September / October 2024
#99

the Medicine Maker®

The Multifaceted Future of Pharma

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Win for Biosecure Act

The Controversial Act has cleared the House of Representatives. Next stop: the Senate.

The Biosecure Act is creeping closer to becoming law in the US after the House of Representatives voted 306 to 81 to pass the draft bill. There is no timeline yet for a vote in the Senate.

The bill would prevent federal agencies, such as FDA and NIH, from contracting with biotechnology companies from certain countries, including China, Russia, Cuba, Iran, and North Korea. It would also prevent agencies from working with or awarding grants to other companies that use equipment and services from the stated countries, which is where the complexity deepens. Chinese companies, in particular, are very prominent in pharmaceutical supply chains. A survey from BIO published in May showed that 79 percent of biopharma companies "have at least one contract or product with a China-based or China-owned CDMO/CMO, and they'll need up to eight years to switch manufacturing partners."

The bill also mentions some companies of "concern" specifically by name: WuXi AppTec, WuXi Biologics, BGI, MGI, and Complete Genomics. Wuxi Apptec – a popular contracting partner in the pharma industry – has issued a statement saying, "We firmly believe that WuXi AppTec has not posed, does not pose, and will not pose a security risk to the United States or any other country and it has not been subject to any sanction by the U.S. government agencies."

The bill includes a grandfather clause to allow existing contracts to be maintained until 2032, but many spooked pharma companies are already making plans to move away from China.

What are your thoughts on the bill? Let me know: *stephanie.vine@texerepublishing.com.*

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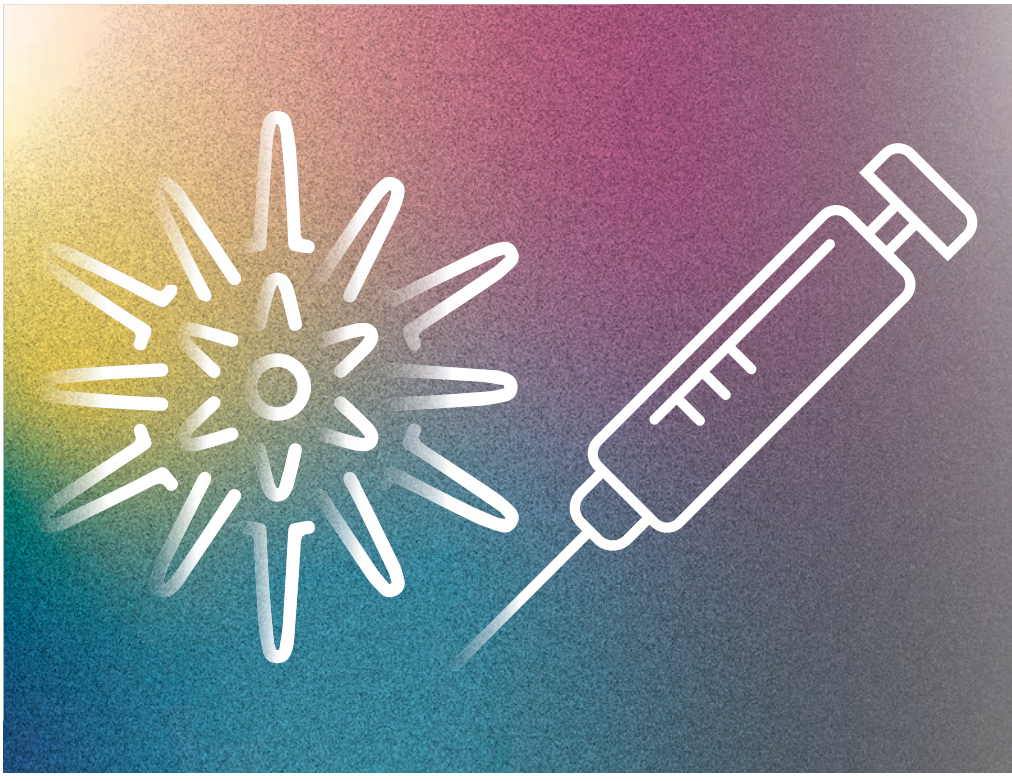
The Virus and the Vaccine

Researchers use modified herpes virus to tackle childhood brain cancers



Scientists have been experimenting with the cold sore virus to find a treatment for children with diffuse intrinsic pontine glioma (DIPG), a type of brain cancer. After undergoing an engineering process and being combined with a cancer vaccine called SNAPvax, the herpes simplex virus type 1 (HSV-1) was able to enter cancer cells and expose them to the human immune system.

A transatlantic team of scientists studied information from between 1995 and 2010 using the US Central Brain Tumor Registry. Five-year survival rates for children with glioblastoma is just 20 percent, and survivors beyond that remain subject to radiotherapy and chemotherapy treatments designed for adults. "There are no treatments that have shown to improve survival, and radiotherapy treatment is palliative. Even with this, children who develop DIPGs survive, on average, for only nine months after diagnosis," says David Jenkinson, Head of Childhood Cancer at LifeArc.

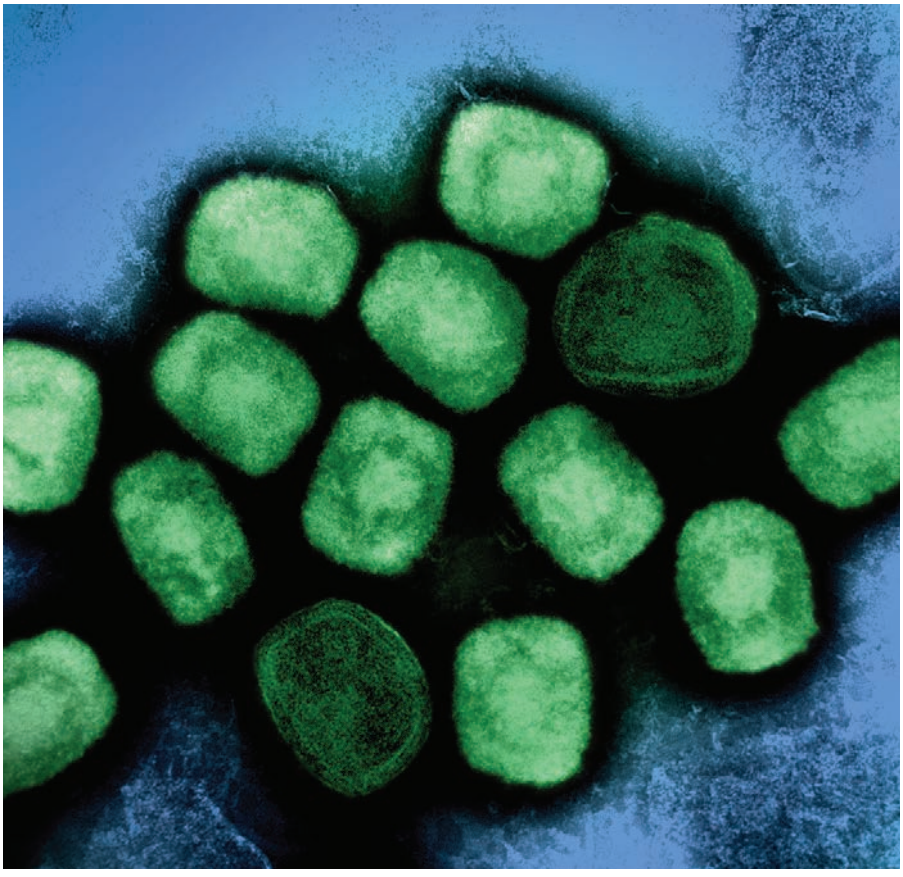
With the work now heading into a phase I trial, we spoke with Jenkinson to learn more.

Why have there been so few breakthroughs in this field?
These tumors are rare, which means there are too few samples for analysis, especially at any single institution. This has driven collaboration through necessity, but we are only just starting to understand the biology of these diseases, which is a critical foundation for any future research. Brain tumors also have the additional complexity of the blood-brain barrier.

How does a cold sore virus fit into innovative cancer treatment?
The modifications to the herpes virus mean that it is no longer able to infect normal cells, but preferentially infects tumor cells. The virus is only able to replicate in tumor cells and has a natural selection for neuronal cells, making brain tumors ideal targets. When the virus replicates, it causes the cancer cell to explode, releasing more virus (as well as antigens) that the immune system can recognize and potentially start to attack. The recent work combines the virus with a vaccine to prime the immune system.

How is genetic modification of a herpes virus considered a breakthrough?
Oncolytic viruses have been approved for treatment of cancer for a number of years. The first approval of this approach by the FDA was in 2015. However, this hasn't resulted in any other viruses being approved yet. Engaging the patient's own immune system is widely seen as important to achieve cures and a number of different approaches are being investigated. The progress of this viral/vaccine combination approach is exciting because there are no treatments for DIPG and the outlook is dismal for these children.

What human stories emphasize the need for new treatments in this area?
When Londoners Namrata and Bhavesh Pandya's 14-year-old son Khushil developed a DIPG brain tumor in September 2017, they focused their efforts on giving him a life as normal as possible. Khushil continued to go to school, and he completed his bronze Duke of Edinburgh expedition in June 2017 in a wheelchair before dying at home in his parents' arms.
Stories like Kushil's remind us every day of why we do what we do. It is easy to just see our work as a science, structural, or business problem to solve, but hearing the personal accounts reminds us of the impact this work can have and makes us redouble our efforts.



Know Your Enemy

Captured at NIAID Integrated Research Facility in Fort Detrick, Maryland, this image is a colorized transmission electron micrograph of mpox virus particles. They were cultivated and purified from cell culture.

Credit: NIAID

QUOTE *of the month*

"This bill is neither strong nor effective. It is poorly written and there are serious issues with its enforcement that would actually undermine our efforts to hold China accountable."

Congressman Jim McGovern gives his view on the Biosecure Act on social media platform X

Awards Season

It's time to share your technological triumphs as we prepare for The Medicine Maker 2024 Innovation Awards!

The Medicine Maker Innovation Awards is your annual opportunity to showcase your company's new technologies – enter now using our online nomination form (https://bit.ly/3Bgk7Fj). The deadline for submission is October 15. To be eligible for entry, the innovation must have launched (or be due for launch) in 2024. Further details are available on our website. A short list will be published in our November/December issue, and the grand winner will have the opportunity to share the story behind their technology in a future issue.

Our 2023 winner was Lonza's Enprotect capsules, followed closely by TriLink's Clean Cap M6 mRNA capping technology as runner up.

With the technology used in today's industrial settings advancing as rapidly as the treatments, we are keen to discover what new technologies and services your company has been developing and launching over the past year. Get your nominations in today to be in with a chance at winning!

