PHARMACEUTICAL COMMERCE
BUSINESS STRATEGIES FOR BIO/PHARMA SUCCESS
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# Pharma & Big Data:
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## LETTERS



## FEATURES





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# The reality of the situation

You have probably heard the term "the digitalization of healthcare" more times than you can count in the last few years, particularly amid the Covid-19 pandemic and the rise of things like telemedicine, virtual doctor's appointments and sales-rep visits, mobile clinical trials and the many more "AI-powered" settings for enhancing—or indeed changing—the clinical-to-commercial journeys for brands and the treatment paths for patients.

But, as the backdrop of Covid has illustrated, the digital dynamic is a very real thing today in pharma and the life sciences. Not necessarily in "end-product" or "proven-strategy" kinds of discussions, but, at the very least, in the transformation that's occurring due to big data, advanced analytics, etc., in providing tangible, or value-add benefits from drug discovery to lifecycle management. If you're not quite sold on this momentum translating to true inroads, look no further than *Pharmaceutical Commerce*'s coverage this month on a wide range of topics springing from, or in many cases, maturing out of the digitization umbrella. You may change your mind.

Among the themes we explore are new opportunities around real-world evidence/real-world data and predictive modeling; cybersecurity and data protection (is pharma resilient enough?); the need to boost capabilities in omnichannel marketing to the next level via analytics; synergizing data-critical functions such as medical information and pharmacovigilance; and the technical but, nevertheless, important advancements of AI-driven support automation and computerized system validation.

Yes, these various digital terrains in pharma remain much to digest—and deciphering the clutter and noise, and fancy new AI, from the strategies and applications that can really move the needle for patients, physicians, drugmakers, suppliers and everyone else with a stake in the game is still not easy. Issues around health data privacy and ownership could continue to complicate things as well. But as our cover story (page 28) outlines, the evidence is in: there are several ways that today's ability to interpret and act on data-driven insights can help drug manufacturers on practical levels and in their dealings with patients, payers, regulators and others. "Stakeholders no longer need to just model various scenarios hypothetically—they can actually track the product's clinical results in real-world use and conduct studies to validate more widespread clinical outcomes over time," says one expert.

Real answers for a new reality. Thanks for reading.

**Mike Hennessy Sr.**
Chairman and Founder
of MJH Life Sciences™



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# AI's predictive power on the big stage

For all the good things AI, machine learning and big data have brought to the world of pharmaceuticals and the practice of healthcare, there is still a strong sentiment out there that these tools have yet to put a dent into the high rates of clinical trial failures. Documented for years, various numbers and odds have been cited. One that's circulated of late finds that about 89% of novel drugs successfully advanced from mice-based studies fail in human clinical trials, with roughly half of the failures due to unanticipated human toxicity.

Analogies attempting to put these numbers into perspective spring up on occasion, also in the context of the high cost of drug development, but this one I heard from Isaac Bentwich, a genomicist and serial AI life sciences entrepreneur, in a recent conversation kind of resonated: "Imagine a world where you're in real estate, and you go to the best architect in the land, the best engineer in the land, and say, 'I want you to build me a skyscraper,' and they say, 'we are the best in our trade, here's what we're going to do: we will help you build 10 skyscrapers, we will guarantee that nine will crumble, we don't know which nine, and we will up the rent of the remaining building."

Isaac Bentwich

Bentwich, today founder and CEO of Quris Technologies, an AI platform company and the third tech outfit that he's launched, acknowledges the dramatic effect, but says the mechanics are spot-on in describing the current state of drug development. Of course, risks and the unknown factor will likely always be inherent in clinical trials—failure is never too far away, with so many variables at play. But Bentwich and others believe the still-low drug success rates are due largely to the lack of efficient ways to predict which candidates will be effective in the human body. Not all biological data is created equal, the belief is, and according to some, current AI-pharma players still rely on traditional data points—limiting their value to the same endpoint.

That's where this whole notion of next-gen AI enters the picture, in Quris's case with what the company calls Bio-AI, a clinical prediction AI approach combining powerful deep learning with high throughput biological validation. Quris, focusing initially on rare genetic diseases that cannot be modeled in animals, works closely with "clinical trials on a chip", a technology that emerged in academic, NIH and some industry research circles in recent years. Also called "patients on a chip," it's the idea of using miniature bioengineered models of human tissues and organ systems, along with nanosensors, to evaluate newly discovered drugs. Quris is developing a self-training AI platform to work with these devices, with the mission of better predicting clinical safety and efficacy for new drug candidates.

"So not in a sense that it replaces clinical trials but before going to clinical trials, you're actually testing the drugs on a bunch of genomic variables," Bentwich told me. "Take 1,000 drugs that have shown to be toxic, and take a thousand drugs that have shown to be safe and run each one of them on this platform of patient on a chip with the genomic variability. And then train the AI using this data. When you then run an unknown drug on the system, you ask the AI is this more like the safe ones that I've shown you or the unsafe ones?"

In securing access to thousands of patient genomes, Quris has an exclusive collaboration with The New York Stem Cell Foundation. The company is also working under the scientific guidance of industry visionaries such as Nobel Laureate Aaron Ciechanover and Robert S. Langer, co-founder of Moderna. Headquartered in Tel-Aviv and in the process of opening a lab in South Boston, Quris is preparing to launch a Phase I clinical trial next year for a drug using its Bio-AI prediction platform. The drug is for Fragile X syndrome, a trigger of autism and cognitive disability, and a notoriously difficult condition to treat.

"It's not just sufficient to have powerful algorithms," says Bentwich. "AI must have predictive value to be effective."

Look for more of my conversation with Bentwich in a Q&A on our website in the coming weeks. Clinical trials on a chip is one example of emerging innovations in AI-powered data intelligence in pharma, some of which we explore in the pages ahead. I remain impressed with efforts around natural language processing (NLP) to help characterize hard-to-diagnose diseases and improve screening rates for clinical trials.

And, as Ariel Katz, CEO of H1, notes in our "Last Word" column (page 47), where he outlines the key trends that will shape medtech in 2022, wearables helped with the Covid-driven shift from in-person to in-home or remote clinical trial reporting, and enabled extended tracking of patients to generate more meaningful and longer-term data. Chances are good many of these new AI approaches will have equal staying power.

-*Michael Christel* *mchristel@mjhlifesciences.com*


# Celebrating a Decade of Empowering Healthcare Success

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## LinkedRx

LinkedRx is the single source for Hand Carry and Direct to Practitioner program execution, reporting, and insight.

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# FDA highlights value and challenges of advanced drug manufacturing

## The goal of agency initiatives is to mitigate quality-related shortages and recalls

In its ongoing campaign to encourage industry adoption of more efficient and reliable pharmaceutical production methods, FDA is expanding its Emerging Technology Program (ETP) and providing more support for new drug applications (NDAs) that present advanced manufacturing technologies. While these initiatives aim to limit drug shortages and recalls due to quality issues, the program also seeks to expand investment in domestic production to enhance access to critical therapies and vaccines for US patients. At the same time, the agency is collaborating with foreign regulatory authorities to support international harmonization of policies designed to advance continuous manufacturing operations for drugs and biotech therapies around the world.

The challenges of meeting urgent demands for more new medical products during the Covid-19 pandemic has highlighted the value of more responsive and flexible manufacturing methods, including manufacturing systems that can be scaled up more quickly and automated processes that can reduce in-plant staff and permit more flexible employee work schedules. These developments have increased collaboration and partnering among biopharma manufacturers over the past 18 months, as competitors have worked together to accelerate access to needed therapies by providing fill/finish services for vaccine makers and active pharmaceutical ingredients (APIs) for finished dosage forms, among other joint efforts.

These issues and opportunities were discussed at an FDA conference on innovations in pharmaceutical manufacturing, as outlined in a report on the topic issued in March 2021 by the National Academy of Sciences, Engineering and Medicine (NASEM). This review, requested by the Center for Drug Evaluation and Research (CDER) on the importance and challenges for advancing innovative biopharmaceutical manufacturing, aims to reduce quality-related shortages that can limit patient access to safe and effective drugs.

CDER support for these initiatives appears to be having an impact, according to in a recent commentary by Center leaders. CDER director Patrizia Cavazzoni and Michael Kopcha, director of CDER's Office of Pharmaceutical Quality (OPQ), note that the agency approved its first application for a drug produced by continuous manufacturing in 2015 and 10 more applications since then that utilize advanced approaches for finished dosage forms, APIs and biological molecules. Now, more than 100 proposals using a range of innovative technologies have been presented to FDA officials, necessitating an expansion in the ETP program to handle the workload.

### International impacts

FDA's advanced manufacturing initiative also aims to enhance the competitiveness of US firms in the global pharmaceutical market by lowering costs and promoting quality. Acting commissioner Janet Woodcock cites visible success in this area in a tweet posted Oct. 15, reporting that more than 80% of the drugs made using advanced manufacturing technologies are produced domestically. An FDA Center for Advancement of Manufacturing Pharmaceuticals and Biopharmaceuticals established in June is coordinating analysis related to such topics as end-to-end continuous manufacturing, portable and modular distributed manufacturing platforms, and use of artificial intelligence or advanced modeling approaches in manufacturing.

At the same time, FDA is promoting international coordination and agreement on the scientific issues and regulatory policies related to implementing and overseeing modern drug manufacturing, as seen in the new draft Q13 guidance developed through the International Council for Harmonization (ICH). FDA recently posted the initial version of this standard for Continuous Manufacturing of Drug Substances and Drug Products, which applies to new drugs, generics, therapeutic proteins and biosimilars. Manufacturers and other stakeholders should submit comments on the draft document to FDA by Dec. 13.

This ICH initiative aims to advance biopharmaceutical quality through operations that reduce manual handling that leads to human error, utilize online monitoring and controls, increase manufacturing speed and efficiency and install smaller equipment that cuts costs. Ultimately advanced manufacturing systems would enable firms to respond "more nimbly" in the event of drug shortages and to adopt tailored systems that fit the needs of precision medicine.

*— Jill Wechsler, Washington Correspondent for MJH Life Sciences' pharma sciences brands*

## Will pharma accept the Democrats' drug pricing deal?

### Complexities remain in Congressional reform efforts

After extensive debate and discussion, Democratic leaders in the House and Senate appear to have reached a compromise on a relatively modest plan for controlling prices on certain prescription drugs. The latest deal (as of early November) greatly reduces the scope of medicines that would be subject to price negotiations, penalizes firms that raise prices faster than inflation and sets a $2,000 annual cap on out-of-pocket (OOP) drug costs for Medicare beneficiaries. An added sweetener is a $35 OOP monthly maximum on patient outlays for insulin, a high-profile consumer issue.

The modified plan addresses several main concerns raised by moderate Democrats in the House and Senate. Rep. Scott Peters (D-CA) has been most visible in proposing to limit Medicare price negotiations to older medicines with expired patents to protect investment in small biotech firms. Peters has been receptive to caps on price increases and on patient OOP costs, but also opposed to taxing firms that don't negotiate.

The latest compromise authorizes negotiations for only 20 drugs per year and only for older products that have exceeded exclusivity periods, with exceptions for small biotech companies. The package won support from Sen. Kyrsten Sinema (D-AZ), who has been a main opponent of drug price negotiations, but broader Senate backing remained unclear as of this writing.

However, industry continues to object to any Medicare price negotiations. Such action gives the government the power to dictate drug value and threatens the development of new treatments, according to the Pharmaceutical Research and Manufacturers of America. The Biotechnology Innovation Organization (BIO) urged its members to carefully assess the latest pricing plan, reminding them that any kind of price controls threatens continued funding for biopharma R&D. Generic drugmakers also object to language that sets inflation-based rebates on generics and biosimilars.

The ongoing debate highlights the complexities of drug pricing. Going forward, policymakers still will consider additional options for reallocating rebates, reducing patient costs, skewing payments to high-value therapies, boosting contributions from the wealthy and subsidizing those who can't afford needed medicines.

*— Jill Wechsler, Washington Correspondent for MJH Life Sciences' pharma sciences brands*



## Survey: The pace of DSCSA readiness preparations has slowed

### Pharmacies are still lagging in meeting looming compliance requirements

The sixth annual Serialization Readiness Survey, a poll of manufacturers and distributors, is out from the Healthcare Distribution Alliance (HDA). As in past years, the main worries are how broadly manufacturers are readying their IT systems to convey required transaction data to their trading partners—specifically, wholesaler-distributors. The worries of early years, in getting readable barcodes on individual packages, have faded but not disappeared. All this is happening in the context (in the US) of the Drug Supply Chain Security Act (DSCSA), which has a November 2023 deadline for a fully interoperable, electronic data-sharing system that can track individual pharmaceutical packages from point of origin to point of dispensing.

Key findings of the 2021 survey include:

- Forty percent of manufacturers are currently sending or plan to send, by the end of 2021, at least some serialized data to their wholesale distributor customers upon shipment. Forty-three percent plan to do so by November 2023. Another 16% are still unsure of when they plan to exchange data with wholesale distributors via EPCIS for all products.
- Over the past three years, the number of manufacturers planning to send serialized data with 100% of product has fallen to 12% from 35% (2020) and 21% (2019). Most manufacturers, 65%, anticipate sending 100% of data with shipped product by 2023, when it is legally required.
- While 45% are currently "aggregating"—the process of compiling all the individual package serial data when shipping in a case or pallets—nearly 40% will do so by 2023.
- Distributors are still preparing for data exchange. Only 60% of distributors can accept serialized data today. However, approximately 48% are receiving serialized data for between 1% and 5% of transactions.

*— Nicholas Basta*

# Decentralized trials specialist Science 37 debuts on Nasdaq

Completes merger with blank-check company LifeSci Acquisition II Corp.

Science 37, Inc., a clinical research company that specializes in decentralized clinical trials, has officially completed its previously announced merger with LifeSci Acquisition II Corp., a blank-check firm aimed toward the biopharma, medical technology, digital health and healthcare services sectors. Shares of common stock of the combined company, which will go by Science 37 Holdings, Inc., started trading on the Nasdaq Oct. 8 under the new ticker symbol "SNCE."

Via the special purpose acquisition company (SPAC) deal, Science 37 received about $235 million total cash, less fees and expenses. With the proceeds, Science 37 plans to fund its decentralized trial technology platform, while also pursuing other relevant endeavors. David Coman, Science 37 CEO, who will continued to lead the combined company, says that the firm's operating system has demonstrated the ability to speed up patient enrollment, provide higher patient retention, reduce patient burden and include participation from underserved patient populations. Since its founding, Science 37 has conducted more than 95 decentralized clinical trials and engaged more than 366,000 patients.

