ecosystem remains essential for progress

Now is the time for us to speak up and champion approaches that will help address unmet needs in rare diseases. We must be innovative, not just in discovering new treatments, but in how we work together and navigate the complex rare disease ecosystem. We must ask ourselves, *What can we do to move innovation forward?* And, *How can we accelerate progress?* Because for many patients living with rare diseases, tomorrow is too long to wait.

About the author

Soraya Bekkali, MD, is senior vice president, EUCAN & international business at Alexion, a global biopharmaceutical company focused on developing life-changing therapies for people living with rare diseases.

About Alexion

ALEXION
AstraZeneca Rare Disease

Alexion, AstraZeneca Rare Disease, is the group within AstraZeneca focused on rare diseases, created following the 2021 acquisition of Alexion Pharmaceuticals, Inc. As a leader in rare diseases for more than 30 years, Alexion is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development, and commercialisation of life-changing medicines.

Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on haematology, nephrology, neurology, metabolic disorders, cardiology, and ophthalmology. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries. For more information, please visit www.alexion.com.

The many trends reshaping clinical trials: An interview with UCB's Dr Iris Loew-Friedrich

Over the last five years, a confluence of trends have combined to reshape the reality of clinical research. COVID-19 pushed decentralised trials into the mainstream. Global conversations about clinical trial diversity and representation came to a head. And the increasing availability of AI and large, historic data sets has opened up the door for synthetic control arms and digital twins.

As chief medical officer and head of biopharma development solutions at UCB, Dr Iris Loew-Friedrich has been in the thick of these seismic changes since 2008, leading the Belgium-based global pharma company into its own clinical trial future. pharmaphorum sat down with Loew-Friedrich last year to get her thoughts on the ongoing evolution of the clinical trial. The interview has been edited for length.

Jonah Comstock: *So, first of all, tell us a little bit about yourself and your role at UCB.*

Dr Iris Loew-Friedrich: **I'm a physician by training. I spent the first seven years of my professional career practising medicine in academic hospitals and then moved to industry, and ever since have been working in drug development. I think it has become my passion and my way of serving patients as a physician. I'm very passionate about providing innovative and differentiated medicines to patients.**

My role at UCB is twofold. I'm the chief medical officer of the company and I lead an organisation called Development Solutions. We are covering the key drug development aspects, including all of clinical development, regulatory affairs, quality, patient safety and pharmacovigilance, real world evidence, statistics, and also the data office for the company.

When it comes to clinical trials, one of the biggest trends is this move towards the decentralised model. How much are you seeing decentralised trials becoming a core part of how you do research now, as opposed to a return to the status quo of site-based?

At UCB, we had started to work on decentralised clinical trials already in 2016. We have evolved into our preference towards a hybrid model – having brick and mortar sites and adding digital elements into the clinical studies, like telemedicine visits, remote monitoring, and other elements. And I think that's also what I'm seeing across the industry.

I think COVID has given decentralised clinical trials a big boost because it was the only way to keep trials going without major disruption, without major loss of data, without taking undue impact on patients. It pushed the industry to move in that direction and I think it's become a lasting change. There are different degrees of adoption, different levels of passion around the topic, but the whole industry is moving towards digitalisation of the clinical studies.

Are there new challenges that are introduced by working in the digital realm? What are the pros and cons compared to how trials were traditionally conducted?

For me, there are two watchpoints. First, you have to rely heavily on technology, and technology needs to work, right? And you have to ensure that, for patients, there's always stable internet access. You have to have technology that is easy to use, that's intuitive. The more specific training you have to offer, the more complex and the more difficult it's getting for patients. So, simplicity and ease of use and intuitive use is kind of the theme of the day.

The other watchpoint is that we are all human beings, and thrive on individual interactions. Trust is an important topic in the patient-physician relationship, and we will have to make sure, dependent on individual patient needs, that there is enough face time with the physician, the study coordinators, and nurses at the clinical sites. It's the balance that we need to strike between technology and human interaction.

Increasingly, the cutting edge of conversation about how technology has changed in clinical trials is really around data, synthetic trials, and synthetic controls. What do you think are the limits of using data and AI, and what are the possibilities there?

Digitalisation, for me, has three dimensions. There's the technology, there's data, and then it's also the change in culture and change in mindset that's required. At UCB, we are using vast amounts of data from very diverse sources, and we have quite sophisticated advanced analytics and artificial intelligence to look for patterns in data.

We use them in the beginning to get a better understanding of patient populations because we truly want to understand the unmet need and how the unmet need connects to the disease biology. Then we are using data to understand where patients are living. Where do we see clusters of patients? Where should we put our clinical sites? And, of course, we are using data to model clinical studies.

What is very real and what we are using quite regularly, particularly in the rare disease space, is synthetic or historical control arms. When you have only very few patients with very severe diseases, you cannot do a randomised controlled trial. You have to find a historical control and that's very often structured real-world data from the literature or from registries. I should also say we are running natural course of the disease studies by using available real-world data.

What about passive data collection, wearables apps, things like that? How are you using those tools at UCB and how does that fit into the larger vision for development?

Yeah, we are using wearables – or I should maybe say digital health technology – for a number of topics. Of course we are collecting data, blood pressure, and heart frequency – standard things. If we have a patient population that's not able to use digital devices, we will provide a paper version, so we really try to tailor it to patient needs. But the standard for this data collection is now through apps and devices.

Of course, the big advantage of the clinical outcomes assessments via technology is that what was previously done at a visit and then at the next visit, maybe four weeks later, can now be collected on a continuous basis. So, you have many, many more data points. And the more data points you have, the more you limit your variability. This way of collecting data also helps us to reduce the sample size in certain clinical studies.

The challenge is, in my view, twofold. First of all, as data integrity is of paramount importance, these devices need to be of medical-grade quality and really need to be acceptable by regulators for the purpose we are using them for. Regulators are not always aligned around the globe in their requirements.

The clinical studies that we are doing are typically global studies. So, we have a lot of different regulations to observe when we choose our devices, or we have to choose different devices, or we have patients who want to use their own device, and then we have to find ways to accommodate that. So, there's much more tailoring to individual needs, which comes with additional effort and additional complexity.