Getting Your First Batch Right – First Time

Your API is about to graduate to clinical trial manufacturing. Now what?!

By Garath Duffy, Director of Supply Chain and Project Management at Vetter Development Services, Austria

There is significant pressure to reach the first clinical milestones as quickly as possible – and it is often the first opportunity for drug developers to be rewarded for years of research and work. However, it is also important to not get ahead of yourself; you will have invested a significant amount up to this point, and the intricacies required to maintain a drug's quality must be prioritized during the development of the first batch for clinical trials – regardless of speed. Producing high-quality clinical trial material (CTM) under cGMP takes time, with many developers choosing to work with a CDMO.

Arguably the most important step in producing your successful first batch is establishing realistic timelines based on product complexity and regulatory requirements. You need a comprehensive strategic plan. Supply chain logistics can be out of your control, so it is important to leverage the expertise of all partners to proactively forecast potential delays and maintain compliance throughout. Begin by creating a schedule that accounts for both aseptic filling and cGMP compliance steps and plan for a batch size that accommodates extra testing, destructive sampling and analyses – making sure to factor in enough material for the first stability studies that are key at this stage to show prolonged product quality. Anticipating and addressing critical planning issues early in the process is a preventative measure against potential pitfalls.

As a product transitions to a manufacturing facility for the first time, regulatory agencies and your CDMO require detailed knowledge about the product to confirm readiness for release to trial sites. To effectively scale the production of your API, a well-defined formulation is imperative. Your CDMO needs detailed instructions on how to produce the API and "build" the formula so that they can understand how to handle the drug substance safely and identify all the required ingredients. You'll also need to evaluate which technology and processes are best suited for handling the API, which a CDMO can advise on.

Characterizing the product – with the specific combination of equipment and expertise that will be used to fill it – is a fundamental step to monitor the entire process from API handoff to product release. This step includes assessing filling machinery, sterilization equipment, container types, tubing, and more. Selecting the most suitable container is a common starting point.

Remember that anything you know about your product's particular sensitivities can be vital to aid your CDMO in making the right choices – first time. This includes selecting and confirming the appropriate filter(s) for the product, establishing locked-in testing methods for multiple points in the process, and asking key questions about analytical methods early on to establish quality safeguards. Also, keep in mind that the critical steps in the validation process (which start during process design), often require a certain amount of drug substance beyond what is needed to produce the CTM, so this must be accounted for in the initial batch size calculations.

When communicating with a CDMO partner, it is important to ask about their approach to analytical method validation and whether they can monitor and verify product quality. Asking these questions early on can help ensure that quality safeguards are in place from the beginning. Comprehensive documentation of the manufacturing process can prove invaluable on the backend, potentially averting the need for re-manufacturing of CTM if regulatory authorities raise concerns or have questions later in the process.

To confirm that the final project outputs meet regulatory requirements and support patients as intended, it is necessary to qualify equipment and manufacturing teams, develop and validate processes, and document every step along the way for the highest quality. A CDMO should demonstrate this approach through meticulous qualification and validation processes that maintain consistency and reliability, leading to successful product development, commercial launches, and lasting partnerships.

Taking the time to get the first batch right will ultimately save you money – and stress – during a critical period during your product's development.

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AI in Pharma: A Game-Changer?

How artificial intelligence can transform pharma manufacturing

By Roger Palframan, Head of US Research at UCB

AI can transform how we develop and deliver treatments. Helping predict drug–target interactions, optimizing drug design, and ultimately saving time and costs, AI's power lies in its ability to generate and analyze vast amounts of data to identify new therapeutic molecules with optimized properties in silico. With machine learning algorithms – and while optimizing properties and interactions with molecular targets – experimental work can be streamlined, enabling us to scan and prioritize large, diverse chemical spaces that a human cannot handle.

And then there are clinical trials. Drafting study concepts and protocols, writing reports, automating regulatory processes, dossier filing, data extraction, auditing, and quality management are just some of the areas where AI could potentially help. AI can also leverage electronic health records, genetic data, and other sources to match eligible participants with suitable trials, and ensure a diverse representation of patients regardless of geographic location.

Much like the way AI can analyze large libraries of chemical compounds, it can also draw on large patient datasets to draw insights that may be missed, and help identify new areas of unmet need to determine which treatment protocols could yield the best outcomes for a particular patient population. Dedicated apps powered by AI can also help to track patient health metrics, medication adherence, and symptoms from anywhere

using smartphones or wearable devices.

There is definitely no shortage of real potential. The question then, is how to implement the technology. Where do you start?

"Implementation should be guided by a clear understanding of goals – be they to streamline processes or reduce costs."

It goes without saying that you should be strategic about it. I advise focusing on where AI is expected to have a significant impact in your organization, such as drug discovery, clinical trial design, and landscape analysis. Where do your priorities lie and what is the most important area to your business? Implementation should be guided by a clear understanding of goals – be they to streamline processes or reduce costs. It's also important to be smart about what we can and can't do; the strengths of a pharma-based innovator lie in drug discovery rather than technological advancement, so don't be scared to collaborate with tech providers to help bridge the gap.

One key consideration is the integration of AI into existing systems and workflows, whilst ensuring a human touch. This aspect is particularly critical for clinical trials. AI has vast benefits during trials, but human interaction is required to address participants' concerns, provide clarifications, and establish trust, which will lead to better engagement and retention in trials.

Wherever AI is implemented, workers will need to acquire new skills and new roles will need to be created, but AI data scientists should not become siloed. Your AI-generated data needs to be integrated into the drug development cycle if you want to get the true benefits. For example, it should be something that can continually inform researchers to help them make better decisions.

When using AI in healthcare, there is also the challenge of ensuring data quality and privacy. AI infrastructure must be robust AI infrastructure, with the necessary data security. AI algorithms can also be designed to incorporate differential privacy techniques, while still allowing meaningful analysis of aggregate datasets.

As computational capabilities expand and more diverse data becomes available, AI and machine learning models will only grow more robust. The increase in relevant datasets will teach the AI algorithms and performance will improve. And advances in algorithms and model architectures are already leading to highly sophisticated and efficient AI systems.

There remain vast knowledge gaps in biological science that require wet-lab translational and clinical validation investigation. The essential role of human curiosity in scientific exploration will not change. As we embrace AI's transformative potential in drug discovery and pharmaceuticals, it's vital to remember the enduring importance of the human touch. Despite technological advances, healthcare remains fundamentally patient-centric, relying on empathy and understanding that only people can provide. Balancing AI's impact with this human touch is key to truly revolutionizing patient care and outcomes.

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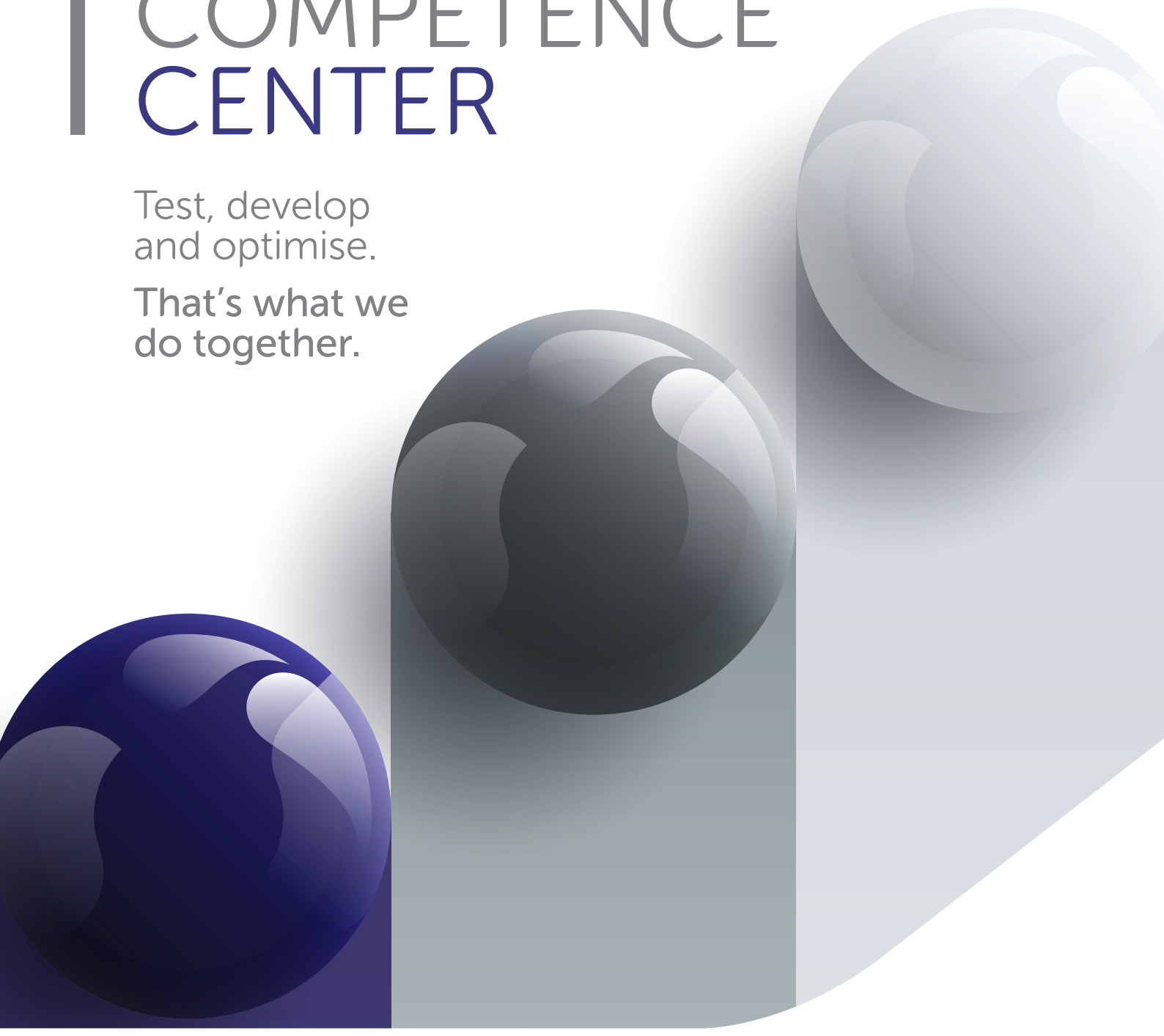

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Dive into the *Heart of Pharma* at CPHI Milan 2024

Join us at CPHI Milan 2024, the premier global event for pharmaceutical professionals, from October 8-10 at Fiera Milano.

The central theme of this year's event is the 'CPHI Celebration' to mark our 35th anniversary and to celebrate the massive role pharma plays in improving the world and advancing health.

