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Can New Tech Outsmart Status Quo?

THIS MONTH, I ENCOURAGE THE READERS of Pharmaceutical Executive to read the December issue of our sister publication, Applied Clinical Trials. The issue focuses on technology and clinical trials software (eClinical), with an eye on where the clinical trials data collection process may have gone awry, may have caused inadvertent complications in the clinical trials process, but still with optimism on how future technologies can improve all aspects of drug development.

In a previous article, Henry Levy, chief strategy officer for Veeva, provided a media roundtable with a brief history of clinical data. “Thirty years ago, electronic data wasn’t common and all of your data was in one place, and it was ugly, but it was centralized and managed in one place. And then EDC was a revolution, with companies like Oracle and Medidata coming in, which was a massive improvement—and that was a really good thing. But it actually broke everything else, because now you had a clinical data management system (CDMS) that was supposed to clean your data, and then you had an EDC system that was doing a part of the data, which at the time, 70% to 75% of your data was EDC data.”

Recent surveys, along with Levy’s anecdotal information, report an explosion in the number of clinical data sources, with EDC comprising only 20% to 30% of clinical data, along with ePRO, mHealth, lab data, and more—leaving CDMS trying to catch up in a decentralized and not-well-integrated landscape.

In addition to the plethora of clinical data sources, David Connelly, CEO of Cmed Group, states, “Over the last decade or so, management of clinical data has been driven down the commodity route, with off-shoring encouraged to save money. In some cases, job roles have been made narrower to allow for a more task-based approach with rapid training of less experienced resources. Nothing wrong with reducing costs, but with clinical trials becoming more sophisticated, the number of data sources increasing and the types of data more complex, maybe this strategy needs to change. Surely we should be applying greater expertise and sophistication to derive valuable information from the data, and sooner? Clinical trial data is not an ancillary byproduct. It is the output of the clinical trial and arguably the whole purpose why the trial was conducted in the first place.”

Connelly continues with the fact that biopharma of all sizes take large risks and spend millions to billions of dollars on R&D for new and better treatments. And maybe spending resources on innovation and management of clinical trials would be well spent.

In January’s issue of Pharm Exec, we will take a look at the trends that will impact the C-suite in 2019. We don’t discuss clinical trials but we do look at technology, specifically uses of AI that are currently in practice and ones that could easily be adapted to the pharma model. Sanjiv Sharma, VP, North America commercial operations, HLS Therapeutics, and Pharm Exec Editorial Advisory Board (EAB) member, believes that AI is going to creep up faster than we think. “The permutations of AI, and adding in real-world evidence, will have impact on the drug and diagnostic areas for the next four to five years,” he says. John Furey, chief operating officer, Spark Therapeutics, and also an EAB member, agreed that AI is already being used to find patients as well as in determining the prospective care of patients. EAB member Jay Galeota, president and COO, G&W Laboratories, says that hospital systems are using phenotypic screening to determine high-responders for certain drugs so they can make P&T decisions based on these algorithms.

Technology layers onto old practices that they were meant to eliminate or reduce

In many industries, it appears that technology and innovation is far surpassing individuals’ and companies’ ability to change course and adapt to the changes. Technology layers onto old practices that they were meant to eliminate or reduce. Technology that was meant to make efficient use of resources that then generates its own inefficient department. As in the cases cited by our EAB members, intelligent uses of technology are being implemented and will—gradually or extremely quickly—break down the status quo.

I was going to close with a comparison of protecting your archaic model vs. creating new models with a not-kind comparison to Comcast and its lack of transparency for its services and prices listed on its website. However, I have to travel overseas and call them when I get back, probably devoting one-third of my day to their sales team. Wish me luck as I try to bridge the new world of technology with the tried and true status quo.
Pharmaceutical Executive’s 2018 Editorial Advisory Board is a distinguished group of thought leaders with expertise in various facets of pharmaceutical research, business, strategy, and marketing. EAB members suggest feature subjects relevant to the industry, review article manuscripts, participate in and help sponsor events, and answer questions from staff as they arise.

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Real-World Evidence

Securing a Winning RWE Strategy
Julian Upton, European and Online Editor

While the increasing importance of real-world evidence (RWE) is widely acknowledged, the dramatic shift required by biopharma companies to embed and secure an RWE capability effectively across the organization is still a work in progress.

Science & Finance: The Biotech Blend

Lisa Henderson, Editor-in-Chief

Pharm Exec convenes a panel of financial leaders from clinical-stage biopharma to discuss the critical role of finance and accounting (F&A) in supporting and sustaining the promising science at the forefront of investor engagement.