I think the other topic that is important is how many apps, how many devices can you really impose in a clinical study on a patient or on a clinical site? And the more you can measure digitally and the less integrated the individual tools are, the more you add complexity by adding different tools, right?

For example, when you run a Parkinson's study, and you have an accelerometer, that's a device in itself. You might still have another device for your patient reported outcomes, and another for heart rate. So, you have to be very mindful of how much you want to impose in terms of complexity on the patient.

It also sounds like it's very much not one size fits all. You have to look at the individual trial, the patients, and the requirements.

But that's the beauty of it, right? In the past, clinical studies have been kind of a highly artificial environment to show that a medicine is efficacious and safe. And there's always been criticism that clinical studies do not reflect the real world.

There's literature that illustrates that, with clinical studies, we only reach a very small fraction of the potentially eligible patient population. And why is that the case? It's the case because patients might live far away from the centres where we have run our clinical studies. They might live in rural areas. They might not be aware of clinical studies, what they mean, and how they could contribute. The digitalisation of clinical studies allows us to reach those patients in a different way than we could do it before.

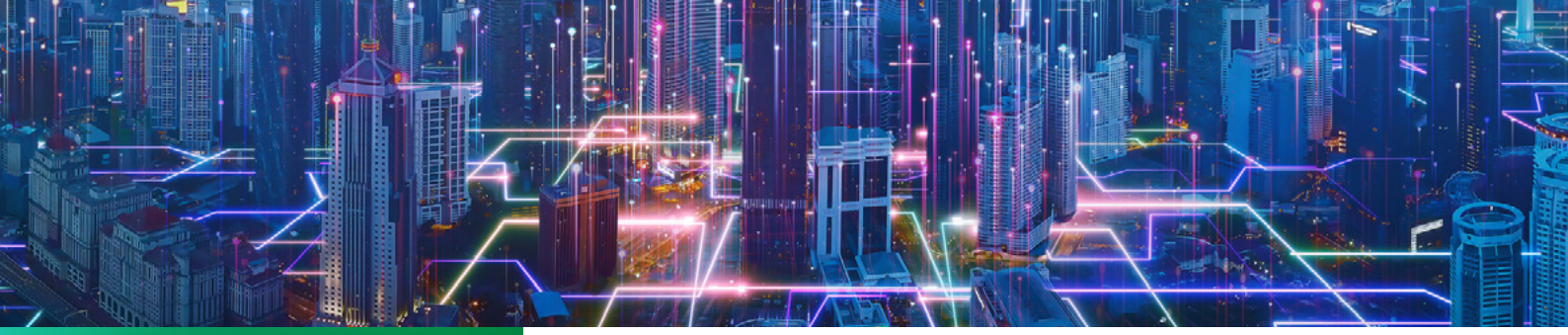
That, of course, then also benefits the studies, which become much more reflective of the patient's reality, of the patient population, and of the way patients live.

The other big trend right now in clinical trials is this awareness of the lack of diversity and lack of representativeness of samples and how that's really affected the quality of the data historically. Is that something that you're thinking about distinctly or mainly just sort of as a side effect of this new, broader approach to data?

It's a mix of both. We are really thinking about it because it's so obvious and so much kind of on top of what we need to do. The FDA is demanding diversity plans now, so you cannot avoid thinking about it. We pay particular attention to bringing our medicines to paediatric populations. We have run some of the most extensive paediatric programmes down to neonates. So, really covering the whole age spectrum, we are aware that age is a topic – older age. We live in an ageing society, so you need to understand how our medicines work in the elderly. We are very much invested into women of childbearing age. We're doing a lot of work in that space. We're thinking about how we reflect the socioeconomic background of the diversity of our population. It's a very broad spectrum that we are looking at and that we are mindful of.

And again, digitalisation of the clinical studies provides us the opportunity to go there. When you go outside the world of academic research centres, outside the world of clinical practices who do a lot of clinical studies, you have to start by building trust that the research that we do is ethical, ensuring that it's understood, why it needs to be done, why it can't be done differently. Trust-building is very important, and that, of course, is based on education, information transparency, and reliability, so there's a lot of groundwork to do before you can really broaden your reach.

For example, one of the discussions that is currently very near and dear to our heart is how we involve the community physician, the family doctor, in our clinical trials? Does it always have to be the 200-mile ride to the clinical centre, or do we find ways to engage the family physician next door? It's easier said than done, but it's a topic that we are discussing because we believe it's important and it's another kind of real-world aspect.



One of the things that's so interesting about clinical trials right now is that there are all these different disruptive trends, but they're all connected. How do you think these trends will evolve in the next five or ten years?

First of all, I believe that, based on the data that we have and with advanced analytics and artificial intelligence, we will be able to modelise clinical trials in a different way than we have done in the past. And if you can modelise trials, then you can improve the design and ensure that you only do the trials that are really helping you advance your medicine or advance the knowledge around medical science.

If you look at the success rates of clinical trials these days, there's a lot of room for improvement and I think data and advanced analytics will help us improve there. If you can run your trial in silico, that will help a lot. So fewer clinical trials with better design and higher probability of success. The other topic is really making sure that more patients can participate in clinical research.

I'm a big believer that patients own their data. Patients should have their electronic medical records and their clinical trial data and it should all be connected so that they can very seamlessly go from everyday care into a clinical study back to everyday care, and the data collection continue throughout. For me, that's the big opportunity, that the frontiers between everyday care and clinical trials blur even more, and that it becomes a fully integrated set of data in the end, which will allow us, in a much better way than we can do it today, to monitor long term effectiveness and monitor long term safety.

About the interviewee

Iris Loew-Friedrich is chief medical officer for UCB, a member of the company's executive committee, and head of development solutions. She provides strategic global leadership for worldwide clinical development, medical affairs, regulatory affairs, quality assurance, statistical innovation, real-world evidence, and patient safety/pharmacovigilance. Her mission is to lead UCB's Development Solutions, ensuring high-quality, innovative, cost-effective development of objectively differentiated patient solutions with proven superior and sustainable value for clearly defined patient populations.