Celebrating 35 years of excellence, this event promises unparalleled opportunities for networking, innovation, and professional growth. With over 62,000 attendees and 2,500 exhibitors, you'll experience insightful seminars, cutting-edge exhibitions, and new awards, including "Woman of the Year" and "Future Leader." CPHI brings suppliers, innovators, industry leaders, and distributors together in a dynamic setting promising unmatched opportunities for networking and partnership.

"This year is a particularly significant one for CPHI as we celebrate our 35th anniversary. It will be the largest event in our history, and we are continuing to innovate with new awards and improved bioproduction zones and content. There has never been a better time to attend CPHI especially as we return to Lombardy – a region home to one of the most vibrant hubs for pharmaceutical manufacturing and API production. I look forward to welcoming the global pharmaceutical supply chain, its partners and drug innovators to Italy. The partnerships made this week will empower the future drug development and ultimately improve patient access and treatments" added Sherma Ellis Daal, Brand Director at CPHI Milan.

In addition to the huge exhibition, networking opportunities and learnings, in the spirit of celebration and in keeping with 'La dolce Vita' of our host city, CPHI will host a number of evening events.

What's new?

This year's edition promises several new features designed to enhance the experience for all attendees and exhibitors:

Supply chain resilience: Stay ahead with insights into new technologies and strategies essential for diversifying and stabilizing the supply chain. CPHI Milan 2024 will showcase the latest innovations and practical solutions to navigate the complexities of the pharmaceutical landscape. The event will focus on strategies for mitigating risks, ensuring continuity, and optimizing operations within the supply chain, all of which are crucial in today's volatile market environment.

Awards Ceremony. On October 8, at Alcatraz Milano, the CPHI Awards Night will commence with a glamorous drinks reception, celebrating excellence and innovation in the pharmaceutical industry across 14 categories. Among the highlights is the new "Woman of the Year" award, honoring women leaders who inspire colleagues, champion diversity and inclusion, and advocate for industry progression. Nominees are evaluated on their leadership, collaboration, and ability to create opportunities for women, while challenging the status quo.

Equally anticipated is the novel "Future Leader" award, recognizing rising stars under the age of 35, with at least five years of experience in pharma, biopharma, or academic institutions. This award celebrates those who bring fresh perspectives, manage teams effectively, and introduce innovative ideas, marking them as the future leaders set to shape the pharmaceutical industry with their dedication and vision.

Digital platform. Enhance your experience with our upgraded online platform, designed to facilitate year-round networking with industry leaders. Whether you're onsite or remote, maximize your engagement and stay connected beyond the exhibition floor. This robust digital infrastructure not only extends the reach of the event but also amplifies its impact on the global pharmaceutical community. Participants can engage with peers, speakers, and exhibitors throughout the year, fostering continuous dialogue and collaboration.

Conference program highlights

The conference program is meticulously crafted to inspire and inform attendees. Industry thought leaders will lead insightful sessions, addressing critical topics such as regulatory trends, technological innovations, and sustainable practices. From visionary keynote addresses in pharmaceutical

R&D to interactive panel discussions on emerging therapies and market dynamics, the agenda promises a wealth of learning opportunities. Learn and gain insights from 150+ expert speakers across 5 content tracks. Key sessions include:

Keynote: Advancing Immunization with Next-Gen mRNA Vaccines

- *When:* Tuesday, October 8, 2024, from 11:00-11:30 AM
- *Where:* Hall 3 - 3F97
- *Speaker:* Cesar Sanz Rodriguez, Vice President, Medical Affairs EMEAC

Delve into the future of vaccination technology as Moderna's market experts unveil the potential of mRNA vaccines. Discover how these next-generation vaccines are reshaping disease prevention and explore the forefront of medical innovation.

Navigating Regulatory Landscapes: Ensuring Quality and Compliance in Pharma Ingredients

- *When:* Tuesday, October 8, 2024, from 1:15-1:40 PM
- *Where:* Hall 22 - 22F50
- *Speaker:* Jyotsna Agnihotri, Head of QA and Regulatory Affairs at Flavine

Gain a deep understanding of GMP principles in pharmaceutical ingredient manufacturing. Learn about auditing processes, including planning, execution, reporting, and follow-up actions. This session is crucial for ensuring compliance and continuous improvement. We'll also discuss comparative analysis of regulatory frameworks across key regions, focusing on differences, similarities, challenges, and effective strategies.

Panel Discussion: Why the Middle East Can Be the New Frontier for Next-Generation Medicine

- *When:* Tuesday, October 8, 2024, from 4:15-5:00 PM
- *Where:* Hall 22 - 22F50

Join our panel of experts as we delve into the emerging opportunities in the Middle East. Discover why it could be the next frontier for groundbreaking medical advancements.

Panel: Excipient Excellence: The Power of Excipient Grade Selection

- *When:* Wednesday, October 9, 2024, from 11:35 AM to 12:45 PM
- *Where:* Hall 22 - 22F50

In this session, we'll delve into the critical role of excipients in pharmaceutical formulations. Discover how excipient quality significantly influences drug performance and stability. Learn about the nuances between different excipient grades and their impact on formulation outcomes. Additionally, explore strategies for ensuring consistent access to high-quality excipients and selecting the optimal grade for targeted drug delivery.

Join us

CPHI Milan 2024 is more than an exhibition – it's a catalyst for change, innovation and advancement within the pharmaceutical industry. With a renewed focus on bioproduction, attendees will gain actionable insights into navigating challenges, optimizing operations, and ensuring continuity in supply. The future of pharmaceutical outsourcing, drug development and innovation is being shaped by the collaborative partnerships and supply side networks built at the event. As pharma looks to lower costs and deliver new therapies to patients the collaborations between CDMOs, pharma equipment manufacturers, ingredient suppliers and pharmaceutical companies will be integral to growth.

Don't miss your chance to be part of building the future of pharmaceuticals. Join us in person at Fiera Milano, Italy, and immerse yourself in a dynamic environment where ideas flourish and partnerships thrive. Together, let's shape the future of pharma. Visit CPHI Milan 2024 to learn more and register today!

La 'Farma' Vita

Fiera Milano: the venue itself, like the wider city, places a much greater emphasis on ensuring attendees have downtime amenities with a wide range of cafes, outside spaces and restaurants – so when you are not empowering pharma you can embrace the spirit of Italy.

CPHI will be hosting happy hours each day of the show and special networking drinks at the Start-up Hub, as well as the eagerly anticipated CPHI Celebration party.

The CPHI Celebration is an exclusive evening of top-level networking, drinks and canapés, set against a backdrop of innovation as we announce the 2024 CPHI Pharma Awards winners. The curated guest list of exhibitors and nominees brings together the brightest minds in our community to make powerful connections in a relaxed environment.

CPHI Milan

The Multifaceted *Future of Pharma*

We asked over 100 industry professionals for their views on the future of the pharma and biopharma industries, including key disrupters and what can be improved… The key trends? AI, cell and gene therapies, and a need to reduce costs and improve patient access.

The Medicine Maker launched in September 2014. (Happy 10th Anniversary to us!). Our mission then and now: to showcase the human side of drug development and manufacturing. There is a huge amount of passion for science and medicine in this industry, but all too often that enthusiasm is stripped away when it comes to writing or speaking about the field, with professionals adopting a sterile business-centric or technical voice. Ensuring the science and technical facts are communicated is vital, of course, but it's not only the research itself that can be inspiring. Human stories – career stories and personal stories – can also inspire. In The Medicine Maker, we offer both, sharing intriguing research and manufacturing stories but also diving into personal journeys – the latter exemplified by our ever-popular "Sitting Down With…" series.

Topics covered in our inaugural issue included the microbiome, neglected diseases, flexible facilities, digital transformation, and the road to commercialization for stem cells (if you'd like to take a look at our first ever issue then check out our website).

Ten years on, we now have FDA approvals for microbiome therapies (the first came in 2022), neglected diseases remain neglected but have seen increasing attention, flexible facilities have become the kings of manufacturing, and digital transformation is one of the hottest industry topics as companies now look to AI and Industry 4.0. Though stem cells may not have seen much on the commercialization front in the last decade, cell therapies have gone from zero to hero.

It's amazing what can happen in 10 years. Actually, it's amazing what can happen in the space of a few months… Remember November 2019 when we were all blissfully unaware of what would happen in the first part of 2020?

Sure, we're looking back a little while celebrating our 10th birthday, but what's most exciting is the future. Personalized medicines that can be 3D printed at home, robots patrolling pharma facilities, AI algorithms that monitor health and help prevent disease from ever occurring, spaceships going to the far reaches of the galaxy, Star Trek-esque tricorders…

Okay, maybe that's going too far ahead, but the opportunities in front of us are near endless.

We reached out to you – the medicine making community – to ask for your views on the future of pharma and healthcare, including key disrupters and the big changes we can expect in the coming years.

Overwhelmingly, well over 100 of you submitted your thoughts. And so, we decided to release The Medicine Maker's Multifaceted Future of Pharma over the course of 10 exciting chapters. You can access a new chapter each month at: https://themedicinemaker.com/the-multifaceted-future-of-pharma.

Over the next few pages, we'll give you a taster of just some of the insights shared.


Chris Arendt

TRANSFORMATION THROUGH AI AND MACHINE LEARNING

With Chris Arendt, Chief Scientific Officer and Head of Research, Takeda

"The integration of AI and ML technologies stands to dramatically transform the biopharma industry. Iterative in silico learning systems that benefit from large training datasets and that integrate wet lab validation experiments are starting to deliver significant gains in the quality and speed of target identification, hit finding, and candidate optimization. The preclinical proof-points are encouraging, and the hope is that these strides translate into higher quality clinical candidates delivered more quickly to the patients who can most benefit.

"On the development front, generative AI-based tools may evolve to be able to support patients and practitioners in navigating treatment algorithms and clinical trials. Ensuring that patient genetics, medical histories, and symptoms are weighed against all available clinical study data and real-world evidence could provide optimized, personalized treatment algorithms. Promising clinical trials could also be flagged on a personalized basis based on totality of evidence, including the mechanism of action of the investigational agent and the knowledge base around the relevant disease subtype. Management of privacy and consent will be critical to the integrity of these approaches.

"We may not be far from a future where AI and ML can assist patients and physicians in predicting and managing health outcomes prior to disease inception, as well as in the early stages of disease. The ability to integrate massive real-world datasets with real-time health monitoring and diagnostics holds exciting potential. To really move the needle on human health, we need to develop and deliver medicines much closer to disease inception, when the biology is at its most malleable."

LEARNING TO COLLABORATE

With Miriam Monge, Head of Marketing Strategy & Customer Advocacy, Sartorius

"One thing that could dramatically change the pharmaceutical industry would be to adopt the lessons we learned during the pandemic around collaboration.