Market Access

Predicting Access Success

By Jonathan Chee, Betty Pic, Julia Fryhardt, and Evelyn Siu

There is still no structured method of assessing pricing and access risk for drug manufacturers. To that end, authors present a straightforward measure for integrating pricing and access risk into portfolio planning and decision-making.
2018 Pharm Exec 50
June issue online
Michael Christel
bit.ly/2yOuPSQ

2018 Emerging Pharma Leaders
October issue online
Pharm Exec staff
bit.ly/2PBEmbs

Pharm Exec’s 2018 Pipeline Report
November issue online
Joseph Constance
bit.ly/2P6FOKh

Top 10 Industry Trends to Watch
Blog post
Archbow Consulting
bit.ly/2APV9rt

Pharma Blasts Pricing Proposal
Blog post
Jill Wechsler
bit.ly/2BGC52F

Most-read stories online:
October 25, 2018, to November 24, 2018

Episode 20: Pipeline Peek
Pharm Exec editors provide a glimpse into the magazine’s 15th Annual Pipeline Report, published in the November issue. Topics include cannabis, CAR-T therapy, opioids, biosimilars, and Alzheimer’s.
b.it.ly/2B6tNkd

Episode 19: The CEO Career Pivot
Pharm Exec speaks with Mei Mei Hu, CEO of United Neuroscience, about how being open to pivoting from your original plan can create a number of opportunities for an executive and their company.
b.it.ly/2JqWJGk

Episode 18: Getting ‘Real’ on Data
Pharm Exec editors sit down with one of this year’s Emerging Pharma Leaders, Christopher Boone, the head of real-world data and analytics at Pfizer.
b.it.ly/2Q7JzAz

Episode 17: Commercializing Research
Martin Low and Phillip Low, CEO and co-founder, respectively, of On Target Laboratories, talk with Pharm Exec about the ups and downs of research that has been previously shelved—and share their strategy in approaching a potential partner to license their technology.
b.it.ly/2mLIW5

Episode 16: Rise of Specialty Pharma
Pharm Exec editors discuss the topic of specialty pharma—featured extensively in our September issue—touching on the areas of marketing, logistics, pricing, and other challenges executives in this once-niche market face.
b.it.ly/2MM0RI

Episode 15: Robotics in Pharma
Learn how life sciences companies are using robotics and AI to enhance patient care, what the funding landscape is in this sector, and what C-suite members need to do now to be at the forefront of this emerging technology.
b.it.ly/2Pk8MqP

On-Demand Contextual Information: Bringing Intelligence to Drug Discovery
bit.ly/2Q5C9O9

Seizing Pharma Market Opportunities in Japan
bit.ly/2BI2XiP

Biopharma Panel: Launching on Your Own
bit.ly/2CHfQLM

Twitter Talk
We spoke to @PharmExec on how the #pharma and #medicaldevices industry can overcome some of the biggest challenges it faces by embracing new technologies in order to empower workforces and allow the industry to move forward. #labeling #artworkmanagement

KalliKAMS, @KalliKAMS
“Labeling: Keeping Up with Compliance”
b.it.ly/2zwXaek

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2019 Industry Outlook
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Challenges to pharma pricing models have escalated this past year, and now threaten to impose significant changes on industry marketing and new drug development. The imperative to reduce spending on prescription drugs is one area of agreement between Democrats and Republicans, and with control of Congress now split between the two parties, political leaders are looking to curb outlays to promote public health and patient access to medicines.

This past year brought kudos to biopharma companies, as manufacturers tested, and FDA approved, multiple innovative medical products, including important new gene therapies, cancer treatments, vaccines, and complex generics and biosimilars. Such advances have benefited from FDA efforts to streamline clinical testing methods, clarify regulatory policies, and accelerate application reviews to speed new therapies to market.

Despite these achievements, industry faces ever more severe criticism for setting prices based on marketing and financial strategies and not actual costs. Traditional claims that high returns on investment in R&D are needed to support costly research programs have lost credibility. Leading manufacturers and industry critics alike acknowledge that list prices reflect what the market will bear, and not underlying research and production outlays, and that drug prices in the US greatly exceed those in other countries with central healthcare systems able to control drug coverage and spending.

The mounting campaign to bring pharma prices more in line with global trends has produced a range of strategies for reshaping biopharma coverage and reimbursement in the US. Congress considered dozens of drug pricing bills over the past year, but only approved measures to eliminate “gag clauses,” which prevent pharmacists from informing patients of cheaper alternative medicines. In May, the Trump administration announced a broad blueprint to lower drug prices that attacks rebates paid by manufacturers to pharmacy benefit managers (PBMs) and payers and proposes notable changes in how the Centers for Medicare and Medicaid Services (CMS) pays for drugs. A recent proposal aims to increase “transparency” in drug costs by requiring manufacturers to disclose list prices in direct-to-consumer (DTC) ads.