About the author

Jonah Comstock is a veteran health tech and digital health reporter. In addition to covering the industry for nearly a decade through articles and podcasts, he is also an oft-seen face at digital health events and on digital health Twitter.

How cell and gene therapies are infusing blood centres with new purpose

As the cell and gene therapy industry grapples with infrastructure challenges, help is emerging from an unlikely source – blood donation centres. Expanding beyond their traditional roles of collecting blood and plasma for medical treatments, these centres are starting to leverage their technology and trained staff, as well as their ties to local communities, to support the next frontier in chronic disease treatment.

New advances are being made every day in cell and gene therapy, and regulators expect to clear as many as 17 new cell and gene therapies this year, with more coming each year after that. However, developing these therapies and getting them through the FDA is only the beginning. Cell therapies face myriad challenges when it comes to actually getting them to the patients that need them most – not only on the reimbursement side, but also logistical challenges.

"A lot of these patients in the autologous realm have to travel very far to get to a collection site or centre, to even be apheresed, and to then travel back weeks later to have that material reinfused into them once the therapy is manufactured," Priya Baraniak, chief business officer at OrganaBio, told *Deep Dive*. "And if you think about these patients, this is their usually third or fourth line of therapy. They've already gone through chemo, radiation, maybe a monoclonal antibody, you name it, and they're highly, highly compromised."

And right now, the number of sites qualified and accredited to administer cell therapies is shockingly small.

"There's a limit on the number of what are called immune effector cell accredited hospitals in the country. There are like 130 of them out of 5,000," Becky Cap, who at the time of this interview was SVP Business Development at BioBridge Global and now works at Vitalant, told Deep Dive. "And if you then look at the number that are NCI-designated comprehensive cancer centres, that's even fewer. So, we have to figure out ways for smaller hospitals – and I don't mean the true community rural hospitals, but the hospitals in big cities that aren't part of those designations – how do we either get them that designation or expand their ability to treat a broader number of patients?"

A lot of the problem-solving in the industry is about how to administer this therapy in more places – not just in few- and far-between academic medical centres, but in community hospitals and, maybe someday, even at the bedside.

A solution waiting in the wings

Meanwhile, there are blood centres. Non-profit organisations like the Red Cross, Blood Centers of America (BCA), BioBridge Global, and Vitalant showed up on the scene long ago to solve a similar problem – how to collect enough blood on a national level to support lifesaving transfusions, and then get that blood safely and promptly to where it's needed the most.

"The use of blood by hospitals is the very earliest form of cell therapy," said Lee Buckler, SVP of advanced therapies at BCA. "The bulk of the cell therapies, almost all the cell therapies that are commercially approved, are somehow derived from blood. It makes perfect sense that people who know how to harvest blood, process blood, store blood, keep blood safe in the medical supply system, are playing an active and important role in those cell therapies."

It's not directly comparable to cell therapy, but crucial components are there, including a localised, community-based infrastructure serving all hospitals, apheresis equipment and staff trained to use it – often designed to be taken on the road to blood drives in schools, churches, and libraries – and a safe, regulated supply chain to get blood from collection to delivery.

In the early days of cell therapy, Buckler explains, manufacturers weren't thinking about blood centres because they lacked certain crucial facilities, such as clean rooms and cell engineering equipment. But as the field has industrialised, there's been a shift in priorities.

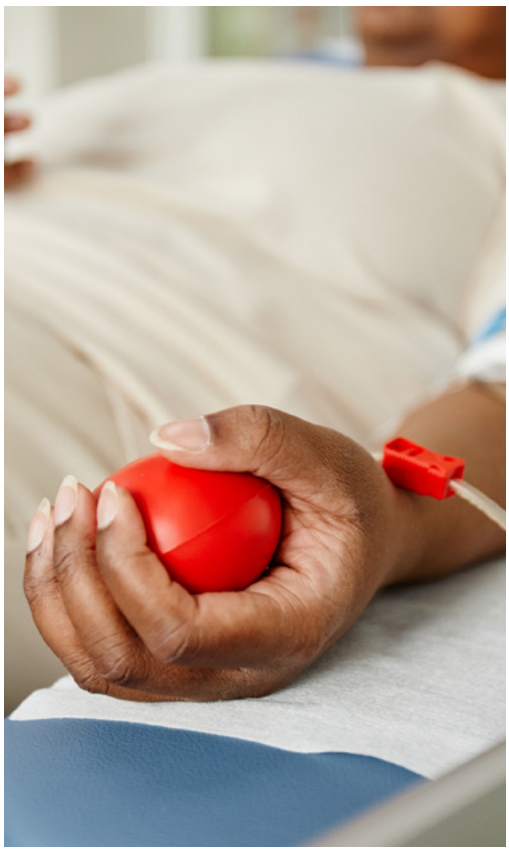
"There's this fascinating sort of trifecta, if you will, of things happening which have brought blood centres back into the equation," he said. "First, it's that automating and simplification of the machines used to manufacture these products, which look a lot like the machines used to process blood. Second of all, many blood centres have built clean rooms to put such machines in. And then thirdly, the therapeutic developers themselves and the clinicians recognise that the hospitals that they need to move into in the Mobile, Alabamas and Tulsa, Oklahomas of the world, don't have the same kind of infrastructure that they do at City of Hope and Memorial Sloan Kettering."

This is more than theoretical at this point, Cap says. While there are still kinks being worked out and a lack of awareness among therapeutic developers, there's also already a lot of adoption.

"It's really about making sure that the therapeutic developers know the capabilities of the blood centres, and the blood centres get comfortable with the specifics of what is being put out there," she said. "But I can easily name half a dozen larger organisations that are really committed to this space, and they have a pretty significant footprint across the country. We still have a few things to work out for it to be smooth across the board, but some of the larger pharma companies are already working with the blood centres to make collection and delivery more efficient."