"To that end, BioPhorum Supply Resilience is scoping out a 'crisis response team' to proactively manage industry readiness and response from 'detection to escalation' of any future crisis impacting our biopharmaceutical manufacturing industry.

"The question is, how can we act collectively as one biopharmaceutical manufacturing industry? There is a need today for a consistent voice that's representative of all major biopharma agencies, but separate from the guidance of the pharmaceutical industry – because our production processes and requirements are so starkly different, all the way down to the molecule.

"For example, during the COVID-19 pandemic, 182 separate vaccine projects were initiated. When similar events occur in the future, we must be able to work with agencies and organizations, such as CEPI, GAVI, and the WHO, to collectively decide how many projects receive approval and prioritize supply efficiently.

"As WHO Director-General Tedros Adhanom Ghebreyesus puts it: 'The world must be ready to respond to the next pandemic. When the next pandemic comes knocking – and it will – we must be ready to answer decisively, collectively, and equitably.'"

BEATING SOLID TUMORS WITH CELL THERAPY

With James Lim, Chief Scientific Officer, Xcell Biosciences

"For all the excitement around cell therapies to treat cancer, the reality is that most cell therapies today are only effective for hematological malignancies. The greatest improvement for pharma would be translating the success of cell therapies to solid tumors, which make up the vast majority of cancers. This would be a game-changer for the industry, and a literal life-saver for patients.

"The fundamental reason cell therapies are stymied by solid tumors is their near-toxic microenvironment. Rife with immunosuppressive mechanisms, low oxygen levels, and high interstitial pressure, tumors thrive in an environment hostile to most cells. Evaluations of therapeutic cells that have been delivered to solid tumors found them to be depleted and dysfunctional. Tumor infiltrating lymphocyte therapy, the only class of cell therapies currently approved for a solid tumor indication, has existed for decades, and yet the challenges of manufacturing these cells to dose while maintaining their potency has stymied progress.

"But there is hope. Mounting evidence suggests that growing cell


Miriam Monge

Monika Paule

James Lim

therapies under conditions more like the tumor microenvironment can make them hardier. Metabolic conditioning with advanced incubators that allow users to fine-tune more parameters, including oxygen and pressure levels, can help acclimate cells, preparing them to survive in an environment that might otherwise kill them. This approach is at odds with the conventional wisdom that therapeutic cells should be grown in conditions that keep them as happy as possible in vitro. Yet it is supported by more and more studies showing that metabolic conditioning can lead to more potent and more abundant cell therapies. This new avenue could dramatically expand the use of cell therapies across a wide range of cancers."

CRISPR MEETS AI

With Monika Paule, CEO, Caszyme

"I envision a future where CRISPR gene editing revolutionizes healthcare through personalized gene therapies tailored to

individual genetic profiles. By precisely correcting underlying mutations, CRISPR has the potential to surpass traditional therapies, offering superior treatment outcomes across a wide range of conditions. To realize its full potential, the development of novel Cas nucleases with advantages over existing CRISPR tools is crucial. CRISPR gene editing also accelerates drug discovery by swiftly generating and validating disease models, expediting the identification of therapeutic targets, and facilitating the development of innovative treatments, while promising shorter timelines from discovery to market, enhancing efficiency, and increasing accessibility of novel therapeutics.

"Integrating other innovative approaches, such as AI, with CRISPR technology amplifies the potential impact of gene editing even further. AI can optimize CRISPR gene editing systems, accurately predict editing outcomes, and refine therapeutic strategies, enhancing treatment effectiveness and safety. This synergistic integration could not only transform genetic disease treatments, but also revolutionize global drug development processes.

"I also think that mRNA-based delivery technologies promise to bring significant changes to the therapeutics field. The SARS-CoV-2 pandemic accelerated development and validation of mRNA vaccines, showcasing the immense potential of mRNA with longer-lasting expression of therapeutic proteins leading to prolonged clinical benefits. With further optimization of mRNA composition and established quality control, mRNA technology could revolutionize the field.

"Over the next decade, I anticipate more targeted therapies tailored to individual genetic variations, facilitating faster cycles of innovation and improving patient outcomes across diverse diseases. Unlocking the full potential of new technologies such as CRISPR gene editing, mRNA, and AI will lead to significant changes in therapeutic development and manufacturing."

OBESITY, PERSONALIZED MEDICINE AND NEW TRIAL APPROACHES

With Sarah Browne, Vice President, Clinical Development, Altimmune

"Rather than a one-size fits all approach to drug design and development, personalized therapies and treatment regimens tailored to the unique needs of patients have become a cornerstone of modern healthcare. A great example of this is the industry's innovation in obesity. As a multifactorial disease, obesity has historically been a challenging condition to treat because of the complex interplay of genetic, behavioral, and environmental factors. However, the development of new


Sarah Browne

therapies that mimic naturally occurring hormones in the body has revolutionized obesity management – improving patient outcomes and bringing a renewed focus on treating obesity as a chronic disease rather than a lifestyle issue.

"In the next 10 years, the obesity drug development landscape will continue to evolve as we place even greater emphasis on patient-centric approaches to develop therapies with enhanced tolerability and enable more precise targeting of obesity-related pathways, leading to treatments that are more effective for specific patient subgroups – addressing cardiovascular health, liver health, metabolic health, and more.

"I also expect we'll see the integration of emerging tools and technologies, such as AI and wearable technologies, into clinical trials to enable accessible and equitable approaches to research and innovation. Integrating AI into clinical trials can drive enhanced efficiency from the patient selection and enrollment process to more robust measurement of clinical endpoints. In preserving invaluable time and resources, while bolstering non-invasive measurement capabilities, these improvements stand to reduce the costs associated with traditional drug development. We can also leverage new technologies to implement decentralized clinical trials. Such trials would allow us to reach a broader, more diverse range of patients including those who may have been previously undertreated

and understudied because of limited access to healthcare and research trials."

"Over the next decade, I anticipate more targeted therapies tailored to individual genetic variations, facilitating faster cycles of innovation and improving patient outcomes across diverse diseases. Unlocking the full potential of new technologies such as CRISPR gene editing, mRNA, and AI will lead to significant changes in therapeutic development and manufacturing."


Tony Lakavage

ADDRESSING ISSUES WITH GENERICS

With Tony Lakavage, Senior Vice President for Global External Affairs, USP

"Over the past 10 years, we have watched drug shortages increase while the market for generic medicines has become a race to the bottom where purchasers focus almost solely on the lowest price. According to our findings and other studies, price erosion at the center of the drug shortage crisis is causing industry contraction. "Over two-thirds of the medicines we use today are generics. Nearly every approved drug that is off patent today has one or more generic competitors, compared with only 35 percent prior to the passage of the Hatch-Waxman Act in 1984. The generics industry has saved American taxpayers nearly $3 trillion over the past ten years, according to some estimates, and even more for patients worldwide.

"However, with the current state of the industry, the savings and access to treatment that generics provide are at risk. Unsustainably low prices are forcing manufacturers to leave the market because of razor-thin margins or a lack of resources to invest in infrastructure and quality improvement. The length of drug shortages, including for life-saving medicines, increased from two to three years from 2022 to 2023.

"Low prices for generics do not

reflect – nor do they provide – a viable return on investment to justify redundancy in manufacturing or next-generation quality improvements. Without intervention, the industry will remain in crisis, and we risk not being able to get critical generic medicines to patients when they need them. As we look to the next 10 years and beyond, policy makers must begin implementing systemic changes that will ensure patient access to necessary medicines – including incentivizing supply chain quality, resilience, and reliability; promoting payment reforms; investing in capabilities to increase supply chain visibility; and strengthening modern manufacturing technologies. We must address the multifaceted issues that have undermined the generics market."


Claes Gustafsson

IMPROVING PHARMA'S REPUTATION

With Chelsea Pratt, BioPharma Segment Marketing Manager, Bio-Rad Laboratories

"The world depends on the pharmaceutical industry for the development of life-saving therapies and vaccines, yet trust in the sector is low. In 2023, a US Gallup poll revealed that the public's perception of the pharmaceutical industry has declined over the last year, receiving the lowest positive rating ever recorded for the industry: 18 percent. This is even lower than the notoriously controversial oil and gas industry, which 24 percent of the public viewed positively.

"A significant factor contributing to this negative perception is lack of education and accessible information surrounding vaccinations and other medical advancements. This was exacerbated during the COVID-19 pandemic, where misinformation spread throughout the general population, highlighting the lack of effective communication outlets for non-scientific audiences.

"As a result, mistrust of pharmaceutical companies has led to a decline in childhood vaccination rates in the US over the last few years, leaving many children at risk for preventable diseases. Vaccines are victims of their own success; with their effectiveness overshadowed by negligible side effects, which the so-called 'anti-vax' community amplifies. This public mistrust may also deter investment into medical advancements as investor confidence wanes.

"To improve the industry's reputation, a shift in communication strategies is essential. Instead of only taking a sales-focused approach, pharmaceutical companies could also prioritize raising awareness about the drug development process and the scientific principles behind novel therapeutics. Integrating transparency into drug discovery workflows can provide the public with insights into the faces and rigorous science behind medications, helping, in turn, to re-instil trust in the industry. This approach will not only elevate company branding, but also have a broader impact on advancing medical innovations.

"In addition to providing better access to resources, increasing investment in STEM education and learning opportunities will help future generations understand the benefits of vaccinations and other novel therapeutics, fostering a positive perception and deeper understanding of the pharmaceutical industry. By prioritizing transparency and accessibility through improved communication strategies, the industry can secure a better future for healthcare innovation and public acceptance."


Chelsea Pratt

ROOM FOR IMPROVEMENT IN BIOPROCESSING

With Claes Gustafsson, CCO, Atum Bio

"Over the past century, production processes for small-molecule drugs have matured significantly, allowing for the large-scale manufacturing of even complicated organic molecules efficiently and at low cost. Notably, 2010 was the last year when all the top ten drugs were small molecules. The introduction of Humira and other antibody-based drugs shifted the trillion-dollar pharmaceutical business from a chemical to a biological industry almost overnight. In the 2023 top ten list, only two of the top ten drugs were small molecules (Eliquis and Biktarvy). Instead, biologics now dominate the drug market, with six of the top ten drugs being antibodies, one being RNA, and one a peptide. The upcoming waves of future drugs are predominantly biologics as well, with acronyms like BsAbs, CAR-T, AAV, TCR, ADC, and many more on the horizon.

"However, the cost, speed, consistency, and complexity of biologics manufacturing lag far behind those of small-molecule chemistry manufacturing. Biological production systems are inherently unstable because of evolutionary factors, an abundance of epistatic variables complicating predictability, and the large number of atoms in each drug molecule complicating consistency and homogeneity.