A main target is to reform Medicare Part B drug reimbursement, which primarily affects injectables administered in doctors’ offices and hospital clinics to treat cancer, rheumatoid arthritis, eye disorders, and immune disease. A new “International Pricing Index” (IPI) payment model links Part B reimbursement to the average price paid in foreign industrial nations and is projected to save more than $17 billion over five years. The Trump administration proposes to launch it as a pilot plan to avoid waiting for Congressional approval. CMS also looks to reduce spending by Medicare Part D plans by limiting coverage requirements for “protected drug classes,” and it is authorizing state Medicaid programs to enter into value-based payment arrangements with manufacturers. 

On the defense
Industry is mounting strong opposition to the Part B reform and the IPI model, but may find limited support. In the fall, pharma companies failed to scale back an earlier Medicare policy change that increased manufacturer discounts for Part D drugs covered by the “donut hole” by an estimated $4 billion over five years.

Several leading pharma companies reduced prices or delayed rate hikes this year in an effort to quell the mounting outcry. In July, according to press reports, Novartis and Pfizer said they would defer mid-year price increases until the end of the year. Merck & Co. and Amgen similarly announced price cuts on certain products and delays in increases.

But such voluntary action is isolated and uncertain. More reports and public hearings on drug pricing will come as House Democrats take over key investigative and health policy committees. A recent report from a bipartisan Congressional caucus attacks high prices for insulin and urges payers to eliminate rebates and shift to outcomes-based payment contracts for diabetes drugs. Even though Republicans maintained their majority in the Senate, leaders of both parties agree on the importance of making new medicines more affordable for public and private payers and for consumers. The larger challenge for all sides is to maintain incentives for biomedical innovation in such an anti-pharma climate.
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Europe’s Digital Health Path Still at a Crossroads

The Commission’s gestures toward enacting formal standards to digitize health records are masking wider policy gaps

The subject of health merits only one mention in the European Commission’s entire planning for next year—and that relates to electronic health records. Advocates of digital health might argue that this shows a belated European Union (EU) recognition of the ever-widening opportunities of digitalization. Cynics might counter that the choice shows how little the EU cares about health in general, and that it has merely taken the easy option of a nod toward a small corner of its wider strategy on the digital economy.

Both could be right.

The formal recommendation in the 2019 action plan is to establish a format for a European electronic health record (EHR) as from the first quarter of the year. This certainly fills an obvious gap in EU planning. Europe’s embryonic eHealth infrastructure is at present limited to patient summaries and e-prescriptions, and does not cover EHRs. Exchange of patient data from one EU country to another currently depends on the voluntary cooperation of health authorities, and is subject to all the challenges of non-standardized systems.

So the Commission is pushing for agreement among national authorities on an EHR exchange format based on open standards—and, of greater significance for pharmaceutical executives, it wants the infrastructure future-proofed to take account of the use of data for research.

The aim is to link EHRs to the currently scattered national and regional banks of -omics data, biobanks, and other registries across the EU. Already there are officials within the Commission who are talking of access to one million sequenced genomes in the EU by 2022, and to a prospective population-based cohort of at least 10 million people by 2025. They see the scope for links to integrated molecular profiling, diagnostic imaging, lifestyle, microbiological genomics, and environmental data.

Action lacking

All very ambitious—in theory. The problem is, there is no real mandate for the EU to act here, and no real money either. Some EU countries—Estonia is always cited as the poster-child—are already heavily committed to taking advantage of digitalization in general, and on health in particular. Many show little enthusiasm. Most do not inject the resources into digital health to make a reality of it—and without that, no EU encouragement can make much difference. The Commission’s action plan makes only an imprecise passing reference to the EU’s own limited research and IT support programs—but as an expression of optimism rather than commitment.

There has been plenty of lip-service over the last few years to the perceived potential of digital health. A European Parliament request back in 2015 urged “improving patient safety to be explored, inter alia, via electronic health records.” The EU Health Council remarked in December 2017 on the need to “remove obstacles to data exchange and sharing between health professionals for the safety and continuity of care.” And a Commission think-paper in April criticized the “incompatible formats and standards in electronic health record systems (that) continue to be used across the EU.” It mooted promoting interoperability of member states’ EHR systems “by supporting the development and adoption of a European EHR exchange format.”

Now, all that has emerged is a skeletal item in the work plan for what is, in any case, a lame-duck administration. This is the last year in office of the current European Commission, before a new set of commissioners—and a new president—is appointed to take over for five years from next November. So the EHR initiative is as easy to depict as a glass half empty as a glass that is half full.

Curious omissions

What is absent from the Commission work plan for 2019 is perhaps as revealing as what is included in it—and particularly for pharmaceutical company executives.

There is nothing, for instance, on what is going to happen to the...
EU’s drug-research incentive schemes. Despite all the fevered debate since the Netherlands suggested back in 2015 that pharmaceutical companies were abusing orphan drug therapy awards or the benefits offered under the pediatric medicines scheme or the supplementary protection certificate, uncertainty will continue to hang over the future.