Manufacturing and allogeneic therapies

While the equipment and training that blood centres have can help them potentially set themselves up as part of the infrastructure for autologous therapies, many blood centres are also looking at collecting biological material for allogeneic therapies – where patients would receive an infusion of modified donor blood rather than their own.



There are a few advantages here. Even though immediacy of location is less crucial than it is with autologous therapies, it's still the case that transplanting blood and cell products long distances is complicated and expensive, so shortening those distances is a positive. Additionally, selling biologic materials to pharma companies is a potential revenue stream for blood centres, which, even though they're most often non-profits, still need to sustain themselves.

"To the extent that there is a revenue or operating surplus motivation, it goes to the sustainability of the organisation," said Cap. "Because if you don't know this, blood centre operations right now, typically a 3% margin is considered good performance."

Chetan Makam, managing director at Terumo, which supplies much of the equipment blood centres and cell therapy developers use, agrees.

"If you look at how the whole blood industry works, our raw material is unreliable at best because we rely on the good-heartedness or the good nature of people to voluntarily donate blood," he said. "I think no other industry or group of people would ever say this is a smart business, because you're relying on the largesse of the human beings to donate. So, yes, people need to operate, and they need to make money to keep this whole system going. But I think they also see it as an additional way of helping."

New challenges in soliciting

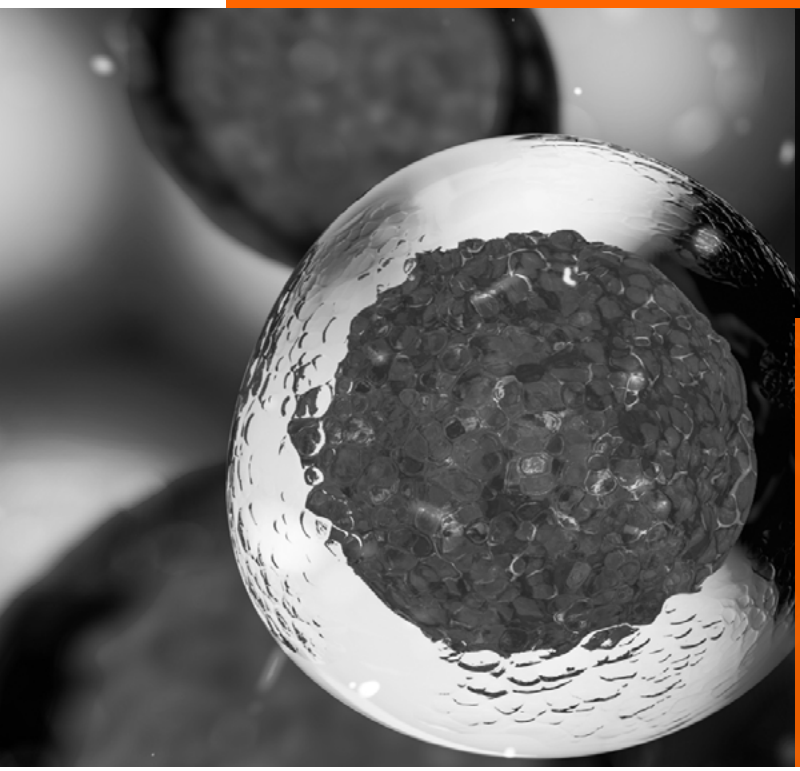
Speaking of good-heartedness, one difficulty of collecting biological material for allogeneic cell therapy is that it's a different sell for donors. With cell therapies going for $2 to $3 million per treatment, people are bound to wonder why they're giving away their cells for free.

"Part of where blood centres or not-for-profit organisations run into some barriers in this realm is that they do not compensate their donors," Baraniak said. "When [...] the Red Cross is calling on you to donate a pint of blood, you know that pint of blood is going to be transfused into an individual who has suffered some traumatic injury or has some sort of disease. There is, an altruistic piece of that. It's much harder for people to grasp that in the realm of allogeneic therapy, where I'm donating something that might go to research, I'm donating something that is a piece of a therapeutic that might be developed, and maybe someday it'll save thousands of lives, maybe it won't. It's a lot harder, I think, to recruit those individuals and keep them engaged for that sort of donation if you're not compensating them for their time."

On the other hand, Baraniak added, the skill of cultivating a donor pool is non-trivial and extremely important for the success of the industry – and it's something at which blood centres are well-practised.

Finally, there are additional concerns when it comes to bone marrow donation and mobilised blood donation (where patients are induced to create extra white blood cells before being apheresed).

"In that case, you have two times in your life that you can be mobilised as a blood donor," Baraniak said. "Shouldn't those two times be used if you need it for yourself or a loved one needs your blood donation, rather than calling you in to get a leukopak for some researcher to put into culture in a lab and then dispose of that material? The ethics around it are quite fraught. I don't know what the right answer is, but we do believe that on the adult side – if donors are coming in and making that donation – they do deserve something for that time."



The future ecosystem of biologics

One thing Cap, Baraniak, Buckler, and Makam all agree on is that the cell therapy delivery groundwork that's being built is not going look like one single entity solving all the problems.

"I think what it shapes up to be is an ecosystem, right?" Makam said. "The way I think of the ecosystem is, where do you start? How does this whole journey progress until you bring it back to the patient who's at the centre of all this? Where is the delivery of the treatment being done? Is it at a hospital? Is it at a blood centre? Is it at the bedside of the patient at home? Don't discount that – I mean, we're doing dialysis at the bedside these days. How are we keeping track of this cell, so that you don't make mistakes? Who's carrying this? Cryogenics, logistics, all these other pieces that surround this ecosystem? So, I look at this evolving as an ecosystem with multiple partners that all work in conjunction."

Baraniak agrees that the need is so great right now it will take many players working together to realise this ambitious goal.

"I think that it is going to be a concerted effort by everyone in this industry who has capacity and capability in any way, shape, or form to move this industry forward," she said. "And we have to find ways of standardisation. We have to find ways of speaking the same language and working together collaboratively to really help patient outcomes. We're going to have to find ways to leverage each other's infrastructure and say, I'm on the East Coast, you're on the West Coast. Here's where the patients have the need. How do we come together quickly and make this happen?"