"There is an urgent need for the biotechnology industry to implement standardization, data tracking, and statistical engineering tools from the design all the way to the manufacturing of biologics. While AI has been a major discussion point since the 2022 release of ChatGPT, no amount of NVIDIA chips and multi-node neural nets can help if the underlying functional data is missing, poorly distributed in multidimensional space, or prone to non-systematic variance. Over the next few years, the drug development and manufacturing industry should focus on designing systematic experiments and data capture in an integrated platform to efficiently seize biological sequence-function space to generate new and improved biologics and manufacturing processes."

CONQUERING THE UNDRUGGABLE

With Anshul Gupte, Vice President of Pharmaceutical Development, PCI Pharma Services

"Over the coming decade, one prominent category likely to drive substantial changes in drug development and manufacturing is AI. Coupled with AI, the areas that bring promising potential include age-appropriate and/or personalized therapies, orphan and rare disorders, real world data-driven decisions related to decentralized clinical trials, and enhanced sustainability in both manufacturing and supply chains.

"Ideally, drug development in the next decade should be driven by advanced delivery of large molecules, nucleic acids, and drug candidates for so-called "undruggable" targets through novel drug delivery technologies. As AI continues to turbo-charge discovery, development, and manufacturing elements, partnerships with CMOs and CDMOs, for example, will become all the more important in optimizing drug product design, development, and logistics for expedited time to market. Considering the advent and rapid proliferation of AI in drug development – and the need to provide patients with life-changing therapies as quickly as possible – augmentation and adaptations to current regulations in areas such as breakthrough, accelerated and fast-track pathways will be necessary.

"Regardless, all signs for the next decade point to customization, with drug manufacturing geared toward targeted, niche medicines produced in multi-product facilities offering potent and highly potent drug-handling capabilities, batch production flexibility, and agile and integrated manufacturing, testing and packaging services that bridge gaps between therapies and patients.

"Finally, the global drug manufacturing community has a shared responsibility to minimize greenhouse emissions, lower waste generation, and reduce water and energy consumption. The increased emphasis on environmental, social and governance issues across various aspects of the pharma sector has been highly encouraging."

Anshul Gupte

HELPING PATIENTS SAVE MONEY

With Christine Marsh, Senior Vice President of Value and Access, Boehringer Ingelheim

"As we think about how to improve the industry, innovation is at the core of nearly everything we do. This starts with developing life-enhancing treatments for patients, but we also must innovate when it comes to improving patient access. When patients walk up to a pharmacy counter or order a prescription online, they shouldn't have to choose between their physical health and their financial health. As an industry, we recognize how crucial it is to lower financial hurdles and expand access to essential medicines.

"In my time at Boehringer Ingelheim, which has spanned more than two decades, I can say that we truly believe in ensuring access to medication through affordability. It shapes our decisions and is foundational to our mission to enhance patients' lives.

"It would be easy to simply say we need to do it, but words must be put into action. Our BI Cares Foundation, for example, invests resources to help people in need get medicines for free. In 2024, we partnered with GoodRx to provide a low-cost option for our biosimilar, making it the first biosimilar with a low cash price on the platform. This is available to any patient with a valid prescription – whether they are insured or not. We also launched a first-of-its-kind program that automatically reduces the costs of our COPD and asthma inhalers to $35 for the vast majority of eligible patients.

"It's important to continue to look for innovative ways to help our patients save money because reducing the cost burden extends beyond someone's bank account – it's also about restoring hope and improving lives.

"Driving down medication prices isn't just about what one company can do – it requires dedication from the broader healthcare ecosystem. We need to foster competition and continue to collaborate. This is a collective effort, where we need each stakeholder's full participation to create a world where access and affordability are the top priority. It not only benefits patients but also adds to a more sustainable healthcare system."

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At the heart of Pharma

Built For You

How Asahi Kasei Bioprocess is incorporating ergonomics into biopharma equipment to make manufacturing safer, more efficient, and more productive.

What's the secret behind great equipment? High-performance and high quality should be a given in the world of biopharma, but we shouldn't forget the overall design and useability of a system, including its ergonomics. Asahi Kasei Bioprocess (AKB) designs and manufactures large-scale biopharmaceutical manufacturing equipment that is deployed all over the world – and it prides itself on deep collaboration with customers. Part of the company's ethos is "Built For You" – a philosophy that ensures equipment is truly made to help biopharma companies to develop the best products possible, in a safe and efficient manner.

Here, we speak with Steve Foy, Manager, Products and Brand Strategy, to find out more.

What inspired AKB's "Built For You" philosophy?

Our mission and vision are centered around being a trusted advisor for customers. The only way to achieve that is to partner with customers – and that means developing and fostering relationships. It shouldn't just be about the sale of products and equipment; we have a duty to help our customers perform to the best of their ability.

We're already well-known for custom equipment, but our "Built For You" philosophy is about more than that. It is a broader story that goes beyond custom equipment into a deep understanding of customers and their specific requirements, and our willingness to design solutions on their behalf to help them be more productive.

Why is ergonomic equipment design so important?

Ergonomics have naturally been an important aspect of our equipment design for a long time, but we've never really focused specific messaging around it. Ergonomics is the study of ease and comfort – and a steppingstone to greater efficiency and safety – and plays a key part in "Built For You". An ergonomic chair, for example, increases efficiency and productivity for somebody working at a desk by increasing their safety and comfort. Poorly designed equipment can potentially reduce productivity and increase risks for the operator, the product, and the company in general.

To give an example of how ergonomics affects pharmaceutical manufacturing, consider our commercial-scale CURSIV™ DAC Ergo liquid chromatography column. Large columns can be difficult and tedious for operators to use and maintain. The DAC Ergo works on a hydraulic system rather than on a solitary frame. The telescopic legs are much larger and separated from the column. A single operator can remove the bottom plate assembly in roughly 30 minutes, which makes unpacking and maintenance much easier.

Is ergonomics typically overlooked by customers?

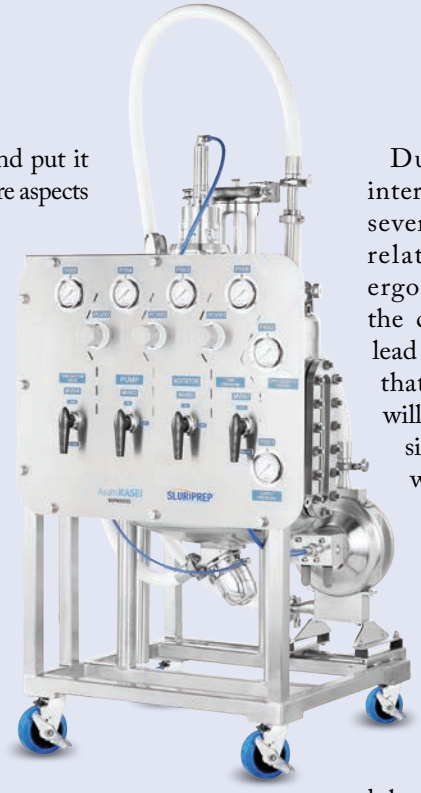
Customers often focus on the technical specifications of equipment. Certainly, equipment needs to have the right specs and deliver high performance. However, if your equipment isn't easily usable – for example, if it is difficult to operate or to learn how to use – then it will be an obstacle to optimized productivity.

In addition to looking at the technical details, we always advise customers to think about ergonomics. For example, look for a human–machine interface screen that is positioned appropriately – not too high or too low; look for a keyboard and a touchscreen input option to make it comfortable for the individual operator. Customers should also think about maintenance and accessibility. Are all the parts inside easy to access? The alternative is to take everything apart, retrieve and

replace a component, and put it all back together. These are aspects that customers may not consider outright.

As well as ensuring our equipment is high quality, high performing, durable, robust, and reliable, we always look at how we can make things more accessible, more efficient, and simpler. All these factors can have a big impact on day-to-day operations. There is a level of productivity that a customer may not even know they're able to achieve. And so, it is up to us to focus on that and innovate on the customer's behalf. Even if they've become familiar and proficient with older designs, could they still save hours of downtime when it comes to process changeover? That could mean extra batches per week.

One driver for the design for our recently released THESYS™ ACS Ergo column was input from customers. Our typical synthesis columns are very high performing – and popular with customers all over the world. But changing the media after synthesis involves unscrewing a series of threaded bolts, which can be a bit tedious. After listening to feedback, we modified the design to improve usability and productivity.



How does AKB work with customers during the design process?

We start by understanding the space we must work with. Very early in the design phase, we ask the customer for a floor plan of their facility that shows the route the equipment will take from the loading dock, as well as the room and location for installation. We note the maximum dimensions that can be safely wheeled through the hallways and around corners, the sizes of entryways, and areas to avoid placing routine maintenance items.

During our formal internal review, we have several checklist items related to safety and ergonomics. We verify the constraints with the lead engineer and ensure that the designed skid will fit. We look at every single component that will require routine maintenance or access to control panels and consider ease of use, such as the position of pump faces and pump motors, the support of heavy valve blocks and other components, and the accessibility of control panels for sensor calibration.

We locate the HMI and keyboard at commonly accepted heights for ergonomic operation and, during our formal external review with the customer, we suggest they include representatives from their maintenance and metrology departments. We then go through all these points with the customer and come out with alignment or suggestions to better meet their needs. It is not uncommon for us to export a 3D model of the system for the customer to import into their plant plans for further confirmation that all components and ports are located to provide ease of use.

Why should new customers consider working with AKB?

I am deeply proud of our people and their ability to develop great equipment and form great relationships. In addition to providing high quality equipment with excellent performance – which can be table stakes – we focus on ergonomics and usability as key selling points. Another differentiator is the service we offer and how we communicate with the customer – from sales and the proposal process, to design, manufacture, installation, and field service.

Examples of Ergonomic AKB Technology

- CURSIV™ DAC Ergo – liquid chromatography columns with a patented design to ensure swifter, safter column changeover between batches.
- THESYS™ C&D Systems – designed to accommodate both DNA and RNA processes with numerous in-process controls to automated steps.
- THESYS™ ACS Ergo – synthesis column designed to simplify time between batches by being built for faster unpacking and cleaning.
- MOTIV® Single-Use Inline Buffer Formulation System – streamlines buffer production by removing CIP/SIP between batches.
- VANTIJ™ Single-Use Virus Filtration Controller – includes features and design elements for reproducible, reliable filtration.
- SLURIPREP™ line of media management products – designed for DAC LC columns to ensure seamless, efficient operation.