## Cardinal Health, FDA team up on RWE study

Pharma distribution giant Cardinal Health has been awarded a $750,000 contract by FDA to implement an 18-month real-world evidence (RWE) study as part of the agency's efforts to advance the applicability of RWE in regulatory decision-making.

The funding will go toward the project "Assessment of a Novel Methodology for Endpoints Assessing Response to Lymphoma Treatment in Real-World Studies," and will assess the precision of real-world data (RWD) for lymphoma tumor response, compared to blinded independent central review, which is considered to be the "gold standard" in randomized controlled trials. Cardinal Health will work alongside the FDA Oncology Center of Excellence to evaluate tumor response in the clinical care setting.

## Pact centers on AI-powered KOL mapping

Ashfield Engage, part of UDG Healthcare, has struck a partnership with PeakData, a technology company that provides AI-fueled scientific landscape analysis. The alliance will focus on enhancing key opinion leader (KOL) mapping and profiling capabilities for medical science liaison (MSL) tools within the medical affairs specialist area of Ashfield Engage. Users would get real-time updates on KOL activity to potentially help strategic decision-making and program design as well as inform and improve customer engagement. PeakData uses AI to draw upon a combination of local web data sources with existing data sources to deepen insights on KOLs.

## ten23 health acquires swissfillon

Pharmaceutical sterile filling company swissfillon is joining ten23 health, a Swiss contract development and manufacturing organization (CDMO) that launched in September.

The acquisition of swissfillon—an FDA- and Swissmedic-approved CDMO, also based in Switzerland—is expected to deliver an offering for sterile drug product development and manufacturing of biologics, molecules and dosage forms, the parties note. Prof. Dr. Hanns-Christian Mahler will serve as CEO of the combined entities under the ten23 health umbrella. Daniel Kehl, founder of swissfillon, will remain with the business, helping to run ten23's new infrastructure engineering projects that occur under the partnership.

## Catalent invests big in gene therapy campus

In order to meet customer demand, Catalent, a contract development and manufacturing organization (CDMO), is taking on a $230 million expansion project to add three additional commercial-scale viral vector manufacturing suites, and support facilities and services at its gene therapy campus in Harmans, MD. This latest investment also includes expanding the site's storage capabilities for just-in-time inventory space, ultra-low temperature freezers and its water-for-injection infrastructure.

## Schreiner an 'Alliance to Zero' founding member

Schreiner MediPharm, a German specialty label developer, is one of eight founding members of the Alliance to Zero, a non-profit partnership whose goal is to improve sustainability throughout the pharma supply chain. Other members include Dätwyler, Harro Höfliger, HealthBeacon, Körber Pharma, SCHOTT, Sharp and Ypsomed, a lineup of component suppliers, machine manufacturers and assembly/manufacturing service providers, manufacturers of primary and secondary packaging, and companies responsible for final product assembly or handling products that have been returned after use.

# The NRx Crisis

## Converging factors are reducing patient access to drug samples

**By Hal Walsh, SymmetryRx**

According to IQVIA, new prescriptions for chronic therapies are at 80% of the pre-Covid-19 normal, only recovering with the start of 2021. We are further informed by these statistics:

- IQVIA: About10% of visits to prescribers are telehealth visits.
- SymmetryRx: 39% of telemedicine patients are appropriate to receive drug samples.
- Accenture: Pharma reps experienced a low of 35% in-person prescriber meetings during the pandemic.
- Datamonitor: 30% of patients only fill their prescription when they get a drug sample, regardless of age, income or gender.

### New sampling channels are needed

Drug sampling is a key driver for familiarizing prescribers with drugs, beginning patient trials and starting patient adherence. Pharma manufacturers have traditionally relied on field representatives to drive the distribution of drug samples to prescribers. Though this tactic is very successful, there has been a shift in prescriber willingness or ability to meet with field reps.

### Untangling the problem

Pharma reps provide critical services to prescribers, including:

- Education on proper use, risks and benefits of medications.
- Access to patient resources (educational & financial support).
- In-practice drug samples.

The pharma rep's efforts, supported by brand marketing programs, create demand and interest in the brand. Prescribers who use drug samples in their practice of medicine rely on a consistent supply of samples to serve their patients.

The problem is one of reach. Decreased prescriber reach by pharma reps and the increase in patient telemedicine encounters are reducing patient access to drug samples.

### Maximizing prescriber and patient reach

SymmetryRx's prescriber self-serve SampleCenter™ eSample® request platform allows Prescription Drug Marketing Act (PDMA)-compliant sample requests for ship-to-practice and direct-ship-to-patient from the manufacturer's authorized distributor of record (ADR). This 4th-generation SampleCenter™ offers critical features that enable health systems and large group practices to easily manage sample requests in a PDMA-compliant, fully digital user experience that decreases practice inventory outages and inventory waste at the practice level.

Of high importance, SampleCenter™ enables telemedicine patient access to drug samples.

Additionally, practices that do not maintain sample inventory can serve all patients through the SampleCenter™ direct-ship-to-patient capability.

### Access, access, access

SampleCenter™ extends the reach of pharma reps by creating the opportunity to reach more prescribers to provide the complete service that those prescribers require. Our sample demand generation and patient medication adherence programs help our pharma clients increase new and total prescriptions and thus expand the success of medication treatment programs.

We integrate our service with your ADR for seamless execution in about eight weeks. All request allocations, processing and validation operate through data integrations with your ADR and allocation system.

SymmetryRx has operated drug sample request websites at brand and enterprise levels for 31 manufacturers while simultaneously giving prescribers sample access through SampleCenter™, the only true industry utility, one-stop sample request site designed for prescribers. The result for our clients is that we are consistently the second-largest source for drug sample requests after the pharma's direct or contracted sales force.

In an ever-changing healthcare landscape, pharma manufacturers need effective solutions to capture drug sample demand from prescribers in the way prescribers prefer. By partnering with SymmetryRx, manufacturers can dramatically extend their reach within health systems, large groups and directly to patients.

**ABOUT THE AUTHOR**

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*Brand Insights - Thought Leadership from Marketers, Paid Program*

# Preserving Drug Sampling Activity

## A dramatic shift to e-sampling has been the effective counter to the reduced number of sales rep visits to physicians' offices

NICHOLAS BASTA, EDITOR EMERITUS

Handing off drug samples to healthcare professionals (HCPs) is a time-honored, integral part of pharma sales and marketing; the industry commits the equivalent of tens of billions of dollars to it in the US and abroad. For sales reps specifically, it is often the best or easiest way to get into the HCP's office. Nor should it be ignored that since the late 1980s, the activity has been highly regulated by the Pharmaceutical Drug Marketing Act (PDMA), which created the need to document how samples are distributed.



Potentially, the sampling practice would have been upended by the Covid-19 pandemic (no, or drastically reduced, sales calls) except for two factors: FDA relaxed some PDMA requirements in early 2020; and the service providers to pharma for sample accountability—a relatively small number of specialized firms—had already developed digital tools to enable samples to be delivered at the HCP's request.

FDA's action came very early as the pandemic settled in—its June 2020 "temporary policy" (still in effect as of this writing) altered the request and signature requirements of PDMA (see https://bit.ly/3GfBFzt):

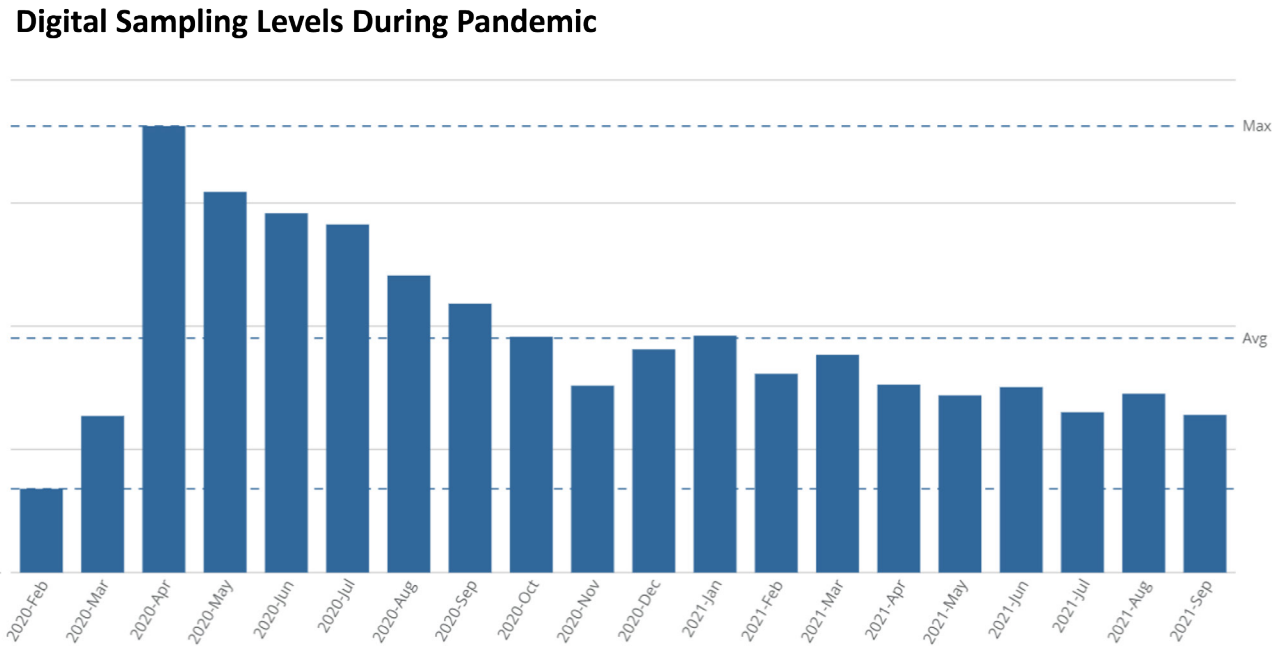
*FDA does not intend to take action against a manufacturer or authorized distributor of record that accepts alternate ways of verifying delivery and receipt of drug samples instead of obtaining the signature of the person acknowledging delivery…*

Additionally, the guidance explicitly allowed for samples to be delivered to the HCP's home, or an associated hospital or pharmacy (there was no specific guidance on this previously). Somewhat controversially, the guidance also allows for delivery to the home of a patient under that HCP's care.

The main rationale for these modifications was the dramatic drop-off in patient visits to doctors' offices, as well as the equally dramatic increase in telemedicine. Telemedicine went from 4% of patient-HCP interactions to 60% "practically overnight," says Maria Whitman, global head of pharma and biotech at ZS, a professional services firm. "The importance of sampling became evident," she says, "and patients and doctors were looking to manufacturers to find workarounds" to the lack of in-person sample delivery.

As a result, manufacturers dusted off, upgraded or swiftly adopted various e-sampling technologies that have existed in the industry for years, but were only slowly being adopted. "The e-sampling and direct-to-practitioner channels—which used to be an option—have now become table stakes for the vast majority of customers we support," declares Eric Johnson, chief commercial officer at J. Knipper. Data the company collected on its digital platforms show a more-than 2x increase in digital sampling channels starting in April 2020 (see chart on facing page). That gradually declined over the next seven months, as sales reps returned to the field, but still well above the March 2020 level.

"For years, I've been talking about the value of a multichannel approach to HCP interactions by pharma," adds Mark Jara, CEO of New Jersey-based RxS LLC. "When Covid-19 hit,




*A 20-month visualization of e-sampling activity in the pharma industry. The data was presented at the Sept. 29 virtual meeting of the Sharing Alliance, a volunteer group of industry sample management professionals.* Credit: J. Knipper



pharma sales organizations weren't prepared for the all-virtual environment, but our digital platforms were embraced quickly, and it has just increased exponentially since."

Industry data cited by Jara and others describe a situation where around 70% of sales rep-HCP interactions were face-to-face pre-Covid, which temporarily went to zero during the early lockdowns. It has since revived to around 30% of interactions—the rest being various virtual calls or digital communications.

E-sampling has taken on a variety of forms, tailored either to the desires of HCPs requesting the samples, and/or the compliance needs of the manufacturers, as well as their preferred marketing methods. A basic form allows a rep (or the CRM system the manufacturer is using) to send a sample-request form to the HCP; this can be accompanied by the digital signature of the HCP, along with required address and affiliation information. Another version allows the HCPs themselves to initiate the request.

J. Knipper announced an upgraded platform, SamplicitySA, in August. The platform enables direct-to-practitioner, direct-to-rep and direct-to-patient ordering; the direct-to-practitioner version is initiated by the HCPs themselves. The "SA" component of SamplicitySA is enhanced sample accountability through a user dashboard. Other components of the platform enable analytics and connection to Knipper's in-house sales support services (Knipper handles the software development of Samplicity, as well as order fulfillment and sales support).

At RxS, there are three interrelated systems: LinkedRx, for overall sample management, accountability and reporting; TeleTargetRx, for accompanying telemedicine/virtual sales situations; and SampleCentral, a portal for HCP-initiated sample requests. The latter emphasizes an "HCP-centric" approach, says Jara; some physicians (especially those in "no-see" offices that don't permit rep visits) prefer to manage their own sample activities. The system is device-independent, running on smartphones, tablets or desktops. "It's a customer-centric process, but the rep is still involved," he adds. "He or she can note what the doctor is ordering, and interrupt the process to, for example, arrange a conversation with that person." Overall, the platforms are intended to give reps a fuller "toolkit" for managing HCP interactions.

SymmetryRx provides a self-service (for physicians) sampling portal, complete with e-signature and e-request features, as well as an IT system, branded as RadiusXP, that can be used by pharma sales teams directly. Most recently, the company built out a direct-to-patient sampling platform.

Data-services firm IQVIA provides a suite of IT solutions for sample management, regulatory compliance and allocation optimization, and works with a strategic partner to provide field services (such as auditing inventories at field offices). These services can interact with the overall CRM solution of the firm, Orchestrated Customer Experience (OCE).

For their part, both RxS and J. Knipper are strategic partners of Veeva, the leading CRM platform for life sciences sales teams. RxS' Jara says his company's software can pull data out of, or provide input to, a Veeva implementation to meet pharma needs.

Other pharma CRM systems, such as StayInFront, Synergistix or Salesforce.com, have various levels of data-sharing or actual sample-accountability management; Synergistix, for example, offers in-house support for regulatory compliance and reporting.