Luckily, that's something else that blood centres have been doing for a long time.

"While each group has a desire to be a leader, a winner in its own right, our goal is to make sure that the healthcare needs of the nation are met," Cap said. "So, if you think about the blood model, we actually have networks of emergency call chains. If there's a disaster in Florida and the blood bank can't collect or it's without power, there is an automatic call that goes out to the other blood centres around the country, and there's a commitment of number of units of blood and blood products that each one will commit to send to that disaster area. So yeah, we're competing until there's a crisis, and then we call it co-opetition."

Cell and gene therapies are poised to transform medicine in dramatic ways. But even if cell therapy is a brave new world, it's going to take the old players, acting – and interacting – in new ways, to make it a reality.

About the author

Jonah Comstock is a veteran health tech and digital health reporter. In addition to covering the industry for nearly a decade through articles and podcasts, he is also an oft-seen face at digital health events and on digital health Twitter.

The backbone of breakthroughs: Insights into clinical trial supply management

Behind every successfully approved treatment lies a nexus of clinical research activities comprising myriad drug types, trial phases, and packaging needs across diverse geographical locations. Compared to the prestige of white coats and stethoscopes, the role of clinical trial supply is often overlooked in the history books of pharmaceutical research. However, without it, the journey of a treatment from petri dish to patient would grind to a halt.

Encompassing everything from planning, procurement, packaging, storage, and distribution of investigational therapeutics and materials needed to conduct research around the world, clinical trial supply management is not simply a logistical achievement; it is a necessity.

So, to learn more about navigating this evolving landscape, Deep Dive (virtually) sat down with Rocco Barone, senior vice president & head of clinical supplies management at Clinigen, to discuss challenges and opportunities to be found in the future of clinical trial supply.

Supplying demand: A race against time

Ensuring that the appropriate drug is delivered to the correct location at the right time is a mammoth task, with myriad moving pieces requiring meticulous planning and attention in order to operate seamlessly. For many companies, this is not a task that can be handled in-house, which is where clinical supply managers, such as Clinigen, enter the picture.

Understanding the specific requirements of the clinical trial landscape is crucial. Different trial phases, drug types, and research locations have individual needs to be accommodated, and choosing the ideal packaging, labelling, and distribution strategy is key to ensuring the safe and timely arrival of the product. Delays can be costly, and not just financially, as any deviation from a trial's tight schedule can postpone the availability of critical therapies.

"We're working on timelines that are very, very tight," he explains. "If something goes wrong with that drug product, not only is it difficult to replace, but it's also difficult to manage the associated delay, because more than likely they're [the client] waiting on that product to arrive, to start dosing patients for those clinical trials."

He continues: "It all comes down to having the right people in the right roles, and making sure that you have the right knowledge and expertise to be able to ensure that that product is managed in the way that it needs to be managed and that you're really tight on those controls because some products are very temperature sensitive. If they go into excursion, they could be lost for forever."

But timelines are not the only problem that keep clinical supply managers up at night. Unlike the commercial supply environment, clinical supply is a changing beast – often beginning in one point and ending with a new suite of packaging and labelling requirements that need to be managed.

"Some of those changes could be the addition of another country, the addition of more patients, other changes that they want to make and incorporate. Again, there's not a whole lot of time to do that," says Barone. "We have to figure out, through our knowledge, expertise, and connections, how to mitigate and manage all of those changes appropriately, so that the FPI [first patient in] is not jeopardised."

Championing the human element

As Barone previously mentioned, at the heart of every efficient clinical supply chain is a team whose skills, dedication, and collaboration define the success of clinical trials. To effectively keep all the wheels turning, and quickly pivot to accommodate incoming changes or address supply chain challenges, you need to have the right people in place: individuals who not only possess the necessary technical skills needed to get a product from A to B, but who understand the dynamic landscape of clinical trials.

"What defined our success, when I was on the client side, was working with a team of true experts who understood clinical supply – not just supply chain management, but clinical supply chain management, because it is its own beast.

"One of the things I did when I came on this side was to make sure people were in the best roles for them and for us, as well as bringing in some people who could really help us elevate our game."

Prioritising the expertise and innovative thinking that individuals bring to the table throughout the organisation is a distinguishing factor for Barone, as he notes that the majority of competitors in clinical trial supply management share similar capabilities in technology, such as refrigerated storage, frozen storage, and ultra-frozen storage.

"We really have spent a considerable amount of time pulling in as much expertise as possible, in the different applications, to be sure that we gave our customers everything that they needed to be able to deliver successfully," he explains. "That includes quality and compliance all the way down to the technician level. Because, at the end of the day, there's a fine line between right and wrong.

"When they're printing those labels, if we don't have good technicians that are physically inspecting those labels and using our automated equipment to inspect them and making sure that they monitor the signals for when something might be incorrect, a label could very easily go out incorrect, and that could destroy the timelines."

Golden rules for clinical supply management

Overseeing so many variables is a difficult task, but there are ways to mitigate the risk of deviation. To that end, Barrone offers three golden rules for companies to focus on, to help clinical supply management deliver their best work.

Rule 1: Information is power

Barone emphasises upfront communication and information sharing as the cornerstone of successful clinical supply management. "The more information we can get upfront, the more we can put together the right timeline and avoid false expectations," he states. This philosophy extends beyond basic details, encompassing a wide range of data crucial for accurate planning and efficient execution.

Rule 2: Building bridges

Collaboration forms the second pillar of Barone's approach. He stresses the importance of fostering open communication throughout the process. This includes building strong relationships with clients, ensuring they understand the provider's capabilities and limitations, and being transparent about potential constraints.

"We can always create more," he says. "We can build more facilities, we can add more, we can buy more freezers. We can do all that stuff, but I want to make sure that we're not over-promising and under-delivering. It's really about understanding what it is they want, and then on our side, doing that right assessment, to say, 'We can deliver on this commitment. We have the capabilities, capacity, and storage'. We don't want to just take, and then fail to deliver."