A cornerstone of AKB is our collaboration with customers. Our success is derived from our customer's success. Our equipment plays a crucial role in bringing new therapies to patients and we have a responsibility to supply the best solutions that can help customers to be as productive and efficient as possible.

https://fluidmgmt.ak-bio.com/

AsahiKASEI BIOPROCESS

CELL AND GENE

The Elite Athlete Concept for Cell Therapy

How metabolic conditioning could improve cell therapy potency and persistence

By Yelena Bronevetsky, Director of Product Management, and James Lim, Chief Scientific Officer, both at Xcell Biosciences

Efforts to improve cell therapies inevitably focus on boosting potency through genetic engineering. If we could design tumor-targeting constructs and perturb exhaustion signaling pathways, surely, the resulting therapies would have the desired effect. However, there's a more reliable and straightforward approach. Scientific evidence supports that incorporating metabolic conditioning, whereby cells are exposed to oxygen and pressure levels found in tumor microenvironments, during development and production of cell therapies will give patients the best chance at healthy outcomes.

Let's take a step back to consider the specific improvements needed in the cell therapy space. First, existing therapies targeting cancer tend to work well in just a small fraction of patients and we need this success rate to be higher (1); lack of response to a cell therapy should be a rare event. Second, current therapies have been predominantly approved for treatment against blood cancer, but with liquid malignancies representing just 10 percent of cancers, there is a pressing need to expand the utility of cell therapies to solid tumors.

To address both of these challenges, we should develop cell therapies with greater potency and persistence. Reaching solid tumors, for example, has proven difficult because cell therapies have to travel long distances to get from the bloodstream to the tumor site – and often arrive depleted. Any cancer-killing function that remains cannot be put to work until the cells overcome suppressive forces, such as low oxygen, high pressure, and the presence of cell populations designed to block T cell function.

Though conventional wisdom says the answer is to genetically engineer cell therapies to withstand these forces, existing data indicates that fine-tuning cell culturing conditions may offer the solution. Here's the theory; cells facing a hostile environment will function best if they've been trained to survive in that environment already. We like to think of this as the "elite athlete" concept; elite athletes train for the conditions they know they'll face, whether that's training at high altitudes for elite cyclists or running steep hills for marathoners preparing to race in San Francisco.

For cell therapies, that training occurs in culture as the cell population expands. Though culturing is typically performed under conditions designed to keep cells happy and dividing as quickly as possible, scientists have run a number of studies showing that harsher conditions may lead to better outcomes in vivo. Restricting the availability of glucose in culture, for instance, leads to cells with enhanced antitumor function (2). Growing cells under hypoxic conditions during the T cell activation period results in stronger cytotoxic function in vivo (3). Other changes to metabolic conditioning regimens have shown promise in boosting cell therapy efficacy – even for solid tumors (4, 5). The cytokine composition of cell culture media also has an important role to play. Though the standard IL-2 regimen leads to better growth in culture, shifting to other cytokines, such as IL-7, IL-15, and IL-21, reduces in vitro expansion but leads to increased potency and persistence once infused.

Taken together, the evidence suggests that the efficacy challenge with cell therapies may not be a genetic engineering weakness, but a conditioning one. The focus on giving cells culturing conditions that solely prioritize rapid expansion leads to cells that thrive only in perfect conditions, but become depleted and ineffective in the harsh reality of a tumor microenvironment. Growing cells instead under the kind of low-oxygen, high-pressure conditions they will experience at the tumor site may not produce ideal results in vitro – cells can divide far more slowly – but once in the body, they are more likely to exhibit potent anti-tumor activity. Toughening up cells in culture provides the training they need to excel in harsh solid tumor microenvironments.

This idea, too, has been supported by research studies. Scientists have demonstrated that CAR T cells targeting the ROR1 protein appear to have excellent tumor-killing potential based on in vitro assay performance conducted under ambient oxygen conditions (6). But when introduced in humans, this function is significantly reduced. Animal studies demonstrated the ability of ROR1 CAR T cells to traffic to the tumor site, but also found they exhibited poor anti-tumor activity and persistence leading to disease progression. Had those cells been cultured under conditions more like the tumor microenvironment, they may have exhibited improved potency and persistence.

Incorporating more biologically relevant conditions during cell culture is an important avenue to pursue, but it should not be left to the final stages of cell therapy manufacturing. We believe that an early and lasting focus on cell conditioning could greatly enhance the performance of cell therapies. From preclinical to process development to manufacturing, a focus on conditioning cells to the tumor-specific environment – rather than on the absolute number of cells grown – may provide the best chance to improve potency and persistence. Ultimately, this approach could help address some of the biggest challenges in the cell therapy field today.

See references online at: tmm.txp.to/0924/elite-athlete

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BIOPROCESSING

A Love for Complexity: ADC Drug Development

Yoonjin Kwon, Lead Scientist, ADC Analytical Method Development, Samsung Biologics, offers her perspective on the future of antibody drug conjugates

How did you first get involved with the field?

I started my journey with antibody-drug conjugates (ADCs) as a junior analytical scientist at a biotechnology company after earning my master's degree. Then in 2014, I moved to Samsung Biologics to lead monoclonal antibody development. With the recent launch of the ADC service, my work has come full circle as I return to working with ADCs. Today, my role focuses on managing ADC development timelines in a way that mitigates risk while providing flexibility. Our ADC plant is under construction, so we have an opportunity to manage the ADC development process correctly – right from the start.

I take pride in contributing to optimal ADC development infrastructure. Soon, I envision working in Samsung Biologics' dedicated ADC facility that would enable me and my colleagues to readily problem-solve any unexpected deviation, and thus execute ADC projects of various scales on time and without compromising quality.

As one can rarely predict when an issue may occur during ADC development, creating and then nurturing a culture of problem-solving readiness is especially important – not only for the safety of scientists, but also for the quality of ADC product delivery. Whenever I have time to spare during my packed lab schedule, I enjoy sharing my know-how on how to keep calm and readily cope in the face of unexpected problems in ADC development!

What makes these therapies so interesting?

The development of an ADC is a fascinating process that requires various complex analytical methods because of the different components involved, including the mAb, linker and the potential cytotoxicity of the payload. To create an ADC, you must optimize the drug-to-antibody ratio, drug load distribution, conjugated antibody, and naked mAb. Separate methods are needed to confirm the identity and purity of the payload, the linker, and the successful attachment. Along with the individual components, the functionality of the ADC needs to be characterized by understanding how to evaluate the conjugation site based on the manufacturing process. Fortunately, analytical techniques have improved alongside ADC candidates. We now have access to approaches, such as liquid chromatography-mass spectrometry (LC-MS), that are incredibly useful for ADC characterization.

None of these tests begins to characterize the potency of the ADC itself. Here, cell-based assays are needed to profile the potency and effectiveness of the ADCs – a crucial step.

Why has ADC drug development faced so many challenges over the past decade?

The previous challenges of ADC development were because of limitations in earlier ADC technologies. The first generation of ADCs suffered from toxicity issues because of the novelty of the linker. However, as ADCs progressed, so too has linker technology, reducing the rates of off-target toxicity and improving patient outcomes. We are now in the third generation of ADC therapies, where site-specific conjugation can improve the safety profile and target specificity. Through experience, we can now better predict site toxicity before a therapeutic enters clinical trials.

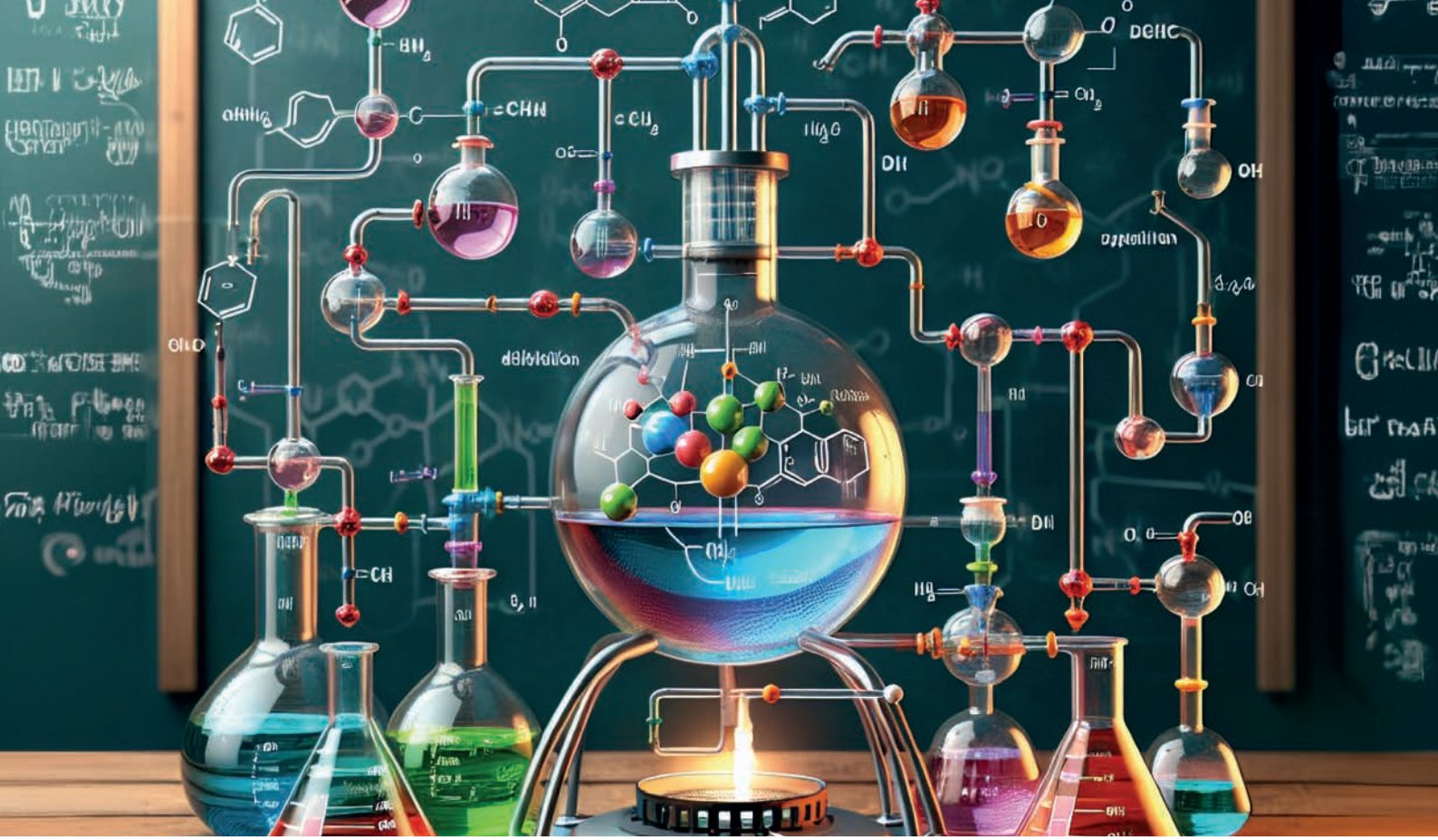
What other key trends are you seeing in this space?