## Post-Covid

ZS' Whitman says that the pharma industry struggled mightily to react to the pandemic shutdowns in switching to vendor solutions like those listed earlier, or to modify their own internal processes. "There were some bumps along the way, early on, with some physician practices complaining of a lack of sample availability, and some worried about an oversupply," she says. "It took some time to get the right business rules in place."

Based on surveys ZS has conducted, roughly two-thirds of primary care physicians, for example, complained of a lowered availability of samples; on the other hand, 73% of oncologists and rheumatologists said their needs were being met.

There could be a particular reason for samples' emphasis in oncology or related specialties, many of which involve injected biologics: a sample prefilled syringe gives the physician an opportunity to guide the patient on proper self-administration of the drug. "Avoiding a second visit to the doctor's office to get a demonstration is a value-add for both the patient and the physician," says Whitman. "Before Covid, and after, pharma marketers in these specialties are concerned with losing patients between diagnosis and treatment. Providing this service is in the mix of patient services and support."



**Avoiding a second visit to the doctor's office to get a demonstration is a value-add for the patient and physician**

Mention of biologics and oncology also highlights another aspect of sample accountability: ensuring that the right physician specialties are getting the appropriate samples. "Under PDMA, there are inclusions and exclusions for samples based on the medical specialty," notes RxS' Jara. Physician affiliations and practice areas need to be documented to justify distributing samples to that physician. According to Jara, this is another reason to consolidate sample administration on one platform across sales teams and across medical specialties—otherwise, the left hand might not know what the right hand is doing.

One aspect of sample distribution during the pandemic whose future is still undetermined is direct-to-patient servicing. "So far, we're seeing this as having limited appeal; industry is waiting for clearer direction from FDA," says Jara. There are numerous risks to the manufacturer in this practice, even with the intercession of the physician making the request.

Johnson of J. Knipper describes the elaborate process on the sample-fulfillment side for direct-to-patient. "First off, the physician's request for a sample is treated as protected health data, and from this point on, the data will be housed in a separate set of data tables with no visibility to our clients and a small subset of need-to-know people on the Knipper side." Then, the system checks to see if both the physician and the patient are in a state that allows direct to patient sampling. The order is then processed against standard compliance business rules as well as client business rules. Finally, if the order passes all of those business rules, it is transferred to the warehouse to be fulfilled.

Still, as Whitman points out, it is likely that telemedicine will remain a larger component of office practices after the pandemic and, if so, direct-to-patient sampling might be equally popular. ZS' physician-survey data show that providing samples is one of the highest-cited benefits the pharma industry can provide, second only to financial support for patients.

**ABOUT THE AUTHOR**

*Nicholas Basta is Editor and Founder, Pharmaceutical Commerce* PCm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# RWD Benefits at the Point of Care

## AI-driven strategies for pharma brand marketing teams to help bridge the gap from data intelligence to execution

**BY MIKE ROUSSELLE, OPTIMIZE RX**

The application of real-world data (RWD) for pharmaceutical brand marketing has evolved from the traditional model of executing on historical data, to AI-driven strategies that allow brand teams to execute on evidence-based insights, drawn from real-time data powered by predictive analytics at the point of care (POC). This gives pharma brand teams the ability to proactively alert physicians about relevant treatment information based on dynamic data about their patient's journey.



As the healthcare industry continues its accelerated adoption of digital solutions—largely spurred on by the Covid-19 pandemic—improving patient care and adherence through technology must start with optimized insights at the POC. Reimagining RWD from how it's been traditionally leveraged by pharma is key.

There is a vast opportunity for life sciences companies to use AI-driven solutions to bridge the gap from data to insights to execution, bringing everything together into one seamless closed loop. The application of predictive analytics, using machine learning (ML) methods applied to RWD, can speed time-to-therapy, and support positive outcomes by enabling life sciences to help healthcare professionals (HCPs) identify patients who may be qualified for specific therapies, by raising awareness of qualification parameters and patient access pathways, as well as identifying early indicators of non-adherence among patient populations in real-time. AI-driven solutions also enable personalized HCP engagement programs based on up-to-date demographics, disease and care milestones of their specific patient panels.

This kind of technology advances POC communication by layering AI-driven algorithms on top of real-world datasets to solve high-impact awareness, access and adherence challenges, optimizing the feedback loop between life sciences and providers, ultimately supporting better patient outcomes.

### Digitizing access is the gateway to adherence

The quest to solve drug awareness, access and adherence challenges is less elusive now than it's ever been, thanks to technology solutions at the POC. What's new is how the life sciences industry and its partners are applying new technologies to proactively support and engage patients.

AI-driven predictive analytics can help pharma brands execute more intelligent digital commercialization strategies to simplify therapy initiation by presenting HCPs with a fully electronic option for enrollment, benefits verification, prior authorization and patient support onboarding. This proactive approach enables pharma manufacturers to support patients by removing an obstacle in their journey and helping them get started and stay on their doctor's recommended course of therapy.


*Credit: everythingpossible/stock.adobe.com*

One example of how AI-driven RWD marketing tools at POC helps patients obtain the therapies they need is in the area of specialty medications. The increasing availability and use of these therapies has exposed unique barriers to prescription fulfillment processes and patient access. This has created complicated healthcare delivery workflows resulting in hurdles that impact a provider's ability to optimally prescribe, and for patients to receive specific therapy. A recent physician survey by the American Medical Association[1] found that prior authorization process delays have a significant effect on patient outcomes, with 90% of physicians surveyed reporting that the prior authorization process has somewhat or significant negative impact on clinical outcomes, and 30% reporting that this process has led to a serious adverse event for a patient in their care.

For conditions requiring specialty medication, AI-based solutions can reduce the provider burden and improve patient care. Predictive analytics simplifies the prescribing process and helps patients access the specialty drugs they need by addressing the enrollment and approval of specialty drugs at the earliest possible point in the prescribing process. It also ensures that key enrollment and affordability information is delivered based on the disease state or benefits profiles of each provider's patient population in real-time.

**The quest to solve drug awareness, access and adherence challenges is less elusive now than it's ever been**

## A novel approach to identify real-time needs

For years, the life sciences industry has been executing on RWD insights months after the data is collected—in a retrospective, rather than prospective needs-based manner. Typically, RWD and predictive analytics are leveraged by pharma marketers to predict the influence of a physician on prescribing trends and anticipate future prescribing needs and volumes based on past physician behavior. Using this model, life sciences sales and marketing teams determine which HCPs may need what information about their treatments.

Evidence-based physician engagement uses a novel approach to understanding which HCPs need specific information, because it doesn't rely on past physician behavior to predict future need. Instead, it uses patient characteristics

like disease progression and coverage information to identify current needs among the patient populations being seen by specific HCPs. This can be seamlessly integrated at POC, including in the electronic health record (EHR).

The benefits of real-time, evidence-based engagement include:

- Bridge the months-long time gap that manufacturers have historically faced between when data sets are created, and when they can execute on that data.
- Enable pharma brand teams to employ a predictive, proactive approach to execution on data insights at POC, bringing it all into a seamless closed loop.
- Facilitate contextual engagement between manufacturers and HCPs at the POC, which can speed patient access to therapy and support adherence that can have a positive impact on outcomes.

To truly capitalize on this untapped value in the short run, life sciences companies need to focus on identifying steel thread use-cases where value can be returned quickly on these projects. One real-world evidence (RWE) use case that is currently being utilized by a top pharma manufacturer is to provide visibility to doctors when Medicare patients' treatment plans are at risk of lapsing due to loss of coverage. An AI-driven RWD engine is helping the manufacturer determine when to notify physicians that patients in their panel may be eligible for financial assistance. This helps ensure that patients who qualify for therapy can continue to follow their physician's preferred treatment plan without interruption, due to new and unexpected out-of-pocket costs.

**The value-driving lever will move from message volume to message quality—measured by relevance and specificity**

## Leveraging AI-driven data throughout the patient care journey

The biggest untapped opportunity is the use of RWD and AI to reach providers and patients with more timely and relevant information at critical junctures throughout the patient care journey, at the POC and beyond. AI-directed, real-time HCP marketing raises awareness of treatment benefits to give patients a timely start on therapy while personalized digital patient support programs help patients stay on therapy. This allows the pharma industry to close the loop and interact with HCPs and patients by enabling care-focused engagement throughout the patient care journey. The ability to leverage actionable, AI-driven, integrated data at the POC and beyond is key to unlocking streamlined communication and processes around timely therapy initiation to reduce abandonment, and design personalized, successful adherence programs to help patients stay on their doctor-recommended course of therapy.

## The future of AI-driven solutions for life sciences

As the healthcare industry continues trending toward interoperability in the coming years, being able to message HCPs and patients at various points throughout the care journey will become the standard. HCPs and patients will be inundated with messaging, and the value-driving lever will move from message volume to message quality—measured by relevance and specificity. That's where RWD and AI will play a key role by enabling precision in messaging—identifying smaller subsets of HCPs and patients that are exactly the right audience for each brand, at exactly the right time for that message to affect behavior—supporting timely therapy initiation and driving adherence.

The real value brands can add to the patient's care journey lies not just in knowing which HCPs to message, but also *how* those HCPs operate in their EHRs and when those physicians are seeing patients who qualify for therapy. AI will be used to glean insights beyond those visible solely in claims data. It will be combined with additional sources of data to compensate for the gaps in conventional RWD—overcoming the time-lag in most RWD datasets, and identifying patients who are eligible for therapy changes even *before* they show that tendency in their claims history.

In short, AI-driven solutions will change the way we think about the entire value proposition of life sciences messaging.

**ABOUT THE AUTHOR**

*Mike Rousselle is Vice President of Data Product, OptimizeRx* PCm

**Reference**

1. https://www.ama-assn.org/system/files/2021-04/prior-authorization-survey.pdf

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# The Pulse of Digital in Pharma

## A conversation with Justin Hoss, technology leader for life sciences, KPMG

**MICHAEL CHRISTEL**

A born Detroiter, Justin Hoss grew up with two loves (besides his sports teams): cars and his family's mom-and-pop pharmacy in Hamtramck, MI, a little community surrounded by the Motor City. As the nature of most family-run businesses, starting at a young age, 10 or 11 as Hoss recalls, he began working at the pharmacy, offering "free elbow grease, as my mother used to call it." But perhaps something else more lasting began to materialize on those days after school and on weekends sweeping the floors and helping count pills as a pseudo pharmacy tech. "It wasn't just seeing my family filling scripts; I got used to understanding how things were ordered through distributors, all the software that was provided to local pharmacies, and then just built my passion for the life sciences and learning how drugs worked."

Though the seed was planted, years later, however, after graduating from the University of Michigan and starting his career in automotive consulting, Hoss faced an early crossroads moment: decide to stay on his current path or break into the healthcare space. He chose the latter; the rest is history. Today, Hoss is the national technology leader for life sciences at KPMG, where he is also a partner in its advisory practice with 25 years in business consulting and industry experience. "Healthcare doesn't just stop at the healthcare provider," says Hoss, who is based in North Chicago. "Healthcare is much more of a broader understanding of products and therapeutics in the pharmacy and how all that works within a value chain."

*Pharmaceutical Commerce* caught up with Hoss recently to discuss KPMG's 2021 Global Pharma CEO Outlook survey—and the new trends gleaned from C-suite perspectives on cybersecurity and digital transformation in particular.

**1. In your pharma CEO survey, results showed a 7% rise from last year and 9% from 2019 in CEOs reporting a "High" M&A appetite (see chart on page 22). What do you attribute for that increase, particularly amid the continued business impacts from the Covid-19 pandemic? Would you say the overall accelerated pace of digital adoption in pharma/healthcare is a major factor driving these decisions—whether confidence on the buyer side in their level of digital agility, or the growing appeal of digital health companies and medtech vendors as important acquisition targets?**

I do think companies are a lot more confident, especially in the life sciences space, making what I would call more disruptive acquisitions. Many of these companies are flush with cash, so they're always looking at what they can do from a portfolio standpoint, whether trimming portfolios or adding back. But what I think has changed recently, and you're spot on, is there's a lot more confidence in their digital agility, meaning being able to make quicker decisions using analytics on whether this is the right buy or not. It is more of a function of their business development groups really looking at the

market, understanding where trends are happening and being a little bit more confident in leading with their own data and going, "okay, we should make this acquisition because it's complementary to our portfolio or it adds additional digital services around it, brings us closer to the patient."

I think that trend was happening pre-Covid; it just kind of got a push during the pandemic. But another aspect during Covid and in this kind of transitional Covid period is us as consumers talking [more] about life sciences. When was the last time you sat down with a friend of yours pre-Covid and talked about vaccine development? Those kinds of conversations are happening now at the dinner table. So as the public started to become more aware of life sciences, I think life sciences firms gathered a lot more confidence beyond just saying, "hey, I think this is going to be a strategic acquisition, a strategic add to our portfolio around a product or service"—but being a little more agile in their thinking and not overthinking some of these things.

**2. Cybersecurity and data breaches have long been a concern of pharma companies, but with the emergence of digitalization and cloud computing, new concerns around cybercrime have been raised. In the KPMG survey, 57% of pharma CEOs claim their organizations are "well-prepared" for a future cyber attack, up 38% from last year; but, at the same time, only 11% say their companies are "very well-prepared," the same figure as last year but down from 29% in 2019 (see chart on page 23). What dynamics need to change for CEOs to start feeling better about their organization's ability to withstand and mitigate the effects of a cyber attack?**

I've been in technology for 25 years and I don't think you're ever at the pinnacle, the North Star of being super prepared for anything that might happen from a cyber standpoint; it's nice to be able to say that. I do think it's become much more of a board-level discussion point, where in the past 10 years with some of the major breaches, it's gotten a lot more visibility into not just the cyber aspect of it, but the trust aspect as well. If you think about healthcare and life sciences companies, we're trusting them with a big part of who we are—our health records, what therapeutics we take, the things that keep us well.