Rule 3: The right people, right tools

If you haven't already noticed, Barone strongly values the contributions of experienced and expert individuals. Investing in people makes a big difference, he explains, because those individuals will ultimately drive your success. "If you don't have the right people, you're not going to get far," he states.

A burden worth bearing

While he may enjoy the challenge, Barone doesn't shy away from acknowledging the complexity of his field. However, he notes that overcoming every hurdle to keep research progressing gets us one step closer to the most important element of the clinical trial supply journey.

"At the end of the day, we're talking about some very sick people who have tried everything else and they're participating in clinical trials because they have no other options," he explains. "Getting them the drug product that they need, and making sure that they get those doses, and not only get them, but get them on time is very important to me.

"It's really non-negotiable. We have to throw everything we can at it to ensure that we can get those supplies to those sites."

As trials grow in both number and complexity, the challenges facing clinical supply management are also evolving. But, for Barone and his team, this is part of what makes the work interesting.

"[Clinical supply management] is a very complicated process, and there's opportunity for mistakes, so many different ways every day," he says. "It's not a routine day in, day out. You really come to work every day, and you're really pushed hard."

"It's probably why I only sleep like four hours a night," he jokes.

About the interviewee

Rocco Barone is the senior vice president & head of clinical supplies management at Clinigen. Barone holds a Bachelor's in Chemistry and a Master's in Business from Moravian University, US, and has more than 18 years' experience in supply chain and GMP related processes. His expertise includes implementation of latest operational strategies & technologies and operational management with successful application of lean methodologies. After obtaining his undergraduate degree, Barone joined Merck (MSD) as a production scheduler and subsequently held roles of increasing responsibility. His last position, before joining Clinigen in 2022, was director and site head of Merck US clinical supply operations.

About the author

Eloise McLennan is the editor for pharmaphorum's Deep Dive magazine. She has been a journalist and editor in the healthcare field for more than five years and has worked at several leading publications in the UK.

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Setting the standard for sustainability in pharma

Sustainability has become a crucial pillar in pharmaceutical development, with environmental concerns and resource management demanding immediate attention from organisations of all sizes and specialities.

Among those leading the sustainability charge is Samsung Biologics. In recent years, the contract development and manufacturing organisation (CDMO) has made considerable strides in advancing sustainability efforts around the world, becoming the first Korean company to receive the Terra Carta Seal in 2022 and most recently earning the prestigious EcoVadis platinum sustainability rating.

With over three decades of leadership experience spanning healthcare and informatics, James Choi – executive vice president, chief marketing officer, and head of sales support and global public affairs at Samsung Biologics – has been well-positioned to witness the evolution of environmental, social, and governance in the industry. To find out more about the company's work, Deep Dive sat down with Choi to discuss the current and future landscape of sustainability.

Eloise McLennan:

Following the top 1% EcoVadis rating, how are you translating that into environmental and societal improvements?

James Choi: EcoVadis is a reflection of all the work that we put in to date. There are several that I could name. We've established an ESG committee on our board of directors. We did that several years ago, with independent board members to really make sure that we have solid governance in terms of ESG across all of our operations. Since then, we've been making a concerted effort to focus on ESG.

One of the things that we're most proud of is our involvement in the Sustainable Markets Initiative (SMI). It was an initiative launched about three UN conferences ago, COP26, by the then-Prince Charles – now King Charles. He launched a task force, comprised of about 20 different industry task forces, of which health systems is one of them.

In the biopharma sector, there are three working groups: patient care pathways, digital health, and then supply chains. I am proud to say that Samsung Biologics was asked to champion the supply chains working group.

When you look at the total carbon footprint of healthcare, supply chains are about half of all of the emissions in healthcare – like 2.4 gigatons of carbon equivalent. We have a lot of work and opportunity to improve and make a difference with climate change and the health sector. We've been acting as champion, working with our peers and public sector companies like AstraZeneca, Novartis, GSK, Sanofi, and Merck [KGaA] to really drive these initiatives.

When you talk about the supply chain, there are so many moving parts and people involved. Where did you start?

We began by laying four large focus areas. The first was establishing supplier standards, starting with minimum standards that all of our member companies are asking our suppliers to get as a starting point.

The second is looking at green energy. How can we take the great success that we've had in the US and Europe with the Energize programme, for example, and use that as a model to launch similar programmes in Asia, where you don't have as many renewable sources of energy, but where a lot of the raw materials and suppliers are located.

The third area is transport. Much of the last leg of raw materials and supplies delivery is done via carbon-intense trucking and freight. We also want to shift from air freight to sea freight. On top of that, leverage, wherever possible, green transportation corridors to reduce the overall emissions footprint in transportation. Some member companies are also looking at sustainable aviation fuel because certain parts of the business rely on air freight no matter what.

Then the last piece is leveraging more recycling, reducing waste, and water efficiency. The most recent area that we're turning our focus towards is the heat. In chemical drug manufacturing, there's a lot of heat used at high temperatures, which requires a green heat source.

What were the biggest challenges that you encountered and how did you overcome them?

There are a lot of challenges. I would say some of the biggest ones are that there are so many different suppliers and suppliers have different levels of maturity in their ability to get to net zero. Some of the larger suppliers have a profile similar to ours. These are large global companies in the industry that also have similar initiatives. We feel very confident that they could partner with us to get there.

Then there's a long tail of much smaller, mid to small companies, especially in parts of Asia, that aren't as mature and don't have the background or the resources to really make these types of commitments to reduce their carbon footprint. Those are the ones that the SMI, especially the supply chains group, is looking to help educate, provide tools where it makes sense. Each of the member companies has supplier days to reinforce these targets and, again, to educate and to provide resources where we can.

So far, you've mentioned quite a few achievements. How do you measure the success of the sustainability initiatives and how do you communicate your progress?

Yes, so the first deliverable is, [the SMI] is a coalition of willing. We're not representing all pharma or all companies within the industry, just the ones that have agreed to participate so far and have signed up to these rigorous standards. So far, we've launched a white paper. We did that two years ago at COP27, which laid out the manifesto for our approach of standards. Then, last year, the CEOs of all the member companies signed an open letter to our suppliers saying this is the expectation that we have if you are going to continue to partner with us to help achieve our net zero targets.