Combination therapies – and that reflects a trend we've seen globally within the cancer therapeutic space. Bioconjugates are particularly exciting because of the many combinations available for experimentation: mAb with cytotoxic molecules (maytansinoids, auristatins, camptothecins, calicheamicins, PBDs, duocarmycins), mAb with protein toxins (diphtheria toxin, Pseudomonsas exotoxin), mAb with radionuclides (131I, 177Lu, 90Y, 225Ac), mAb with nucleotides (siRNAs, antisense), mAb with immunomodulators (STING agonists) and mAb with protein degraders. A second trend is the miniaturization of therapeutics. As we understand and optimize ADC therapies, we may be able to deliver effective treatments that do not require a complete antibody. I also see a trend towards bispecific antibodies (which can bind two antigens at the same time).

What's next for the ADC field?

ADCs continue to advance rapidly. We understand how to use ADCs to activate the immune system to destroy cancer cells, allowing us to design targeted agents and potent payloads. Alongside ADCs, new companion diagnostics will enable us to match patients with the correct therapeutic for maximum response.

There are also exciting applications for bioconjugates beyond ADCs. With our advanced understanding of linker technology, we can combine two other modalities to create new functionality. For example, new bioconjugates could pair a vaccine with different polysaccharides – or nanoparticles could be combined with small molecules to deliver targeted treatments. These exciting technologies can help us push into the next generation of personalized medicine.



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SMALL MOLECULES

MR Control

Developing modified release formulations can be complex – but a translational pharmaceutics approach can smooth the path

By Asma Patel, VP, Integrated Development Services, and John McDermott, VP, Scientific Consulting, both at Quotient Sciences

Small molecule drugs still make up the majority of newly approved medicines. In 2022, 57 percent of FDA drug approvals were for small molecules

Traditionally, molecules for oral administration have been formulated into immediate release (IR) products with a rapid onset of action. Although instantaneous relief can be advantageous for the patient, these formulations can also suffer from a short duration of therapeutic effect, resulting in the need for more frequent dosing. And because the drug immediately enters the bloodstream, it can cause significant peaks and troughs in circulating drug concentrations.

To overcome these limitations, modified release (MR) formulations can be designed that allow more control over the rate and location of drug release in the GI tract. MR formulations can also provide added benefits, including i) the maintenance of drug plasma levels, wherein a prolonged period of release stabilizes drug plasma levels, reducing the dosing frequency requirements, ii) attenuation of peak-to-trough ratios, which can lead to lower peak-related adverse effects and improve therapeutic efficacy, and iii) targeted delivery or the specific release of a drug at a particular site in the gut tract (to either target gastrointestinal disease or reduce the impact of delivering a drug to absorptive regions).

Under the broad MR term, there are many formulation technologies that provide individual benefits and support different release profiles, including sustained or extended release, gastric bypass, biphasic/pulsatile release, and gastro-retention. Although increasingly used for the development of new chemical entities, MR technologies have traditionally been adopted for life cycle management strategies. This modest reformulation of changing from an IR to an MR allows for the extension of drug product patents and provides continued market exclusivity.

Navigating MR, however, can be a complex process as differences in the regional absorption of the GI tract, which are not normally a consideration for IR dosage forms, come into play. An example of this is a sustained-release formulation that transits to the GI tract while continuously releasing the drug. Therefore, the drug must be sufficiently absorbed in the lower GI tract to maintain a therapeutic effect. Accordingly, the development team will typically need to conduct multiple cycles of formulation development, in vitro screening, preclinical, and clinical studies to find the balance of drug release rate and dose to achieve the target plasma concentration profile for the MR formulation to succeed.

One way that development teams can improve this optimization process is to employ a "translational pharmaceutics" approach and evaluate a "design space" within a clinical study. The design space concept is rooted in ICH Q8 quality-by-design principles and allows a range of drug release rates and doses to be advanced to clinical assessment. The research team can therefore conduct a series of "make" and "test" cycles to iteratively explore a range of doses and drug release rates. This method considers using the in vivo product performance in humans alongside the ability to correspondingly support changes in drug product composition to identify the optimal formulation composition to deliver the target profile, and quickly and precisely identify the formulation that is effective while meeting patient needs.

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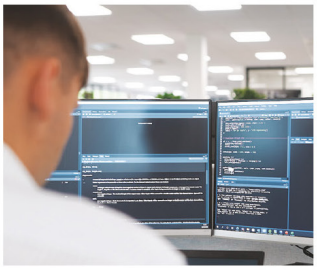

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BEST PRACTICE

Nonprofit Model for Manufacturing

All companies claim to put patients first, but how true is that really when the corporate pharma business revolves around profits? Civica Rx is approaching generic drug manufacturing using a nonprofit model – and it's working. We get the story behind their facility in Petersburg, Virginia.

By Stephanie Vine

groninger nest filling line with SKAN isolator2

Civica Rx was launched in 2018 as a nonprofit drug firm with a focus on essential generic medicines that were being under-delivered in the US market. The problem – in Civica's words: "Unfortunately, when a supplier is able to concentrate market power with [essential generic] drugs, they are able to wield an exorbitant amount of influence on the price."

The company was founded by several US health systems (Catholic Health Initiatives; now CommonSpirit Health, HCA Healthcare, Intermountain Healthcare, Mayo Clinic, Providence St. Joseph Health; now Providence, SSM Health, and Trinity Health) and philanthropic organizations (the Gary and Mary West Foundation, the Laura & John Arnold Foundation, and the Peterson Center on Healthcare). Martin Van Trieste, former chief quality officer at Amgen, came out of retirement to sign on as Civica's CEO – a role he took on with no compensation. At first, 14 generic medicines were identified by the company as being a priority.

Six years later, Civica is supplying nearly 80 different generic medicines to member hospitals – and has provided over 185 million doses to date. It has also opened its own manufacturing facility in Petersburg, Virginia, with the capacity to produce 90 million vials and 50 million pre-filled syringes of medicines every year. Crucially, a study published in NEJM Catalyst found that the company had been able to improve generic drug access through its unique model (1). Civica has also expanded its ambitions to new areas, such as insulin. In 2023, the state of California announced a 10-year agreement with Civica for the manufacture of "CalRx" insulin.

Jason Winfield, site director of engineering and technical services at Civica, thinks that much of the success comes from the company's can-do attitude and the lack of bureaucracy. "From the start, Martin made it our mission to do what's in the best interest of the patients. We continue that motto today. A lot of people are very engaged in our mission – there are a lot of good names on our executive team. As a nonprofit, we are able to come to work to help patients rather than earning extra dollars for shareholders. Not having to go through third party distributors, distribution centers, and pharmacy managers has allowed us to get products to hospitals and patients at a reasonable cost."

Bringing it home

Van Trieste has now headed (back) to a well-deserved retirement, but Civica continues to grow. In March 2024, the company announced a collaboration with groninger and SKAN for new filling lines for their facility. Winfield told me more about the decision – and groninger and SKAN also gave their input.

"This was a really rewarding project to work on," says Matt Clifton, business development manager at groninger. "A lot of people are affected by drug shortages – including people I know personally – in the US. Civica has a real patient-centric focus. I always focus on getting the best machine for the client but this project felt close to the heart."

But why build your own facility – especially in an expensive Western country like the US?

According to Winfield, there has been a big focus for some time on bringing production closer to home. Winfield says, "A lot of pharmaceutical manufacturing takes place in India and China, but there have been widely publicized issues with sterile injectables. And although India is a great provider of APIs, they get a lot of their precursors from China. During the

COVID-19 pandemic, materials stopped moving – and this hasn't ended with COVID; there are still ongoing challenges in supply chains."

Civica already had diversified suppliers of APIs from the start, prioritizing the US and Europe, but drug shortages and the industry's reliance on existing supply chains inspired the company to open the Petersburg plant. But even with its own manufacturing capacity, Civica doesn't put all of its eggs in one basket.

"No more than 50 percent of our hospitals' demand comes from any one facility," says Winfield. "We've moved things around strategically; if there is an upset in any facility manufacturing our private-label drugs, we can source it from a different place. We have second and third line suppliers, but our Petersburg facility is the ultimate control. We can manufacture our own medicines, across the spectrum of presentations, including vials, syringes, and cartridges. If any of our suppliers are unreliable or if there is a market shortage, we can quickly ramp up manufacturing at Petersburg. We've also got technologies at the plant that allow us to have quick transfer of products in and out."

Choosing equipment – and suppliers

Winfield got his role at Civica in 2020 – after the decision to build a facility had already been made. Two weeks before he was officially due to start his new role, he was brought in for critical decision making on what filling lines to purchase. An architect engineering general construction firm had proposed using filling lines from different vendors – as well as isolators from different vendors. "It didn't make sense to me. At the time [COVID], there were big challenges in resources – including people, materials and equipment. I wanted to standardize how we ran the facility," explains Winfield.

"Big challenges" is perhaps an understatement. During the pandemic, companies with large manufacturing contracts, such as for vaccines, were buying out equipment, consumables, and PPE. Many companies didn't yet know what their manufacturing processes were going to be so they hedged their bets. "Folks were buying every single-use mixer they could," says Winfield. "At one point, there was an 80-week lead time on single-use bags. After COVID, many items were thrown away because they were never used at the commercial scale. Regulators are now starting to look at manufacturing processes and controls – and encouraging companies to be more careful. For example, if you throw away 25 percent of your product because the containers aren't sealed, it's not okay to do that anymore because you are taking away consumables that could be used by someone with a more controlled process."

Winfield made calls to vendors he had worked with previously. After a bidding process, a deal for filling machines and isolators was agreed with groninger and SKAN. Although they are separate companies, only one contract was involved because the companies already have their own corporate partnership in place.

"SKAN has always focused on high quality containment," says SKAN's Marc Suter. "Back in the 1990s, the industry started thinking about separating the filling process and began looking into isolator technology. We were one of the first companies to build a commercial-scale filling isolator in Switzerland and with the industry heading this way it made sense to partner with a filling company. groninger is an expert with filling lines, and we had a similar mindset and similar leadership in technology, so we joined forces. Our goals are the same: to make reliable lines that are easy to handle for manufacturing products. Today, we collaborate together on a lot of different products."