**When was the last time you sat down with a friend of yours pre-Covid and talked about vaccine development?**

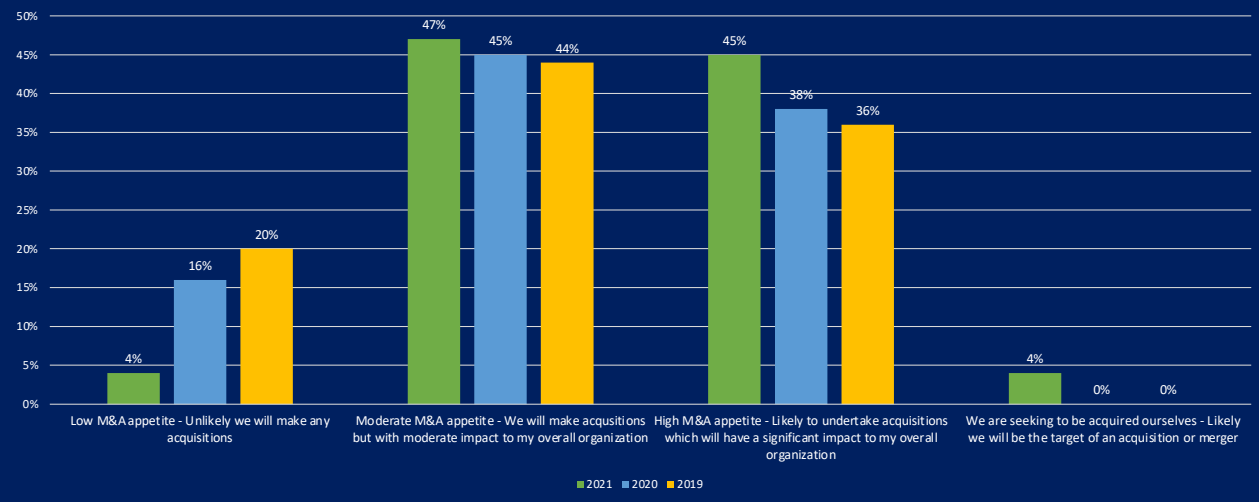


From a confidence factor, I do believe that [pharma leaders] think that they're well-prepared. But I also believe that there's a move toward, it's not just about cyber and the physical aspects of protecting your data and your IT systems, but there is also this overarching trust kind of view about, does a customer trust me, as a brand, to hold pretty sacred data about my health?

I think there's a lot of, are we not only doing the right things physically to protect ourselves from cyber attacks, but also are we doing the right things to instill that trust with the consumer and the patient? Those are some of the reasons why that confidence factor has increased, because it's not just about cyber, even though that's what we always talk about.

An increasing board-level responsibility in understanding the reasons why cyber is important has been a positive factor of that. It's not just a conversation that the technology groups are having with the CIO (chief information officer) and a couple key people on the

## Over the next 3 years, how would you describe your organization's M&A appetite?




Source: KPMG CEO Outlook (pharmaceutical/biotech executives only)



audit committee. It's much more of a broader board-level conversation. When more people understand why cyber's important and more people are actually in the box around helping with the trust and the cyber factor, that confidence factor goes up. I think there's a lot more positives just by the nature of how relevant and critical cyber has become as a board-level conversation; and not only at the board level, but the shareholders and stakeholders as well. It's part of this overall ESG (environmental, social and governance) theme that's going on.

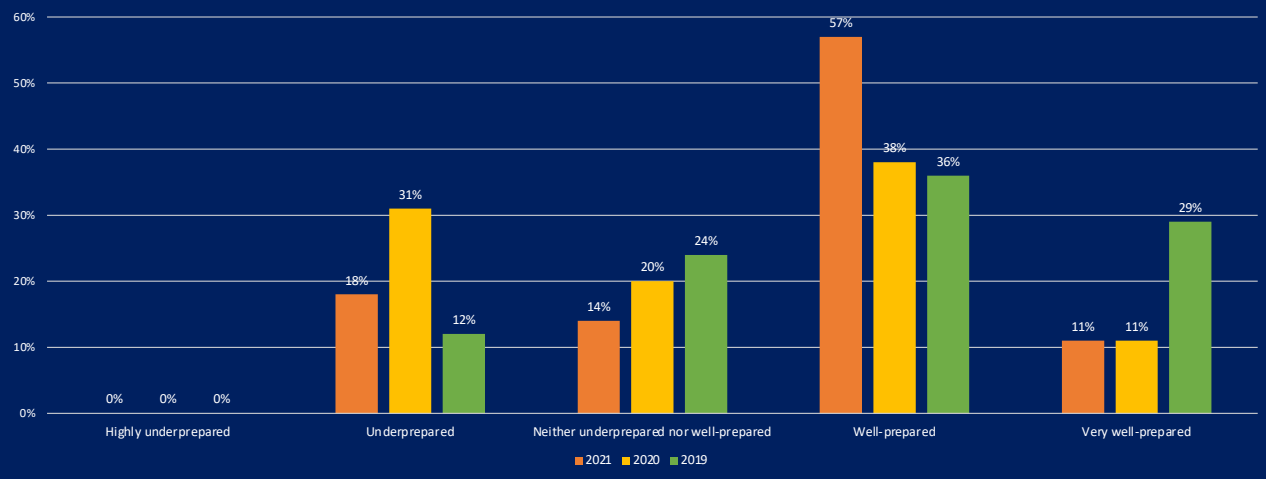
**3. There is the thought that the pandemic—and, for example, the rise in telehealth as the crisis took shape in 2020—created more of an opportunistic setting for cyber crimes. Do companies feel that there have been lessons that they've learned in data protection/compliance/governance while navigating Covid that will help them combat future cyber threats?**

There was a lot of need for speed. But when you also have speed, sometimes you don't have the security-by-design thought processes. But, honestly, if you start thinking about the pandemic specifically and people being opportunistic about telemedicine and teledocs and all that kind of stuff, there were some [threats] out there, but there wasn't a ton. It does show me that leading into the pandemic, there was a lot of preparedness before it hit. But healthcare companies are among the No. 1 targets right now; it used to be mostly financial data organizations.

**4. Your survey also touches on the concept of disruptive innovation, and, interestingly, weighs views of technology disruption as an opportunity or a threat, and if companies are being proactive disruptors themselves. Regarding the latter, 60% of CEOs, an increase of 18% from last year, agree that their organizations are actively disrupting the sector in which they operate, and only 8%, down from 20% in 2020, disagree. In that same vein, though a bit of a drop from last year but still significantly higher than in 2019, 59% of leaders agree that their companies see tech disruption as more an opportunity than a threat. What's your take on these two trends and the factors behind them?**

I think, finally, regardless of whether it's in the CIO function or beyond the C-suite, it's this commitment to embracing technology; they understand that technology is the strategy right now. If you're truly talking about types of innovative business models, and changing the way the life sciences and pharma company value chain looks, you have to be able to harness the power of technology, not just for cost-efficiency purposes or the back-office function.

## How well prepared is your organization for a future cyber attack?




Source: KPMG CEO Outlook (pharmaceutical/biotech executives only)



And so that means making these huge bets—not only with your own internal AI or data-analytics capabilities, and thinking about real-world evidence and doubling-down on that, but also thinking about innovative ecosystem partnerships where you can have these partnerships around technology that help you actually resolve a therapeutic state or some kind of challenge that you're having. Organizations don't mind that the technology discussion has left the CIO office and has become a value-chain conversation.

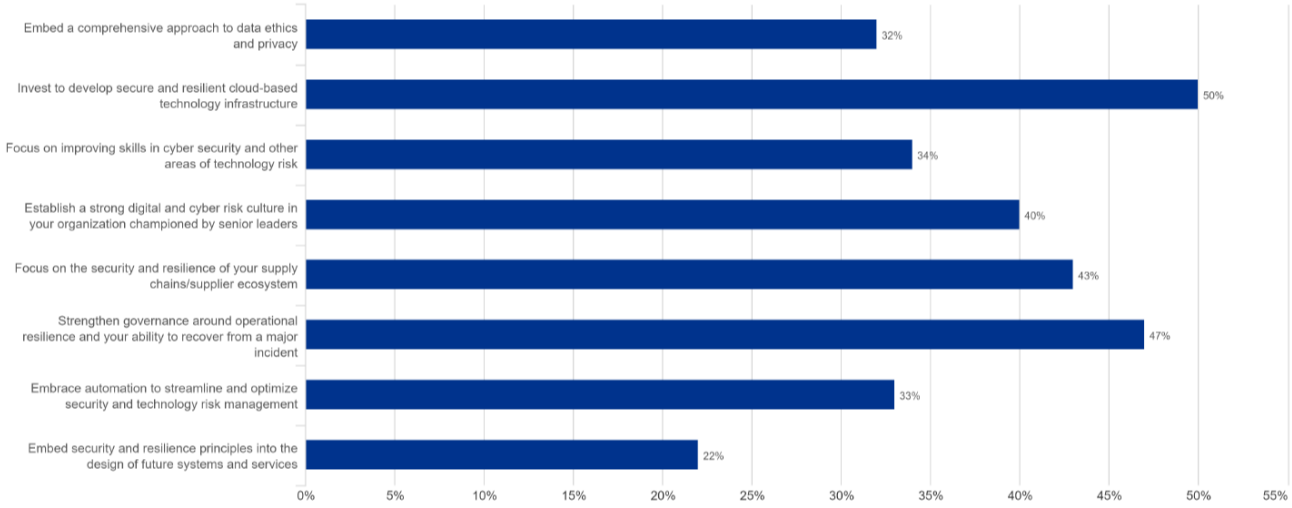
**Digital transformation has little to do with the technology itself; it's thinking about the value chain differently and how you interact with your end-customer**

It's funny, we did a previous study around digital, and [the sentiment] across time went from, we're really big on digital in the life sciences to, "God, we hate digital, we got no value on it." To now, it's all about digital again. I think organizations finally discovered the true value because of the pandemic. It was the last push for those companies to get it—that digital transformation has little to do with the technology itself; it's really about thinking about the value chain differently and how you actually interact with your end-customer. Life sciences firms are finally stopping the argument that it's the payer that's the customer, or it's the distributor that's the customer. No, it's the actual patient that's the customer.

So how do we push into that patient-care continuum and provide services around products that we already have in the market? What are those digital services that we can wrap around it? But in order to really do that, we have to rethink our value chain.

When companies first started talking about digital, it was much more functionally-lead. For example, digital transformation within finance or within commercial operations. Now, it's really thinking about that whole value chain, and using digital technologies to do things like, how do I look at supply risk and can I do that in a digital twin modeling exercise, and predict where I'm going to have supply chain issues? Not just from a security standpoint, but what if another pandemic happens? Where am I going to have choke

## What are the key steps you plan to take to build digital resilience over the next 3 years?




Source: KPMG 2021 CEO Outlook (pharmaceutical/biotech executives only)



points? Where am I going to need to get different APIs in from different areas of the world? Where do I have to set up an API manufacturing plant to make sure I'm not dependent only on India and China? You can do that with digital technologies, you can model that without really messing with your supply network—and can try it out in real time.

All that stuff is being embraced because the C-suite is finally saying technology is our strategy—we are going to become tech firms in the future.

**5. So as more pharma CEOs consider their companies digital disruptors, they must be seeing proof in action—these technologies paying off value-wise, correct?**

Well, think about the Covid vaccine itself—the race for a vaccine we had. You can debate all you want, but the use of analytics, AI, machine learning, real-world evidence—and ultimately platform-based therapies, which a lot of the [new]vaccines are based off of—all that happened because of technology. We wouldn't have been able to have something like 16 vaccine candidates in the course of eight months. Obviously there was a lot of money thrown at it and a lot of collaboration; I'm not saying it was technology only, but technology was one of the catalysts. It was like, wow, we can actually start seeing it come together.

**6. Are these trends also illustrative of senior leaders, in integrating data science and big data into their decision-making, increasingly weighing competitor and customer perspectives?**

People are understanding that in order to really be digitally-enabled, a lot of the stakeholders that are in this value chain—whether it be regulators, patient advocacy groups, or pharmacies—they all have to be part of the chain. Not that long ago, your front door to your healthcare was your primary care physician. Now your front door is virtual telemedicine, it's a platform-based service that helps you with your diabetes care, for example; it's the pharmacy where you can get your vaccines.

There's multiple front doors to your healthcare now. What has changed is the demand for all these digital services from a consumer experience standpoint.

**7. As you mention in a KPMG report on AI, 81% of life sciences executives want their company to be even more aggressive in adopting AI, and 93% say these tools have the potential to make their organizations more efficient. But you also bring up the disconnect at times in who is leading the AI charge inside these companies, citing a wall between what IT thinks is happening and the actual business execution on the clinical R&D and**

**commercial sides with the technology. What can be done to bridge those internal gaps?**

The disconnect, I think, is happening because of the nature of what IT historically used to be and where innovation actually happened within the life sciences or pharma company. If you think about drug discovery and R&D, and coming up with a new large or small molecule, that's innovative, right? So that's where the innovation used to happen, and IT was really a support-enablement function [to that]. I still think there's some history that says IT is not doing much AI because you're only seeing it from their four walls or what IT is responsible for, versus what's happening in the drug discovery and R&D space of a large pharma company.

At the same time, I believe that a bridge is forming kind of naturally—that there will be a time when we'll go to a pharma company and say, "where's the IT department?" and you won't be able to see it; it won't be as tangible as it is today. It will be very blended and integrated with the business operations within the value chain. It's not going to be this separate function; it's going to be included with cloud technologies, automation, AI, all that kind of stuff. It's going to be a part of the connective tissue of the value chain itself. That's a natural evolution and I think that bridge is already being built.

**I'm a big believer in disrupting from within, otherwise you get disrupted from external forces and you're on your heels trying to react**

**8. How much does the continued emergence and creation of new tech-focused C-suite positions at pharma companies, such as chief data officers, chief innovation officers, chief digital officers, etc., play into that equation?**

It's part of that. I'm a big believer in disrupting from within, otherwise you get disrupted from external forces and you're on your heels trying to react to it. I wrote this article around five years ago that asked, "CIOs, are you ready to disrupt yourself?" Because chief information officer is not enough. If you're just providing information and you're expecting the database to take that information and create innovative products or provide insight, there's no role for you in the future. Then, around the time that article came out, you started seeing CDOs, chief data officers; and new CTOs, chief technology officers. That trend is happening.

**9. So you're seeing adjustments being made not just fundamentally in IT function, but on the C-level operational side, as alluded to?**

On the C-level side, it's becoming so much more tech savvy. The business folks that are coming in with MBAs, they all know how to use technology. They start working in sales and marketing or commercial operations or quality control within a pharma company, and they're already looking for innovative ways to resolve problems using technology. The whole federated technology model within life sciences firms is being bred based on the talent that's coming into those organizations at a young age.

But because of that, they're also competing for talent against Google, Amazon and Facebook, and the next innovative startup. So how do you compete with them in order to basically say, "you're going to have a [leadership] path here?"

It's not just a talent issue, it's also the opportunity issue that they'll be able to apply the digital tools that they've learned.