The overall progress is being tracked. We look at how many suppliers were able to get to commit to the minimum standards, but we've got to do it in a careful way to avoid any kind of anti-competition or collusion type laws. We make sure that we provide as many incentives and education and resources where we're available to help the suppliers to understand why these are important and how we can together achieve net zero, which is really all of our goals.

Looking ahead, what are the biggest potential disruptions or opportunities that you see on the horizon for sustainable manufacturing and sustainable supply chains?

I think there's potential for innovation to play a large part, obviously, to give an example, one of our suppliers of glass is looking at ways to provide the same capacity of glass vials, but with much less material. The amount of heat that it takes to form that glass is less than it was before. Little examples like that, where even a small incremental improvement in the manufacturing of a certain raw material will have its impact overall as you start adding things up and contribute to the overall reduction in carbon footprint.

Some of our member companies are looking at different ways to package certain supplies. We're looking at ways to leverage more digital capabilities to reduce the overall carbon footprint of a certain process. I think innovation will play a huge role in helping us get to net zero.

Speaking of digital tools, how are you using technology to help you in this journey?

As an example, we've invested in a utility management platform that helps us to really analyse and monitor all of our energy consumption across the board and look for ways to optimise that across our operations. In fact, our newest plant, Plant 5, is going to be 20% less carbon footprint than the previous plants for the same capacity or more. That tells you that we learn through our optimisation in ways to reduce the overall carbon footprint of our operations with every plant that we expand.

Moving away from the bigger Samsung Biologics picture, if you could wave a magic wand and solve one major sustainability challenge, which one would you choose?

That's a trick question! If I could make a magic wand, I would make everything sustainable overnight. I think, let's say, within the bounds of reality, I would make affordable green energy more available in more places where it is needed the most because that's exactly what it's going to take. There are reasons why those energy sources don't exist because of economics of supply and demand. As long as more companies that believe in this movement drive demand, that's the financial incentive for them to make it more available and thus more affordable over time.

Finally, what advice would you give to other companies, particularly the pharma industry and biopharma industry, who are just starting their sustainability journey?

You have to start with looking at the overall footprint of your operations, looking at availability of green energy sources, and making sure that as your operations start to grow and expand, that you keep sustainability in mind to make sure that you grow with an efficient carbon footprint and not make it a bigger problem as you continue to grow.

Note: This interview has been edited for length and clarity

About the interviewee

James Choi is executive vice president, chief marketing officer, and head of sales support and global public affairs at Samsung Biologics. Choi has over 34 years of senior leadership experience in the healthcare and informatics industries in various roles, including information technology and security, customer service, and operations.

Prior to joining Samsung Biologics in 2014, he was CIO at Beckman Coulter's Clinical Diagnostics and Life Sciences businesses, CIO and CTO at Altegrity, and senior director of information systems, customer services e-business technology, and site planning at Philips Healthcare.

Choi holds a Bachelor's degree in mechanical engineering from the University of California Irvine and an MBA from the University of Southern California.

About the author

Eloise McLennan is the editor for pharmaphorum's Deep Dive magazine. She has been a journalist and editor in the healthcare field for more than five years and has worked at several leading publications in the UK.

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Event details

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Chicago, US
Event details

PCC Canada 2024
04-05 June
Toronto, Canada
Event details

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ASLM 2023
12-15 December
Cape Town, South Africa
Event details

DUPHAT 2024
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Dubai, UAE
Event details

SAPHEX
13-14 March 2024
Johannesburg, South Africa
Event details

GCC Regulatory Affairs Pharma Summit 2024
22-23 April 2024
Dubai, UAE
Event details

Europe

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London, UK
Event details

Pharma 2024
16 -18 April
Barcelona, Spain
Event details

Pharmaceutical Manufacturing and Packaging Congress 2024
22-23 April
Amsterdam, The Netherlands
Event details

NEXT Pharma
14-15 May
Dubrovnik, Croatia
Event details

Asia Pacific

Clinical Trial Supply East Asia 2023
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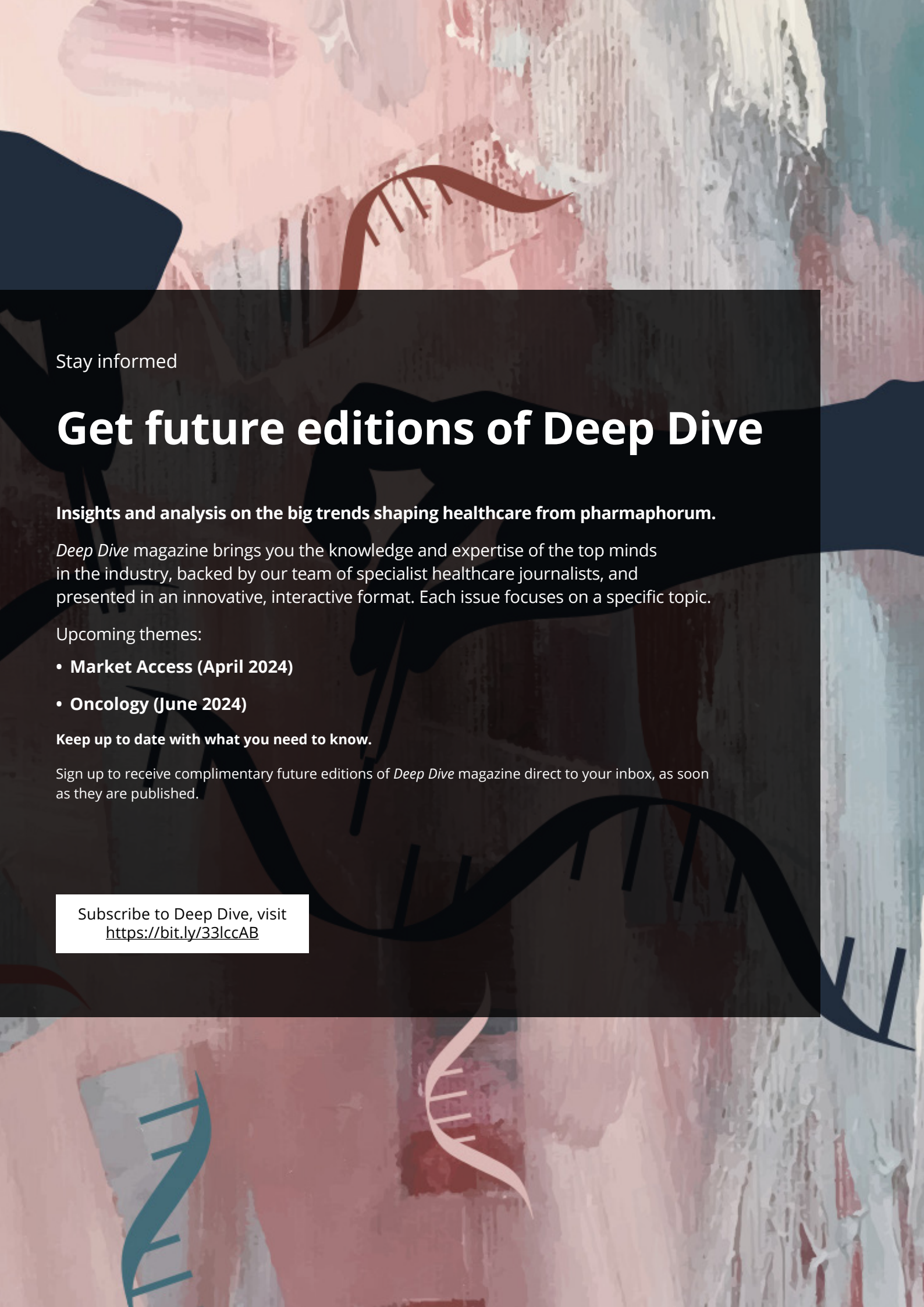
Seoul, South Korea
Event details