The companies have their own separate products and different areas they want to grow in, but where there is crossover there is close collaboration. Nevertheless, they did need to reassure Civica that two

TOP TIPS FOR PROCURING EQUIPMENT

- Always prioritize quality and performance.
- Be as cost effective as possible. This is not just about the cost of the initial investment; also pay attention to the cost to operate and overall cost of ownership.
- Consider vendors with experience in similar projects; for example, a company that has worked within the government system and US funding will be well placed to do similar work.
- Understand what timezones your vendor works in and what geographies technical experts are based in for services.
- Consider a brand that is already trusted in the industry; it can give you immediate confidence in your equipment.
- Check the supplier can meet your timelines and schedules.

companies were definitely better than one. Clifton says, "We've been working together for years. Many of the long-standing staff at each company are very familiar with one another. In many cases, machines are designed together. However, some people do have questions about how the process works when you have two separate companies involved. They may feel they have to send two purchase orders and worry about things falling through the cracks. But we trust one another enough that one company takes the lead and responsibility for the project. A turnkey approach makes it easier for the client."

Talking about the Civica project, Suter adds: "Civica were using a greenfield site and it was a very ambitious project. As a vendor you see a lot of ambitious plans – and you also see plans that fail. How Civica approached everything was amazing. Today, the facilities are up and running. Everything has been installed and qualified."

One of the main reasons that Civica went with well-known companies for process equipment was simple: you know what you're getting from an established name in the market because the reputation is clear. "Quality and reliability should always be at the forefront. Cheap capital ultimately leads to bad operations," says Winfield. "Saving a few million dollars on a piece of capital or other shortcut will cost you a lot more in unreliability and rejected batches in the future. Buying good quality, reliable equipment sets you up for a culture of success. Bad equipment also affects people – who wants to fail? I wouldn't hang around for long at a company that was taking shortcuts."

Shortcuts aren't just investing in the wrong equipment; many companies try to cut costs by getting more life out of older technologies as opposed to investing in something new. Networks that don't invest run the risk of requiring complete remediation in the future. "Unreliable lines also mean that you're asking staff to do more with less," adds Winfield. "This type of cultural pounding can really demoralize people over time."

groninger outer bag removal

Docking a beta canister on an MTI (material transfer isolator) from SKAN

The technology itself is only one aspect of selecting the right suppliers. You also need to consider how the vendor will support you. Suter says, "There are many vendors out there that will all give you a great sales pitch, but it comes down to trust. Before committing, you need to ask yourself: do I trust this partner? Can this partner get me to the finish line on time? The finish line is not equipment delivery; it is when everything is qualified and approved by the authorities."

"Find a vendor that will help you install and qualify the equipment – and do whatever else it takes to make the project a reality and maintain the equipment for its life cycle," adds Clifton. "Don't forget to check out your vendor's service capabilities because this is something companies often don't look at until they actually need it."

As well as technological support with servicing, Winfield explains that time zones are a common issue. "There are a lot of amazing technology companies in Europe, but when you're stateside you

really need stateside support," he says. "If I have something that is broken, I need your help today. I can't wait 8 or 12 hours until the clock resets on the other side. Everything in this project from installation, to start up, commissioning, and so on was based out of the US."

Also think about the capabilities when times are rough. The COVID-19 pandemic has taught us all about preparing for unexpected disasters. "Since this project took place during the pandemic, travel was curtailed for many," said Suter. "Usually, vendors offer facility tours or site tours of other facilities using their technology, but we had to use virtual reality for mock ups and tailoring the line. This worked really well, but you have to have those capabilities in place and be adept at using them to make the project a success."

Civica has installed two full filling lines, which include four SKAN isolators (three as part of the filling lines and one as a material transfer isolator). One line is an integra line for bulk vials, and includes a tunnel and capper. The second line is for nested RTU containers. Both are flexible enough to cope with frequent format and product changes, and they have been designed to minimize product loss.

"Because the isolators are all manufactured by SKAN, it allows me to reduce my overall spare parts in the facility because there's a common spare part to each one," says Winfield. "I also have increased operational savings when it comes to HEPA certifications because I don't have to call in different vendors. This is also useful for audits and inspections. The true cost of ownership goes far beyond what the capital is."

Disrupting the status quo

From the very start of Civica, Clifton says that the company was unique. "A lot of people in the industry were watching them closely. They had smart people on the business side and significant industry leadership with a ton of manufacturing experience. However, I think there was some doubt early on about whether they could pull it off and if there would be any compromises on quality. But they've done it – their products are excellent and their facility is one of the best facilities I've visited. They use high-end solutions and they understand what they are doing. They are top tier in the industry."

"Somebody needs to tackle drug shortages, but it may not be profitable to do so for some companies. The result is that patients have to go without their medicines."

"It's hard not to feel personally moved when you hear the story behind Civica," adds Suter. "Somebody needs to tackle drug shortages, but it may not be profitable to do so for some companies. The result is that patients have to go without their medicines. I'm proud to be part of a project that is trying to change that. Civica isn't just focused on generic drugs; they also have plans to make affordable insulin. I really wasn't aware of how difficult the situation is in the US compared with Europe, where I am based. If you follow through and stand with something, you can make changes in this industry. It's been amazing to watch what Civica has done."

In some ways, it can be said that Civica found success in its insulin plans before supplying a single vial. In 2022, Civica announced plans for an insulin with a price of $30 for a 10 mL vial, and $55 for a five pack of 3 mL – which includes the cost of distribution and pharmacy dispensing. In 2023, the price of insulin in the US was capped at $35 per month for Medicare Part D, as part of the US government's Inflation Reduction Act. Insulin makers Eli Lilly, Sanofi and Novo Nordisk have all since reduced their insulin prices. Although the Inflation Reduction Act will have played a large part in the companies' decisions, it's also likely that they were feeling pressure from Civica's 2022 announcement.

Winfield says, "Civica put out a public statement in 2022 and we meant it. I'm not an industry expert on pricing or pharmacies, but ultimately other countries are able to provide affordable insulin to their citizens so there's no reason why this can't be done in the US too. We're not naive to think that we will take over and supply the world with insulin from our Petersburg site, but we hope we can prove to the industry that it is possible to sell insulin for less – and that others will follow."

Companies need to make money and they should be allowed to do so. Not everyone can be a nonprofit (although more nonprofits may certainly benefit the world), but the industry must find a way to address drug shortages while balancing profits. Perhaps this means rethinking certain aspects of business. Civica will continue to pursue its unique model, but companies like groninger and SKAN also have a role to play.

Clifton says, "It's our job to make the most efficient machines possible that can deliver the best products while helping to lower manufacturing costs. That has all come through innovation, which we don't want stifled by companies becoming unprofitable. I don't think the problem lies with the companies, but more with certain healthcare systems. Change is possible though – as Civica has shown."

Reference

1. *C Dredgs, S Scholtes, "Vaccinating Health Care Supply Chains Against Market Failure: The Case of Civica Rx," NEJM Catalyst, 4 (2023). DOI: 10.1056/CAT.23.0167*


"We need to start thinking across the lines to improve delivery and outcomes for the patient."

Preservation and Perseverance

Sitting Down With… Stella Vnook, CEO, Likarda

How did your journey to CEO begin?
I completed my pharmacy school residency/internship in oncology and quickly became aware of the lack of treatment options when assessing patients' brutal regimens. As a healthcare provider, whether you prescribe the medicine or the regimen, you're working with what you have. I chose to follow the path of drug discovery and commercialization by joining the pharmaceutical industry because I wanted to expand the portfolio of available drugs. It was a very long road to learning everything from drug development and healthcare education, to marketing, strategy, and managing markets, but every step got me closer to having enough ammunition and knowledge to be a good CEO.

What makes Likarda unique?
To optimize CAR-T, you can spend five to 10 years perfecting a specific construct to assure maximum efficacy or increasing the number of cells to reach the target tumor. Every time you change a construct, however, you have to go back to in vitro research, which costs a lot of time and money. Likarda's technology can safely encapsulate the cell therapy without the need to increase the number of cells. In fact, the technology can decrease the number of cells because we're delivering the treatment directly to the targeted organ.

What do advanced therapy stakeholders need to do to maintain momentum in the field?
We have focussed so much on creating new treatments that sometimes we don't think enough about the role drug delivery plays in the evolution of healthcare. We can improve therapeutics and cell therapies so much more if we can figure out how to deliver the therapy directly to a tumor. We need to start thinking across the lines to improve delivery and outcomes for the patient.

We also need common goals and purposes towards improving patients' lives. Some products have marginal benefits for the patient, but the cost is driven up to suit the system, which is not in the best interest of health economics. I would rather see efforts focussed on untapped innovation and truly meeting unmet needs.

You grew up in Belarus. How did your early experiences contribute to your career?
In a communist country, your choices are dictated by the government and by policy. I think this made me very driven when I arrived in the US and it helped me to be structured. Following the Chernobyl nuclear disaster, I activated my survival mode and found the resilience required. I understood the benefits of passion in doing what you love, as well as enabling people to do their best.

When I left Belarus, my mother and I were refugees. It was a traumatic experience and I haven't looked back. Until there's some type of political change, I can't imagine returning. Maybe decades will pass before people understand the freedom of following their dreams.

How might your mother feel about having such an impressive legacy and her daughter being the architect of that legacy?
My mother is my hero. She gave up everything – including family and friends – to take me across many countries, with no money, to reach the US. When Chernobyl occurred, people weren't allowed to know about or diagnose conditions that could transpire from the explosion. My health started to deteriorate and people said, quietly, that unless I received treatment, I probably wouldn't have a very long lifespan. My mother sold what she could so she could get us out. She worked many odd jobs through near starvation and poverty until she earned her dietary degree again here in the US. It was just the two of us, so now I feel pressure to not let people down – especially those who have given up so much.

The journey must have taken you to many places. Do you have a favorite?
I have a deep love for Italy. When we left Belarus, we weren't allowed to take much. It was one suitcase per person and very little money. We went through Poland and Austria into Italy, where we ran out of money. I was barely a teenager, and my mom was very young. She was trying to be brave but I was very angry because I didn't understand why I had to leave everything and everybody. I hadn't eaten for a few days, but some local women came and gestured to us to come and eat. They gave us pasta and helped with a place to sleep. I'll never forget that meal and how they embraced us. It was so heartwarming coming from a harsh "survival mode" environment. That type of kindness to a stranger really shaped my life and my journey.

What do you hope to achieve in the future?
I want to ensure that the technology that Likarda has developed is in good hands with a logistics partner and a cryopreservation partner, and that a manufacturing partner will embed it into what they do. The goal is to take it to where it could be available to every therapeutic that could potentially benefit. I'm going to stay focussed on the goal. Ultimately, at Likarda and beyond, I want to leave the same legacy my family has left for my children – it has to be inspiring and empowering, because when a person is powerless, their bar is low. I hope to live and lead with passion and purpose. I aim to demonstrate that as long as you believe in yourself, you can champion that change. If people are inspired, and they understand how to channel that passion into the purpose, we can enable new generations of leaders to continue to advance healthcare.

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