**10. How do you view the potential of advanced analytics and data mining capabilities, including the use of predictive modeling, to generate reliable real-world data to really benefit patient and physician engagement—across all aspects of treatment and disease progression?**

I am very bullish on use of AI, advanced analytics and predictive model, not only for patient/healthcare professional (HCP) engagement—a current example is the "check-in process" that the CDC is running when you get your Covid vaccine shots, but also in the use of current therapeutics for different therapies and disease states. We are seeing the "real-world" use of RWE/D in COPD (chronic obstructive pulmonary disease), RA (rheumatoid arthritis), diabetes, heart disease and other aliments in both patient engagement, but also identifying 'tweaks" to either their wellness plans, type of therapy or amount/type of the drug/biologic.

What these firms' data scientists are finding is small tweaks based on personalized medicine on the information gathered via RWD can go a long way to treating the patient as an individual vs. a patient population. PCm

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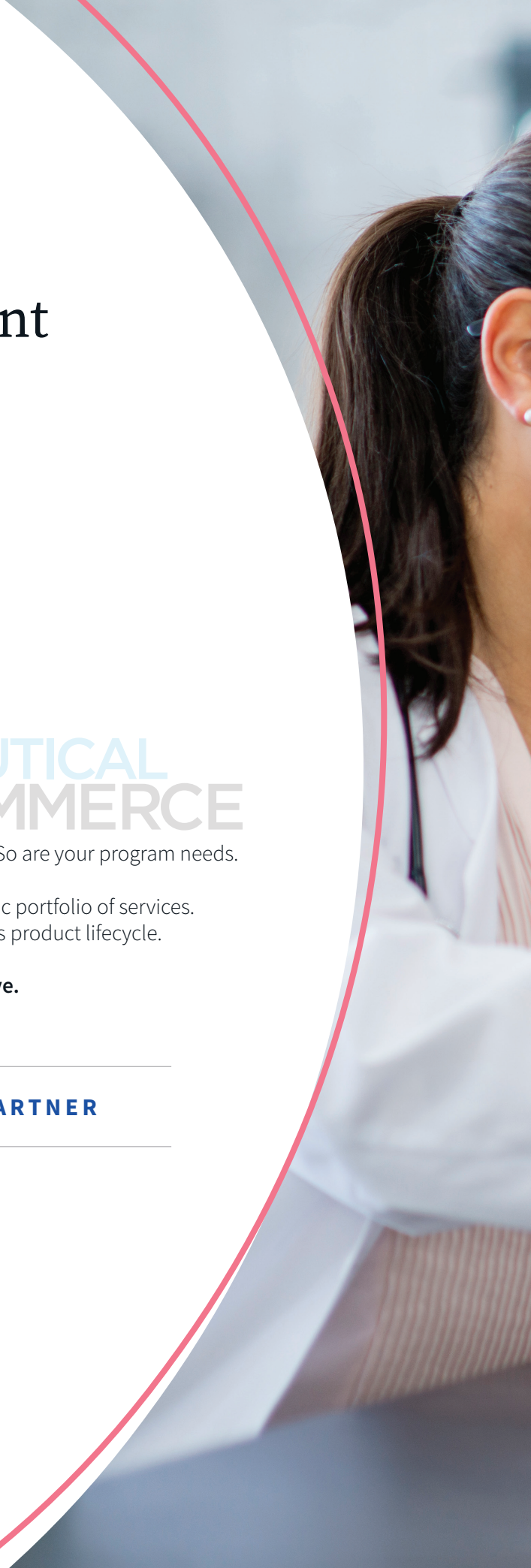

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# Pharma & Big Data: New Heights Beckon

Ongoing strides in data mining, data analytics, modeling and simulation are opening up new and more definitive opportunities to apply data-driven insights to improve everything from upstream drug discovery and clinical trials, to the downstream go-to-market strategy and post-patent lifecycle management

**SUZANNE SHELLEY, CONTRIBUTING EDITOR**

The ongoing evolution of computing and cloud capabilities, modeling and simulation methodologies and data analytics techniques continues to shape how the pharma/life sciences industry carries out all of its essential activities—from upstream drug development and regulatory approval to downstream commercialization and lifecycle-management efforts beyond patent expiry.



"By augmenting proprietary data with external sources of both real-world data (from payer claims databases, electronic health records [EHRs] systems and more) and aggregate data available in published clinical trial results, companies can dramatically increase their confidence in the most critical drug-development decisions and drive greater efficiency," says Matt Zierhut, PhD, vice president, integrated drug development for Certara, a biosimulation software and technology company.

"Whether it be a subpopulation where there is believed to be a particular advantage for the treatment, or a related setting outside of the original target population where benefits have been observed, strategically tapping into available external sources may help drug sponsors to validate clinical beliefs that were initially based on anecdotal evidence before making big investments in a new direction," adds Roman Casciano, senior vice president and head of Certara's evidence and access consulting business.

The goal of data-analytics efforts is to not only improve the operational efficiency of all phases of the clinical trial, "but to increase the probability of high-validity clinical findings that can support product-development decision-making and define all of the most promising therapeutic indications and the right patient populations to target, and then optimize the protocols to improve the odds of success for that particular trial," says Javier Jimenez, MD, PhD, executive VP, real-world evidence and late phase, Syneos Health, a contract research organization (CRO).

Another important use for advanced data analytics is to help ensure that clinical trials produce higher-validity, regulatory quality data, by sorting and classifying data-entry errors, outliers, inconsistencies and misreported adverse events that could undermine clinical development findings, notes Niranjan Kulkarni, PhD, senior director, consulting services for CRB Group, an integrated solutions provider to advanced technology industries, specializing in the life sciences markets.

A key challenge for high-cost prescription therapies has always been how to translate what happened in the trial to the actual outcomes that occur once the therapy becomes available, in order to justify its price. "Today, stakeholders no longer need to just model various scenarios hypothetically—they can actually track the product's clinical results in real-world use and conduct studies to validate more widespread clinical outcomes over time," says Jimenez.

Such studies can demonstrate how the product performs on its own, how it performs in comparison with competing products in the same therapeutic space and how it performs in

patients who are decidedly different from those enrolled in the underlying trial.

Similarly, by identifying patterns over time in actual prescribing and related healthcare data (particularly with regard to off-label use among real-world patients), biopharma manufacturers can sometimes identify promising—previously unknown—uses for the medication. When such additional indications can gain regulatory approval, the effort helps to provide additional clinical benefit for patients and ongoing revenue to the company. In September, FDA released draft guidance[1] that describes how real-world evidence (RWE) can support label-expansion discussions.

Today, there is growing collaboration among the traditional epidemiology community, data scientists, drugmakers and the regulatory community, who are working to develop methodologies that reduce bias in findings derived from data-analytics effort, says Jimenez, who adds: "The goal is to be able to design and carry out targeted studies involving real-world data (RWD) that are able to replicate the findings in a clinical trial, and to be assured that the data-driven clinical findings are real—not biased perhaps because of how the population was selected or due to other confounding factors."

## Improving clinical trial design and recruitment

The randomized clinical trial (RCT) process—as valuable as it is—remains inherently flawed, mainly due to the limited overall scope of any trial, in terms of the number and type of patients involved, trial duration and the clinical endpoints studied. "RWD studies can be very helpful in contextualizing results from clinical trials, especially where the trial population does not adequately reflect the diversity of real-world patients taking the medication," says Aniketh Talwai, delivery lead for Medidata Acorn AI. Recent work by that company with clients in the cardiovascular space to determine the real-world, age-stratified risk levels for some common agents, "found that they can vary quite a bit from the results published for the trial population," he adds.

"Today, RCTs, coupled with advances in computing power, accessibility and recording of additional data modalities via medical imaging and EHRs that are available for trial participants, and innovations in statistics, machine learning (ML), and big data analytics can help generate a completely new environment for drug exploration and validation," adds Yvonne Duckworth, PE, associate, senior lead automation engineer, Pharma 4.0 SME for CRB Group. Such efforts can also be used to quantify and prioritize unanswered clinical questions in the absence of published evidence, she says.

## Value-Driver Across Continuum

The ability to develop previously unavailable data-driven insights that have statistical validity and evidentiary value is helping drug manufacturers in many tangible ways to:

- Support or refute assumptions that were used during study design.
- Improve the identification of hard-to-find patients for trial recruitment (which is especially important in rare diseases and for biomarker-indicated therapies).
- Identify previously unknown patterns in both current and historical trial data and real-world data (RWD).
- Inform the overall go-to-market strategy.
- Inform negotiations related to innovative "outcomes-based" contracting and pricing models with payers, by allowing drug sponsors to articulate a richer, data-driven value proposition, based on evidence of favorable clinical outcomes in real-world patients and among targeted patient subgroups.
- Inform the overall lifecycle management strategy—in terms of identifying new clinical and financial opportunities that can benefit the aging therapy as it approaches its patent cliff.
- Repurpose older drugs using new evidence that could support additional regulatory approvals for existing medications in different therapeutic indications.
- Recognize and respond to factors that can undermine a patient's adherence to therapy (both in clinical trials and in real-world settings), to enable faster interventions.



However, not all types of RWE have equal evidentiary value. So-called research-grade RWE—which refers to high-accuracy evidence that is developed when complete medical records are linked with outcomes using advanced technologies—is required "to help clarify endpoints that would be of interest," says Anand Shroff, co-founder and president of Verantos, an advanced RWE company based in Menlo Park, Calif.

Such efforts "help drug sponsors to improve trial design and pressure test common flaw areas," adds Lucas Glass, VP and global head for IQVIA's Analytics Center of Excellence. He notes that such efforts support the ability to:

- Estimate the target study population and evaluate which inclusion and exclusion criteria are best.
- Compare potential study endpoints to recent similar trials to highlight similarities and differences that may help the drug sponsor to identify a competitive advantage to pursue or to better align efforts with emerging trends.
- Identify extraneous and missing procedures (extraneous procedures can be costly and impose unnecessary burden on sites and patients, while missing procedures can result in failure to achieve statistical significance on a primary or secondary endpoint).


*Credit: terovesalainen/stock.adobe.com*

- Examine patients' familiarity with significant or invasive procedures and identify sources of patient burden that may create barriers to recruitment and retention.
- Conduct sub-population analysis to better understand and predict how different patient cohorts will respond to new therapies.
- Analyze previously published RCT data to predict the probability of technical and regulatory success.
- Model disease progression

Using an appropriate data-analytics strategy, drug sponsors can also "merge databases from different sources to filter out patients that do not meet basic requirements for the clinical trial," says Duckworth. "Patterns in different studies can be analyzed using artificial intelligence (AI) techniques to evaluate risk factors and effectiveness of the intervention, help to create a general prediction model for the patient and assess the cost effectiveness of the treatment."

According to Talwai, Medidata Acorn AI is interrogating its compiled immuno-oncological trial records to identify risk factors for key treatment-related adverse events (TRAEs). "The findings can then be translated into a trial protocol that is stratified by baseline patient risk," he adds.

One of the perennial challenges in harnessing RWD is fragmentation across payer systems, EHR systems' diagnostic lab-testing records and other desirable sources of data. "One big advance in practice that looks poised to help finally overcome this barrier is the advent and real-world implementation of privacy preserving tokenization approaches, which can help to bridge patient data across silos in a compliant way," says Talwai. "We've seen this successfully implemented at scale recently through one pro-bono, public-private Covid-19 consortium involving nearly a dozen companies[2] (Medidata is a founding member)."

This effort brought together Covid-related records for millions of patients across claims, EHR and consumer data sources over a multi-year period, "which would have been immensely challenging to execute even a short while ago," contends Talwai.

Similarly, the design of the clinical trials carried out in pursuit of the Covid-19 vaccines "was driven directly by patient-level data and public health data that was studied to target areas with high early infection rates," explains Jimenez of Syneos Health. "Efforts to recruit patients more effectively really showed how making the best use of available data guided the decision-making and helped sponsors to evaluate the benefit of the vaccine in the trial. This helped to accelerate those trials much more than anyone anticipated."

## Using RWD signals to target adherence

Poor adherence to therapy within a clinical trial can undermine the caliber of the study findings and reduce actual clinical outcomes in real-world use. "In a typical trial that spans many sites, sponsors are sort of rolling the dice when it comes to ensuring that participants are actually taking their medications,"

says Rich Christie, MD, PhD, chief medical officer at AiCure. The company is using AI and ML tools, along with digital biometric markers (discussed ahead), to confirm that patients enrolled in a trial are staying on therapy. The resulting insights allow for faster human intervention from trial operators to keep the study on track. And Christie notes that now that AiCure has tracked more than a million doses for its clients, "we have the scale of data that allows us to go from just knowing who's taking their medications to predicting—with accuracy—which types of patients are likely to take them or not. This enables better real-time interventions."

When matching potential patients with specific trials, the use of digital biomarkers that are captured "in the wide part of the funnel" during screening efforts can help to narrow down the field to enroll the best participants. Examples include using audio and video tools to assess hand tremors in patients with Parkinson's disease, or to assess cognitive changes based on voice and language in Alzheimer's disease patients. "I think there will be a lot of real impact in this area in the next few years," says Christie.

**Stakeholders can convert inherent uncertainty into statistical risk that can better support decisions about resource allocation and portfolio optimization**



Similarly, advanced data analytics are being used to improve trial site identification by studying the prevalence/incidence of the target patient population in different countries, notes Glass of IQVIA. He says that in one recent client engagement, for example, the trial had very challenging inclusion/exclusion criteria whereby patients had to have been on a new therapy for 12 months prior to being eligible for the study. "We were able to see a comprehensive landscape of how much of that medicine was flowing through all hospitals globally over the past 12 months and avoid sites that were not yet prescribing the medication at scale," says Glass.

## Addressing knowledge gaps in trial results

Many clinical trials, on their own, don't have a sufficient sample size to use advanced data-analytics techniques to conclusively identify patterns or signals, but could be used for hypothesis generation," says Terri Madison, PhD, MPH, senior vice president and general manager, evidence and access, Certara. "However, we have several examples where we are either combining indication-specific data across trials, or across trials plus RWD sources, and then using AI, simulation and other advanced analytics techniques to strengthen understanding of a disease and the potential impact of therapy."

By way of example, Madison notes that in one rare, serious genetic disorder, AI has helped to identify factors associated with earlier disease onset and rapid disease progression— critical information that can then be used to fine-tune future trial inclusion/exclusion criteria and help in targeting trial participants who have the factors associated with rapid disease progression. "Such efforts would theoretically lower the sample size requirements (due to a larger anticipated effect size) or could reduce study duration (by enriching the trial with patients anticipated to have worse/accelerated disease-related outcomes)," says Madison. "This could also enable accelerated approval in certain patient subgroups, thus strengthening the investment via earlier commercialization of the therapy."