CPHI Japan
17–19 April 2024
Tokyo, Japan
Event details

Clinical Trials Festival Asia 2024
20-21 March 2024
Singapore, Singapore
Event details

PSANZ Annual Congress
06-10 April 2024
Christchurch, New Zealand
Event details



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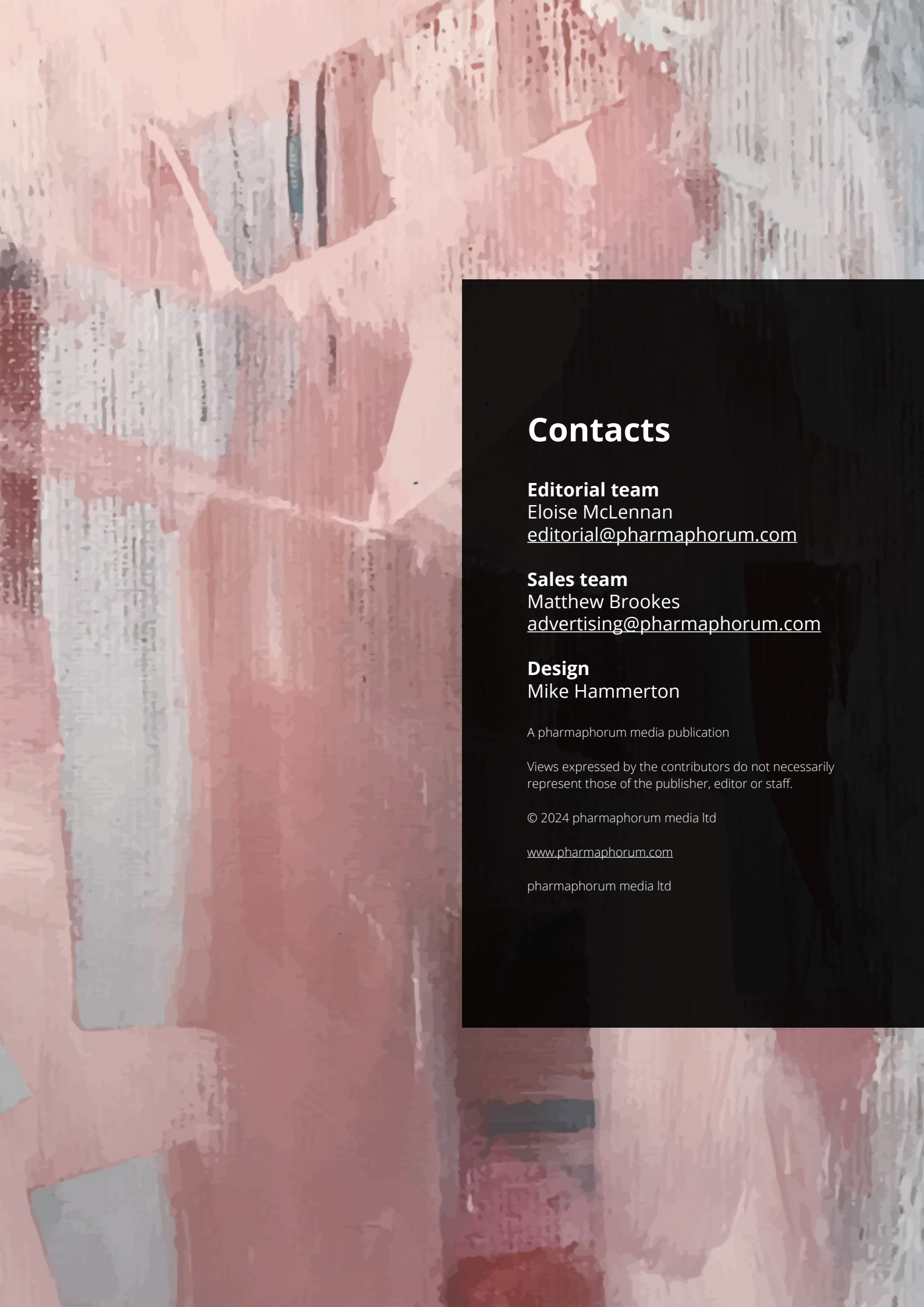

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