## Model-based meta-analysis

Today, model-based meta-analysis (MBMA) is a popular meta-analytic technique that incorporates pharmacological principles (such as dose-response) and outcome changes over time to yield longitudinal data. It is being used to synthesize rich aggregate datasets from prior clinical trial publications into more easily understood predictive models. MBMA, experts say, can be used to better define efficacy and safety targets that are needed to achieve differentiation, says Zierhut of Certara.

Specifically, according to Certara's Zierhut, MBMA is helping drug developers to:

- Reduce trial size and trial duration (lowering costs).
- Increase treatment effect precision at same costs (increasing confidence).
- Enable more precise assessment of relative effects for a new drug versus key competitors without needing a risky and costly head-to-head trial.
- Decide whether to advance or kill their own investigational drugs—before needing to gather definitive data from expensive Phase III trials.

"By analyzing consolidated insights from past clinical trials, stakeholders can also convert inherent uncertainty into statistical risk that can better support decisions about resource allocation and portfolio optimization," adds Talwai of Medidata Acorn AI.

AiCure's Christie believes the biggest factor to truly moving the needle in using predictive analytics to advance clinical and commercial objectives is to be able to do it at scale.

## Making use of unstructured data in EHR systems

While mountains of data are collected throughout routine healthcare activities today, extracting the most relevant information is not that straightforward. Today, by some industry estimates, unstructured data constitutes up to 80% of information available in EHR systems.

"Natural language processing (NLP) can help users to understand the unstructured data in medical records and thus help investigators to truly understand a patient's health status," says Shroff of Verantos. For example, it is difficult to look at the structured portion of the medical record and understand if a patient suffering from asthma has symptoms consistent with severe asthma over time. "However, NLP can inspect the unstructured data and help make that determination," he says, adding: "An ML algorithm can also detect comorbidities based on a number of data points, and a deep learning algorithm can predict patient outcomes based on extensive training and validation."

"When similar variables are collected for multiple patients across geographies, the data quickly becomes complex and abundant," adds Kulkarni of CRB Group. "Handling such a large number of variables and deciphering patters is not a trivial task." He says the data-reduction technique known as principal component analysis (PCA) is helping current pursuits. "The literature is rich with applications of PCA to identify common or underlying root causes in clinical settings, as well as for defining pricing strategies," says Kulkarni.

## Creating new opportunities for existing drugs

One particularly lucrative opportunity for data analytics in pharma is the ability to mine existing RWD related to both on- and off-label prescribing, and earlier trial data—and identify clinical findings that may allow existing drugs to be reevaluated as treatment options for entirely new clinical indications. This can be done sometimes in different patient subpopulations within the same disease category, and other times, in entirely new therapeutic spaces.

In October, a study funded by the National Institutes of Health (NIH)[3] found that a commonly available oral diuretic pill (bumetanide) may be a potential candidate for an Alzheimer's treatment for those who have a particular genetic profile. Specifically, NIH researchers looked at EHR data sets from more than five million people and split them into two groups—adults over 65 who display a certain genetic signature of interest (a form of apolipoprotein E gene called APOE4) and took bumetanide, and a matching group who did not take the oral diuretic. The analysis showed that those who had the genetic risk and took bumetanide had a 35%-to-75% lower prevalence of Alzheimer's disease compared to those not taking the drug, according to NIH. Ongoing studies are underway.

Meanwhile, in 2019, Pfizer made headlines when it received FDA approval for Ibrance (palbociclib; in combination with an aromatase inhibitor or fulvestrant) as a first-line treatment option for men with hormone receptor-positive/human epidermal growth factor receptor 2-negative (HR+/HER2) metastatic breast cancer. The novelty of this additional indication was that regulatory approval was based solely on studies of EHR and other post-marketing data—IQVIA Insurance database, Flatiron Health Breast Cancer database and the Pfizer global safety database—related to male patients who had the same form of biomarker-directed breast cancer, and were being treated with Ibrance off-label due to an unmet medical need and a lack of alternative treatment options. No additional clinical trials were carried out.

UK company Healx focuses on using AI methodologies (via its Healnet AI drug-discovery platform) to help identify novel therapy options and combinations of existing drugs that can help patients with rare diseases. In October, Healx received investigational new drug (IND) approval from the FDA,[4] along with an orphan-drug designation, for the Phase IIa clinical study of HLX-0201 (initially approved as a non-steroidal anti-inflammatory drug) for the treatment of Fragile X syndrome—the world's leading inherited cause of autism and learning difficulties, for which there are currently no treatment options available.

HLX-0201 was identified as a potential treatment for Fragile X syndrome by Healx's omic-based drug-matching methods, "which compare the gene expression profile for a disease with the gene-expression profiles from Healx's curated drug database to find entirely novel connections and disease pathways between the two," explains the company. Several other compounds identified by Healx's AI methods are also progressing toward the clinic, with the ultimate aim of finding a combination with synergistic mechanisms of action. Recruitment for participants in a clinical trial is set to begin in the coming months.

Credit: LuckyStep/stock.adobe.com

## The power and promise of predictive modeling

Thanks to the availability of vast data sources and novel data analytics tools, predictive modeling and simulations are also gaining traction among drug developers, to investigate a variety of queries. "Predictive modeling based on RWD can be used to make decisions at different levels—prescriptive analytics, predictive analytics and business-intelligence analytics," says Kulkarni. "The type of data that are required varies according to the objective—for instance, in some cases, the analysis requires wide data (ample data related to a sufficiently large population); in other cases, deep data (a significant amount of data about one patient) is required."

So-called artificial neural network (NN) learning models also "do a particularly good job of memorizing, generalizing and generating recommendations, as long as the available data is sufficiently wide and deep, adds Kulkarni. "Such models allow the user to enter certain inputs and contexts i.e., input to the EHR notes in natural language), and the models produce outputs in a hierarchical manner [as actionable recommendations]," he explains. "The wide and deep model when 'memorizing' will rely on frequency of certain keywords, features and the underlying 'intent', and use linear model (i.e., logistic regression) to determine an output."

One promising application for using simulations is to test the validity of tapping data coming from various wearables/digital health monitors—as surrogates of expensive and labor-intensive, gold-standard measures for the same endpoints, notes Madison of Certara, adding: "This could potentially save millions of dollars on future trials, if the right wearable proves to be as good as the current gold-standard measure for gathering key data during the trial."

Simulations based on RWD are also being used to evaluate what is the optimal treatment sequencing in crowded therapeutic spaces such as multiple sclerosis, where knowing which early/first-line therapies, or the optimal sequencing of therapies, can produce the best overall prediction of long-term delay in progression, "This could have a huge impact for patients, and for payer decision-making," says Madison.

## Creating insights that matter to payers

Increasingly today, there is growing focus on tying drug pricing and formulary decision-making to value—and in terms of actual clinical outcomes in real-world settings, not just early findings produced in the trial. When data analytics efforts are able to demonstrate better outcomes for different patient populations, "the two data sets become the basis for conducting drug-pricing negotiations," says Shroff of Verantos. "In the future, we expect to see real-world evidence being more rigorously produced in line with recent FDA guidance and becoming the basis for value-based contracting arrangements between pharma and payers."

Syneos Health's Jimenez believes the ability to study genetics and genomics information to better differentiate likely responders from non-responders has the potential to not just inform study design and trial enrollment but shorten the duration of the study, and thus the costs; reduce patient burden;

and speed time to market to address unmet clinical need. "And payers are keenly interested in this approach, because it is in their best interest to pay for products that will have a higher probability of clinical success in specific patients," he says.

"The question isn't whether a solid data-analytics strategy can support commercialization efforts—it is an absolute necessity, because without a doubt, the real-world situation is not as neat and tidy as one might expect if we were to read the treatment guidelines or listen in on the latest advisory board meeting," adds Casciano of Certara. "Our development programs and pivotal trials only give us a very narrow view of the heterogeneous treatment context."

Thus, Casciano continues: "It is essential to be able to take what we have learned from our clinical research and apply it into real-world situation with all of its nuance. This is an impossibility without the proper insights from a well-executed RWD program, and, frankly, no responsible payer would give favorable access to a new treatment without an assessment of the likely consequences in their population."

**No responsible payer would give favorable access to a new treatment without an assessment of the likely consequences in their population**

It is important to understand whether certain real-world patients that will get the treatment will be in any appreciable way different from those studied in the clinical trial programs. "if so, does this mean we should expect better or lesser results when compared to the trials?" says Casciano. "Without the answers to these kinds of questions, entering into a complex pricing or reimbursement arrangement would be nothing more than a leap of faith."

Pfizer is so confident in the real-world capabilities of its lung cancer therapy Xalkori (criotinib), for patients with metastatic non-small cell lung cancer whose tumors are anaplastic lymphoma kinase (ALK) or ROS1 positive, that in October, the pharma giant announced a new outcomes-based contracting arrangement called the Pfizer Pledge Warranty Program.[5] Through the program, the company will refund eligible patients for out-of-pocket expenses, and will reimburse payers (including private insurance, employer-sponsored health plans or Medicare Part D) if patients suspend their prescription (due to clinical reasons) before the fourth 30-day supply is dispensed. Refunds to the payer plan will be up to the cost of the first three bottles (30-day supply) of Xalkori—a maximum of $19,144 for each bottle, or an aggregate maximum of up to $57,432—according to the company.

"Similar to drug-pricing negotiations, payers are looking for evidence that a new therapy delivers additional benefits to their members before including the therapy in their formulary," says Shroff. "Pharma can support these discussions by measuring outcomes of a cohort over time and comparing them against outcomes of another cohort which is phenotypically similar but on different treatments."

Similarly, while formulary structures can be an effective way to help payers control spending, they don't serve all patients equally well. Consider specific patients (or categories of patients) who don't benefit from the top-tier formulary therapies, but are forced to cycle through them before getting to a more ideal treatment option.

"This adds costs and stress, and delays can impact quality of life, worsen disease progression and engender wasted costs," says Talwai of Medidata Acorn AI. "Insights from RWD and pooled clinical trial data can play an invaluable role here in identifying those patient subpopulations for whom preferred agents, drug classes and/or mechanisms of actions may have inferior efficacy or safety profiles." This combined intelligence, she says, can thus lighten the burden on patients and healthcare providers serving these underserved treatment segments.

"At the end of the day, data-analytics efforts should be used to bridge the endpoints from clinical trials to real-world value and to help address remaining knowledge gaps," adds Christie of AiCure.

When it comes to big data in the pharma and life sciences industry, the old adage applies: Go big or go home. PCm



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# When Operations & Analytics Collide

## How to best plan out and implement a marketing data hub as a key tool to harnessing today's omnichannel transformation in pharma

BY PHILIP DANIELS AND REBECCA LORENZO, AXTRIA

In recent years, the pharmaceutical industry has undergone significant digital and omnichannel transformations. Amid the global Covid-19 pandemic, the pace of this transformation has accelerated. Fewer face-to-face interactions between pharma reps and healthcare professionals (HCPs) forced many companies to cram five years of digital transformation into one year. For pharma companies, the call for truly mature omnichannel capabilities is growing louder.



In response, many drug firms have shifted explicitly to formal omnichannel strategies over the past few years. They have brought in customer experience (CX) experts, mapped out customer journeys and hired significantly from outside of pharma. However, building omnichannel capabilities into an organization's DNA is much more challenging than buying tools or hiring. Manufacturers face both regulatory and organizational constraints when undertaking these efforts. For companies looking to expand their omnichannel toolkit, a marketing data hub (MDH) is a great tool to help build a solid data foundation.

### What is a marketing data hub?

An MDH is a two-way data exchange that enables both marketing operations and analytics. This data repository should contain records of all marketing interactions with customers and serves as the basis for omnichannel marketing coordination. It is both an operational data store and an analytics data mart.

On the marketing operations side, an MDH helps coordinate real-time marketing activities in conjunction with traditional data sources like customer master lists or preference lists. It acts as a two-way data exchange between channel touchpoints, generating list feeds for a range of outbound marketing and re-marketing activities—be it email campaigns, targeted display ads or even webinar invites. Here, coordinated automation is a priority.

On the analytics side, an MDH should serve up cleaned and harmonized data, supporting an array of analytics activities, from the channel and campaign-level performance to segmentation exercises, marketing mix and machine learning. Literally and figuratively, an MDH is the central data hub around which omnichannel execution and analytics revolve.

### Break from tradition

While MDHs, traditional data warehouses (DWs) and customer data platforms (CDPs) serve similar functions, they differ significantly in complexity, time-to-build and more.

DWs are often read-only databases intended to support reporting, business intelligence and analytics. DWs tend to be IT-centric projects with correspondingly long development times. Due to perennial scope creep and constantly changing

business needs, the success rate of DW projects over the past 20 years has been shockingly low.

In contrast, CDPs provide digital coordination and customer identity resolution in specific digital spaces. CDPs also add some level of marketing automation and insights. CDPs are digital-first, working well for fast-paced e-commerce or consumer-oriented organizations with relatively straightforward customer activity flow. Pharma, however, must consider a range of privacy concerns, fragmented data inputs, overlapping lines of authority and the added complexity of rep-driven marketing. For these reasons and more, pharma can face challenges in adopting CDPs successfully.

An MDH is typically small and flexible enough to jumpstart within a few business cycles, while creating significant impact at the enterprise level. It is not a read-only exercise like a DW, nor is it digital-centric like a CDP. Unlike most DW projects, minimum viable product approaches to MDH implementations can be developed quickly.

## Objectives for a marketing data hub

Here are three broad objectives for any MDH implementation. An MDH should:

1. Coordinate and automate interactions between marketing systems and channels.
2. Enable rapid customer and channel performance insights.
3. Enable granular insight into data availability, data quality (DQ) and analytics readiness.

## MDHs in action: A real-world example

A top-10 multinational pharma company with a complex marketing ecosystem faced several enormous data challenges, including inconsistent data availability and poor DQ. The company's ecosystem included 120+ distinct data vendors, 12+ direct-to-consumer and HCP marketing channels and 100+ monthly inbound files. These challenges resulted in impeded analysis and insight generation and choked decision-making. The company needed faster, analytics-ready data to support more frequent marketing spend recommendations and analytics at scale.

To improve data issues, Axtria employed its MDH concept against nearly $150 million in spending. As a result, the pharma company reaped the following benefits:

- 400% faster time to data readiness.
- Clear line-of-sight into data status and quality for all data, with quality key performance indicators (KPIs), tracked across time.
- Always-on channel performance insights.
- Near-real time channel coordination capabilities to support marketing operations.
- 150% faster turnaround times for complex analytics via "analytics-ready" data sets.

## What principles should organizations follow in early-stage MDH planning?

**1. Don't try to solve everything at once.** Does the company need to fix a fundamental operational problem around marketing coordination? Perhaps, they must work more quickly to differentiate good versus poor campaign performance or are facing vendor DQ challenges or delivery challenges. Solve a few core problems quickly and ride that momentum into the next set of challenges.

**2. Go all out on DQ.** All the hard work and cool technology will be for naught if users don't trust the data. A basic set of DQ rules that evaluate data as it arrives will catch the most egregious errors. Finding errors before users do builds credibility.

**3. Publish both successes and challenges.** Socialize the status of the data, errors uncovered in the DQ process and the impact the MDH has on the organization.

**4. Envision what success feels like for the team.** Within most marketing data ecosystems, "cat herding" often occurs, where companies chase vendors to gather data and scramble to gather the latest data to generate the freshest reports. An MDH running on all cylinders ingests and processes data in an organized manner. That creates trust and confidence in data, which, in turn, empowers other teams to do their best work, too.

## Bottom line: Omnichannel requires robust and efficient data capabilities

Pharma executives should understand the differences between DWs, MDHs and CDPs. While building an MDH can seem daunting at the outset, teams with moderate technical skills can build the fundamental components in a short period. Importantly, those fundamental components are the same whether a company's marketing ecosystem contains 10 or 100-plus vendors' worth of data.

The results include significant near-term impacts to marketing efficiency, improved channel coordination, rapid performance insights and a clear-eyed awareness regarding the status of all the available data.

**ABOUT THE AUTHORS**

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# The Key Force in CGT Commercialization

Industrialized collaboration is critical to taking next-generation cell and gene therapies to the next level—if companies can build on pandemic-partnering momentum

BY ADLAI GOLDBERG, ERNST & YOUNG

Since 2020, the world has been in the grips of a faceless and cunning enemy. At this writing, according to the World Health Organization (WHO), there were 233,136,147 confirmed cases of Covid-19, including 4,771,408 deaths.



Thanks to science, we turned a corner with the development of not one, but four vaccines in the US and Europe. And, when we consider that vaccines often take years and sometimes decades to develop, test and approve for public use, we have to believe that something more was in play.

The life sciences industry has increasingly embraced external partnering or "co-opetition" to bring medicines to market faster. But the pandemic has shown how much more is possible with "co-opportunity," doing the right thing and creating opportunities for all, especially society.

At the height of the crisis, the degree of interorganizational collaboration between private and public entities, as well as large and small organizations, was unprecedented. In an April 9, 2020 press release discussing Pfizer's partnership with BioNTech on a Covid vaccine, Mikael Dolsten, Pfizer chief scientific officer and president, worldwide research, development and medical, discussed how combating the pandemic will require unprecedented collaboration across the innovation ecosystem, with life sciences organizations coming together to unite capabilities like never before.

Pharmaceutical and biotechnology companies, academia and technology joined forces to deliver rapid vaccines, diagnostic tools and therapeutics. It was, in effect, an industrialized level of collaboration that, if replicated in the cell and gene therapy (CGT) industry, would be the single most important factor in scaling these next-generation therapies.

Why and how should the CGT industry build on the momentum of pandemic partnerships to create a collaborative culture and an interoperable, digital ecosystem that ensures scalability?

## The industrialized collaboration movement

Medicine is transitioning—from mass-produced drugs to individually tailored therapies, from maintenance regimens to one-time curative treatments. With 17 approved CGTs to date and an active pipeline of reportedly more than 3,000 treatments in various stages of development, there could be an influx of 200 to 300 CGT product approvals between 2022 and 2027.

But these drugs involve complex manufacturing processes and a complicated, onerous supply chain. Currently, patients can wait an average of six to eight weeks for their treatments, and up to 90% of CGTs are not delivered as originally planned. In addition, advanced therapies come with higher-than-average prices.

How can these life-changing therapies scale up to help all those who can benefit, when the thousands of CGT patients today turn into hundreds of thousands by decade's end?

It's going to take a movement, called "industrialized collaboration," to advance CGTs. Building on the momentum of collaboration inspired by the coronavirus pandemic, its purpose is to help ensure CGT universal accessibility. And its vision is one of an interoperable CGT ecosystem.

## The barriers to universal CGT accessibility

The journey of an individual CGT, from the patient to the clinician, to apheresis collection, to multifaceted manufacturing and back to the patient, presents three main challenges:

**1. Fragmented supply chain.** Instead of the well-worn one-directional supply chain of mass-produced therapies, autologous CGTs depend on a bidirectional supply chain with nearly triple the number of steps. CAR-T therapy, for example, involves over 40 coordinated digital and analog handoffs between disparate teams of healthcare providers, manufacturers, third-party logistics companies (3PLs) and payers. Maintaining chain of identity and chain of custody while reducing the time it takes for therapies to move from "vein-to-vein" is critical. Some allogenic CGTs also have complexities in their supply chains that must be overcome. For both, the supply chain is fragmented, with many different software systems that don't "talk" to each other, limiting members' real-time visibility along the patient and therapy journey.

**2. Bespoke manufacturing.** With the expected increase in patients, the industry is focused on solutions that scale at 1,000 times the current rate. Companies are investing in technologies and larger production facilities; creating processes to help products scale or creating new products that scale better; and using technology to streamline quality control without sacrificing integrity or compliance. Still, the timelines are often too long and the therapies too expensive to reach many patients.

**3. High prices.** While CGTs can deliver tremendous value, there is uncertainty around response durability. Current models of drug pricing, which are based on utilization, won't work well. CGT companies and payers are considering new payment models, including outcomes- or value-based contracts (OBCs/VBCs), "drug mortgages" and subscription-based models, which charge a flat rate. Furthermore, there is an urgent need, highlighted by the pandemic, to extend essential services and offerings to the disadvantaged.

## The vision: an interoperable CGT ecosystem

The Covid-19 pandemic was declared by WHO to be a global emergency, a fire that needed extinguishing. But there are millions of people around the world battling their own fires, such as cancers, inherited genetic disorders, autoimmune diseases and chronic illnesses such as heart disease, diabetes, hemophilia, and HIV/AIDS. The CGT revolution offers them hope for a better, longer life and maybe even a cure.

Many in the industry are calling for better collaboration and industry standards—including a unified digital platform—to cost-effectively and safely facilitate the anticipated high volume of therapies and patients. But how do you build a plane while you are flying it?

## Cultivating a culture of industrialized collaboration

At the Alliance for Regenerative Medicine's Meeting on the Mesa in October, we asked participants to a workshop we hosted: What will be the greatest barrier preventing the needed collaboration to scale the delivery of CGTs—now and in five years? Respondents said organization and industry culture (23% now and 25% in five years) along with treatment journey complexity (19% now and 25% in five years) was at the top of the immediate and forward-looking time frames. We need to focus our attention on solving both.

Not until Covid have we witnessed the level of partnership required to address the CGT industry's current shortcomings. It is on an industrial level, where collaboration is woven into and throughout the fabric of the CGT industry and input is sought from all relevant stakeholders. These include the life sciences industry, logistics firms and medical, regulatory, policy, payer and patient representatives.

## Transforming supply chain into value chain

Now that patient numbers are small, the supply chain relies on highly manual processes by many skilled operators. But as therapies, patients and human touchpoints multiply, so will variability and risk. There is a need for a digital infrastructure that is secure, trusted and interoperable, to facilitate collaboration and real-time transparency and to ensure future scalability, shorter treatment delivery times and reduced costs.

EY teams estimate that a shared digital infrastructure can save the industry roughly $1 billion that they would spend building their own data systems. EY believes that a unified platform can also inform and improve treatments today and tomorrow.

**ABOUT THE AUTHOR**

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*The views reflected in this article are the views of the author and do not necessarily reflect the views of the global EY organization or its member firms.*

# Aligning Medical Information and PV

As companies seek interoperability across their organizations, a joint approach to medical information and local pharmacovigilance may deliver future benefits

BY ALISA HUMMINGS AND SIMON JOHNS, IQVIA

Pharmaceutical companies are in a period of significant change as they seek greater efficiencies and operational control through cross-disciplinary approaches across various departments and regions. As instant, digital communication from around the world has become more widespread over the past 20 years, the industry has widely acknowledged that there are opportunities to pivot toward more collaborative and interconnected processes.



Breaking down the historic barriers of entire companies—especially those with hundreds or thousands of employees and decades of legacy processes—can seem daunting and take years of careful, long term planning. One area of early focus is the potential across medical information (MI) and pharmacovigilance (PV), especially at the local or affiliate level.

By removing the historical barriers between local affiliates and combining certain elements of MI and local PV, organizations can become more efficient, creating greater consistency and improving overall customer satisfaction. Rather than treating these as separate and siloed business units, pharma leaders are noting that there could be emerging benefits from combining certain aspects of these two operations for heightened synergy, communications and, ultimately, patient safety.

## The current status and inefficiencies

Many pharma manufacturers face the challenge of managing numerous local affiliates across a multitude of countries. For MI, each local or regional contact center serves as the critical information source for customer inquiries. Meanwhile, a separate local PV team is responsible for adverse event (AE) intake, regulatory reporting, literature review and a source of local qualified person for PV (QPPV). These provincial partners frequently act as independent entities, meaning there is little transparency between them and the larger pharmaceutical parent company. Their varying approaches can have a negative effect on oversight, efficiency and what is actually happening on a global basis. Moreover, these individual approaches can be challenging to overcome with success relying on detailed planning, education, management and oversight to implement new and more standardized, streamlined processes.

Those pharmaceutical firms seeking synergies from handling both MI and local PV intake in a joint, more unified approach will often require partners to assist with regional issues such as language support and regulatory reporting. For instance, service providers can assist with MI inquiry management, translation and PV post-marketing safety activities. Staffs who understand local dialects reduce the reliance on translation vendors, increasing quality and reducing the cost of fielding, processing and transcribing these calls across the various countries. Often this can be done more efficiently from more centralized locations where staff can be shared. This also creates

more knowledge about specific regional requirements, for example, deep cultural knowledge and experience with specific local tools and templates in local language.

Use of technology can also overcome inefficiencies when combining some aspects of MI and PV and working in above-country models. Bringing in a centralized system is key, allowing information entered at the local level to be translated into English and sent to the global processing department to more quickly and accurately identify trends or areas of concern.

## The future of joint MI and PV strategy

Traditionally, PV operations and functions are managed by biopharmaceutical organizations as part of their compliance requirements, while MI teams were either part of the PV or medical affairs group and focused more on customer service.

MI teams are usually responsible for staffing contact centers and answering medical and scientific inquiries about new medicines, especially when they first come to market. However, MI also increasingly represents an important source of information around AEs. MI experts triage these events, gathering some initial data from callers and then passing them off to the PV team to intake, process and report.

With their background, however, MI staff can be trained to perform PV activities along with their current MI remit, such as local AE intake and entry into the global safety database, resulting in heightened efficiency. A centralized management structure for overseeing these local affiliates enables streamlined service delivery and improved compliance and oversight. For example, a major pharma company may be distributing products in 100 countries with a partner affiliate in each that operates by its own rules and processes. As a result, this highly disparate structure reduces transparency and introduces inconsistencies in costs, volumes and quality. The first step in creating a more cohesive approach involves analyzing what is happening at the country level—including such factors as the volume of MI inquiries and PV intake events—and then performing a gap analysis. Armed with new insights, companies can create a new plan, streamline processes and add technology to optimize resource deployment, including for low-volume geographic regions and during volume surges.

Improved oversight can result in greater standardization. The benefit of a joint strategy is immediately apparent from the planning and start-up phases and continues as experience is gained working at the local country level. When implementing a more structured approach to working with the various affiliates, pharma organizations will need to influence these groups to adopt a new style of working to ensure that reporting of information is done consistently. Transition support will be needed to adopt a more centralized, compliant approach to gathering and reporting information to ensure compliance, transparency and standardized KPIs at a global level. Cross-training teams on information and processes, along with introducing better communication technologies, allows for some greater synergies. However, it is important to note that MI individuals can be cross trained to support the PV team as needed to provide additional depth and knowledge to the team. That is not the case for the PV team; many are not trained at the proper level to assist with MI objectives.

## Next steps for leaders

The continued advancement and adoption of technology in the pharmaceutical space has reached a point where there are now tangible business benefits to creating more centralized management and interaction among departments such as MI and local PV. AI, automation and the cloud are transforming how information is shared, communicated and captured at contact centers. Technology will aid in the industry shift to a more combined MI and local PV strategy, jointly with a human-based model.

Training, education and new process implementation will also be required to break down the traditional silos that exist between functional areas, local affiliates and corporate headquarters. Thoughtful change management is also critical, incorporating cross-functional training and communication backed by deep industry expertise and process implementation that is unique to every organization based on its size and needs. Furthermore, drug manufacturers who succeed at bridging the gaps between local and global business units will be those that work collaboratively with industry experts with specific local-level knowledge to not just ideate a strategy that works for them, but also to oversee its implementation.

The question is not whether there are benefits to combining MI and local PV, but rather how to go about it in a successful manner. Employing life sciences understanding, advanced technology and a careful roadmap for centralized management and local implementation, biopharma companies can more fully realize the financial and operational benefits of MI and local PV connectivity while helping to mitigate risk.


### ABOUT THE AUTHORS

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# Chatbot Potential in Clinical Research

## How AI-powered support automation can solve the data problem in clinical trial management

**BY DAVID KARANDISH, CAPACITY**

Throughout the life of a clinical study, research associates, site coordinators, principal investigators and patients contend with an overwhelming volume of data: documentation, visits, calls, video meetings and emails. Executing, collecting, recording and filing trial documents may seem like a mundane part of the study process. However, the information within those documents can mean the difference between a trial that moves to the next phase and one that stalls out.



From interviewing and prescreening hundreds of potential patients to onboarding site staff, maintaining compliance and tracking patient progress, clinical trial teams face a host of competing priorities in need of their valuable time and skills. Neglecting any facet of the life of a study has consequences, none more so than the ability to recruit and retain the right patients to give a trial the best chance to succeed.

AI-powered support automation platforms offer solutions for patients and trial administrators, providing an interactive, informative interface, integrated cloud storage, intelligent document processing and a centralized knowledge base that keeps critical information accurate, accessible, and secure. Potential patients can learn about trials and start enrollment processes, while trial team members can complete patient selection, site compliance and trial analysis tasks more efficiently.

### Improve the patient experience, starting with recruitment

Deloitte projects that all clinical trial stakeholders will move toward processes that center the patient experience in an effort to recruit and retain committed patients to support clinical trial goals and get more trials to the finish line. An AI-powered support automation platform can improve clinical trial recruitment and retainment with features that cater to potential patients while facilitating faster, more efficient recruitment selection for trial goals.

The recruitment process often starts with a website designed to give straightforward information to prospective patients who fit the criteria needed for a given trial. While a website with study information helps patients learn about the study itself, it doesn't help research organizations glean any information about their prospective recruits. For that, companies often rely on site visitors to contact them via an email address on the website. Then, trial team members spend time reaching out to each prospect, responding to questions and interviewing the prospects, first to see if they qualify for the trial and then to learn whether they're interested in participating and likely to stay with the trial. Since not all the interested candidates will qualify, and some will decide against participating, team members waste time and resources on those lengthy interviews.

By implementing an AI-powered conversational chatbot, organizations open up the line of communication on their recruitment websites. Prospective patients can ask questions about the study and get instant responses about a trial. The chatbot answers by leveraging an organization's knowledge base, which is constantly updated to ensure accuracy. That means the chatbot gives the most current answer every time. If it can't find an answer, it seamlessly escalates the request to a human expert who can answer a site visitor's inquiry. The human expert's responses are then added to the chatbot's knowledge base, improving its abilities as it works.

While the chatbot informs site visitors, it also determines whether prospective patients fit trial criteria, saving team members hours of time and effort and providing them with a smaller pool of better choices for a trial. A chatbot learns about those prospective patients through guided conversations created and customized by trial administrators, giving the chatbot clear paths to follow as site visitors respond to its questions. An AI-powered chatbot can start the recruitment process, collect patient information and conduct prescreening questionnaires, making it easier for study coordinators to step in and complete the process.



## Equip team members with actionable information, quickly

While much trial data is processed through electronic data capture, built-in system redundancies often mean humans are still manually sorting and moving records, leaving room for human error in e-filing and categorizing. And because each trial uses multiple clinical sites required to provide the same kinds of paperwork at regular intervals, the chances of sending documents to the wrong people or filing them in the wrong place are high. Too often, clinical trial documentation takes a slow journey from clinical site to research associate, finally landing on the desk (sometimes literally, as paper documents continue to plague the industry) of a trial assistant already working through an avalanche of papers.

Clinical research associates need documents to be timely, organized and accessible. That ideal scenario facilitates accurate reporting, follow up and next steps. Frictionless retrieval of accurate trial documentation, enabled by support automation, is essential to a clinical study's compliance, safety and success.

The same chatbot that engages potential patients—answering questions and directing them to relevant information—also works for trial team members, mining an organization's knowledge base to respond to inquiries about trial processes, contact information and site communication. Rather than searching for a specific document or data point in multiple places or interrupting a co-worker, trial administrators can ask the chatbot. It will guide them to the answer, whether it's the expiration date on a medical license, an overview of a study population or a site coordinator's phone number.

As part of an AI-powered support automation platform, the chatbot can also initiate customized workflows that use robotic processing automation (RPA) to streamline work processes. Workflows initiate a series of actions, ensuring the right team members, documents and processes are engaged—all while integrating with other systems to send emails, upload records or create action items. By integrating workflows, team members don't have to think through the trajectory of a document each time they encounter a new one. They can work with a chatbot to follow a path that includes what needs to happen with a record, who should see it and what follow-up processes it triggers.

Intelligent document processing can function in tandem with workflows, allowing a trial team to create and follow templates for different kinds of documents, then batch upload trial records for processing. Machine learning classifies the documents against the customized templates, mines the data and pulls a human expert into the process to deal with any missing information. From there, trial teams can export the data, push the record onto its next step in a workflow or refine the template to capture information more precisely. Teams can verify the templates and provide feedback, so the system improves with use. The more documents are uploaded, the better the AI gets at sorting. And once records have been added, they become part of the knowledge base team members can access through the chatbot.

While a good support automation platform equips trial teams with the information they need, it must protect the security and privacy of the clinical work. Its deep knowledge base should have robust access controls that mirror the permissions trial team members have across applications to ensure the right people have access to the right information. That means if a team member asks the chatbot for information they can't access, the chatbot won't give it to them. In the same way, when a team member requests sensitive data, the system should provide it but keep the data private by not caching it in its server.

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# CSA a Game Changer for the Life Sciences?

## Exploring the FDA's latest proposed guidance on computer system validation, and the movement toward a less burdensome approach

**BY BRIAN STEPHENS, CAI**

In 1979, when the FDA began enforcement of 21CFR 210/211, a modern version of the good manufacturing practices (GMPs) for drug manufacturing, the pharmaceutical industry responded by creating a set of formal, documented internal practices and procedures to ensure that their manufacturing equipment, systems and processes met the requirements defined in these laws. The industry's execution of these practices and procedures, to ensure compliance to the GMPs, became known as validation, or process validation.



When microprocessors and computers became more involved in manufacturing processes in the 1980s, the FDA generated specific guidance for validating computerized systems. Because of the inability to visually view much of the software's operation, the complexity of some of the software programs, and the lack of familiarity of computers' structure and operation, early computerized system validation (CSV) guidance promoted the generation of massive amounts of formal testing and documentation to achieve compliance. This resulted in arduous and sometimes difficult validation efforts that were, and still are, burdensome to many projects that involve computerized GMP equipment and systems.

The amount of additional work required to meet the agency's computerized system GMP guidance motivated most life science companies to create specialized CSV departments to generate, execute and approve the tests and other documents needed to verify that these computer programs and systems were installed and operating as specified. The CSV effort had become a significant portion of the cost and time required to implement new or modified equipment and systems in the life science industry, in many cases becoming a barrier to process improvement.

In response to the expensive and time-consuming CSV processes, the FDA has been pushing industry to adopt a "least burdensome approach to validation" for the past several decades, with less-than-desired results. The latest FDA guidance that proposed significant changes in the industry's approach to validation, the *Pharmaceutical CGMPs for the 21st Century – A Risk-Based Approach*[1], was released in 2004. This guidance promoted the use of the latest technology, adoption of the newest quality methodologies and development of a risk-based assessment of system functions to achieve GMP compliance more efficiently than the methods used in the 1990s and earlier.

The industry has been slow to adopt the suggested changes in this guidance due to the initial cost to employ new technology and processes, the perceived lack of maturity of

some of the technology, and the resistance of companies to change their processes from the FDA "known and accepted" validation methodologies that they have successfully employed for years.

The FDA's latest proposed guidance on computerized system verification and validation, tentatively named *Computer Software Assurance for Manufacturing, Operations, and Quality System Software (CSA)*, is designed to help the industry overcome the obstacles that prevent the application of the least burdensome approach to validating computer-based equipment or systems. The proposed guidance will present the latest views that the agency has on testing and documenting requirements for non-product software used in the drug manufacturing process by presenting a new approach that emphasizes critical thinking early in the process to develop a verification plan that assures the software meets its intended use.

**The computer software assurance model is designed to move the industry to an intelligent approach that utilizes input from all stages of development and implementation**

This move from a CSV model to a computer software assurance model is designed to move the industry from the current validation process of completing a prescribed set of documents for every CSV effort, to an intelligent approach that utilizes input from all stages of development and implementation to verify that the system meets its intended use.

## Computer software assurance offers a streamlined and efficient approach

Although the proposed CSA guidance does not modify or supersede previous GMP guidance, and it does not change any existing predicate rules or 21CFR Part 11, it presents a different approach to meeting regulatory compliance than previous guidance and identifies two new strategies that manufacturers may want to use to assure the quality of software in GxP applications:

- First, the new guidance emphasizes the verification and validation process to prove a system's fitness for use rather than depending on the current method of generating a mandatory set of deliverables to achieve this goal. The proposed guidance supports a CSA program that uses rapid learning and continuous improvement to create plans to achieve compliance rather than the existing CSV programs that are locked into achieving compliance through the generation of a prescribed set of documentation, with little motivation for improving the validation process.
- Second, the new guidance includes expanded instructions on the use of automated testing as well as recommendations for the acceptable use of vendor documentation and testing in the formal documentation of system installation and operation. This approach reduces the amount of redundant documentation and testing generated during validation, which increases the efficiency of collecting and documenting the specifications, installation and testing required to verify that a system is fit for use.

In addition to making these suggestions to improve the efficiency of assuring that the equipment or system software is fit for its intended use, the FDA Center for Devices and Radiological Health's (CDRH) presentation on the draft CSA guidance proposed a four-step process to identify and apply the least burdensome approach for achieving computer software assurance. These four steps are:

- Identify intended system use in the specifications by listing all requirements that affect product safety, quality, efficacy or identification.
- Use risk-based assessments to identify each feature, function or operation's risk to product safety or quality and identify appropriate activities for each risk level to ensure that they reliably perform as intended. These activities could occur in any stage of the system development and implementation, based on the feature, function or operation's level of risk.
- Leverage and use existing activities and supplier data, as appropriate, to assure proper system operation. This includes automated testing results, electronically collected data and records, vendor documentation/testing

generated during system development and agile and unscripted testing created during unit and system testing and commissioning. Employ process controls to mitigate risk when possible, and use any information gathered on the system from the beginning of the selection process until the system is approved-for-use to assure system operation.

- Define and document appropriate records to decrease the focus on creating documentation "to meet compliance standards." Collect data that is useful for the manufacturer to verify that the system operates reliably and as specified; do not create or collect data for the sole purpose of satisfying an auditor.

## The benefits of a CSA guidance

The proposed guidance reiterates the FDA's desire to have life science companies implement quality and testing efficiencies that other industries have already employed to drive down product costs and increase product quality. The agency expects the CSA guidance to move the needle for the pharmaceutical industry more than the previous guidance because it presents a detailed road map that uses critical thinking early in the process, implements risk-based assessments during planning and leverages vendor documentation and testing during validation. The goal is to change the paradigm of computerized system validation from a massive, document-driven practice with redundant testing into one that requires reduced amounts of testing and documentation to achieve the same results—that the software is assured to perform as expected during commercial operation of the system.

## So, is CSA a game changer?

I know of several companies that have used "CSA-type" strategies for years, confident in their ability to show adherence to the Code of Federal Regulations (CFRs) as they reduced the amount of testing and documentation using aggressive risk-based approaches. There are also a few companies that completed pilot CSA programs at their facilities, using the guidance to significantly reduce the effort and cost of verification activities. But most organizations continue to follow their established CSV programs because of the barriers mentioned earlier.

This guidance, providing implicit FDA approval for the use of these methods, as well as some direction on how to implement them, should eliminate most of the apprehension that the industry has in moving to a CSA methodology to verify that computerized systems perform as intended. The guidance's promotion of critical thinking, rigorous risk-based assessments and use of automated testing and vendor deliverables should motivate all companies to embrace an efficient "FDA-approved" process that assures software quality for any non-product software.

**This could finally permit the FDA and industry to announce that they have actually defined the long-desired goal of a 'least burdensome approach to validation'**

This result could finally permit the FDA and industry to announce that they have actually defined the long-desired goal of a "least burdensome approach to validation," which will most certainly lower the cost and time required to develop and implement (or modify) GMP equipment and systems that use non-product software without sacrificing quality. These results would definitely make the CSA guidance a true game changer for the industry.

**ABOUT THE AUTHOR**

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# What's Next for Med Tech?

## Four trends shaping healthcare in 2022 and beyond

**BY ARIEL KATZ, H1**

The biopharmaceutical and healthcare news of 2020 and much of 2021 has been dominated by Covid-19. The pandemic has been a driver and accelerator of dramatic changes that will shape the healthcare ecosystem in 2022 and beyond. The impact of Covid and other important trends and new legislation were widely discussed at this year's MedTech Conference. Here are four that will have a major impact.

### Patients demanded ownership of their healthcare data—and finally get it

In April 2021, the Interoperability, Information Blocking, and ONC Health IT Certification, went into effect. This rule requires that healthcare providers give patients access to their clinical information (e.g., consultation and treatment notes) without delay. By late 2022, those clinical notes must also be shared with patients' health apps. While this might seem like a small change, it has a big impact on patients, who can now demand a copy of their healthcare record and are guaranteed to receive it in a timely manner.

In the past, it was the patient's responsibility to ensure doctors had all their medical records. This put an enormous administrative burden on patients, who had to keep track of the information, chase down physical copies (e.g., x-ray images) and personally deliver them to healthcare providers (HCPs).

Apps can reduce this burden and the Cures Act now ensures that patients have unfettered access to their data and can easily share it with other HCPs (e.g., specialists, emergency doctors).

### Patient data are better protected—and could start making patients money

The advantages and convenience of generating health data using electronic devices and health apps come at the price of privacy. While HIPAA privacy rules prohibit the disclosure of protected health information, it does not prohibit the commercialization of health data collected by fitness trackers, wearables and health apps. Currently, this data can be sold by the technology companies collecting it to health insurers, employers or third parties like Facebook without the consumer's consent or knowledge.

At MedTech, Sen. Bill Cassidy (R-LA) discussed legislation that would enable patients to opt out of the selling/sharing of that data and not be left out of money shared during the exchange of their data. Similar legislation will give individuals control of the data they generate and usher in a wave of possibilities, not the least of which is the commoditization of that data.

### Insurers have to provide accurate information about their physician network—or be fined

A recent study by The Centers for Medicare & Medicaid Services (CMS) found that almost 50% of providers listed in the directories of Medicare Advantage Organizations had at least one inaccuracy (e.g., wrong location or phone number) that can create barriers for members seeking services. As a consequence, CMS is now empowered to enforce hefty penalties for such errors, ensuring that patients experience fewer challenges in getting the care they need.

### Clinical trials will be decentralized, longer—and more inclusive

Covid forced drug sponsors to change the way they undertake clinical studies; wearables helped with moving from in-person to in-home or remote reporting and also allowed extended tracking of patients to generate more meaningful and longer-term data. The trends toward decentralization of clinical trials and remote tracking are here to stay and will make it more feasible for underrepresented communities to enroll in clinical studies, adding much needed diversity.

Comprehensive databases support this trend by giving life science companies the tools they need to better plan clinical trials and expand their understanding of investigators and sites.

The common theme of these trends is easier access to more data will improve health outcomes for patients and help generate more meaningful data, but safeguards are necessary to protect patients' sensitive health data.

**ABOUT THE AUTHOR**

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