A Commission official involved in these lengthy reflections confirmed in November that no moves toward decisions would even begin to be discussed until 2020.

There is nothing either on what the EU should or should not do in respect of drug pricing—despite increasingly loud clamoring for tighter controls, particularly on high-priced innovative products. Hardly a day goes by in Brussels without a new call from some influential quarter for radical change, principally a stricter reimbursement system.

There is nothing on how to reconcile the conflicts facing the research-based industry, which feels—as senior executives candidly admitted at a conference on pediatric medicines in Brussels in late October—under growing pressure, caught between governments and society demanding cheaper drugs, and physicians and patient advocates urging increased investment in R&D.

The pressure is intensified by the wave of concerns now sweeping European activist circles that society is paying twice for new medicines—not only in reimbursing pharmaceutical and biotechnology manufacturers, but in subsidizing their research through public funding of the underlying science they depend on.

Nor is there anything on how the Commission intends to square the circle of Europe’s health technology assessment (HTA) debate.

Its proposal of streamlining the multiple national HTAs into a single agreed joint assessment at EU level is stridently opposed by national governments and the European Parliament demanding any number of get-out clauses that would in essence perpetuate the current duplication, and defeat the object of the exercise.

**Future feasibility**

The debate rumbles on without any sign of a breakthrough, leaving pharmaceutical executives facing persistent uncertainty over when they can expect a more rational European approach to evaluation of their products.

And there is nothing to signal any clear pathway toward adapting regulatory procedures that can accommodate the needs of the growing number of new therapeutic approaches, where classic randomized clinical trials may no longer be the best procedure for evidence generation to support marketing authorization applications for new treatments.

For more than five years, the discussions flashing across Europe have illuminated the urgency of building on real-world evidence, even in small populations, and on opening up the rigid frameworks of 20th century regulation.

Innovative efforts, particularly in rare and complex diseases, could benefit from a more flexible and benign climate, but opposition from national regulators and payers, anxious over relinquishing their habits, seems to have stymied for the present the thinking that had been pioneered not just by industry but also by the European Medicines Agency (EMA).

In other words, on many of the strategic issues of concern to the pharmaceutical industry right now, the Commission has little or nothing to say. In its defense, it must be acknowledged that the Commission’s own powers are limited, and especially on health. Much of it remains under national control—and therefore subject to agreement within the European Council of Ministers.

But those finer constitutional distinctions matter little to a company facing real-time decisions about its European future. Regardless of which EU institution or national government is to blame, the situation for companies seeking a conducive operating environment is always going to be judged on feasibility—and right now, feasibility is difficult to judge in European health policy.
Biotech Business Ramp-Up: Adding Credibility to Narrative

Financial leaders from clinical-stage biopharma gather to discuss the critical role of finance and accounting (F&A) in supporting and sustaining the science at the forefront of engagement with investors.

At the recent CBI Finance and Accounting for Bioscience Companies conference, professionals from small- to mid-sized biopharma gathered to learn from each other and discuss their unique challenges. Typically, smaller-staffed organizations, with their smaller-staffed departments, require their executives to be accountable for much more than those serving at a larger pharma manufacturer. They wear multiple hats, get involved with diverse projects, and make decisions impacting the future financial vitality of their respective organizations—and their scientific prospects as well. RSM, hosting a breakfast roundtable for these professionals, graciously allowed Pharm Exec to moderate “The Role of the Finance Leader in
Scaling the Biotech Business.” What follows are edited excerpts from a very insightful and robust discussion.

**PE:** How do you stay on top of both the financial insights as well as the science information needed to do your job effectively?

**STEPHEN GARBACZ, Spero Therapeutics:** In terms of obtaining the latest financial information, I use online systems like NetSuite and Coupa, where I can access up-to-the-minute information to stay informed on where we are. For the science, I talk to the professionals in our organization, many of whom are leaders in their field. I also read select publications like Pharmaceutical Executive and a number of blogs, such as the Luke Timmerman Report, Life Sci VC, and Endpoints.

**NANCY DONG, ContraFect Corp.:** In our company, we’re relatively small still, about 30 people in the office. And because I have responsibility for HR, I have become more aware of the staff’s needs. Over the last two years, we have had a Lunch and Learn and invited our staff scientists to talk about what they do. In the past, I would try to pick up the science in the hallways or get myself invited to clinical team meetings or the project team meetings. But the Lunch and Learns have been very successful for everyone.

**RHONDA CHICKO, Scholar Rock:** I consider what the company will need to finance and fund the operations for the long term. We will regularly consider what resources are required to continue progressing the science. I try to look well beyond the current plan. I am always looking to understand our budgeting and cash position, and making sure that the decisions we’re making today will enable us to be viable in 18 months to two years out.

Even for the early stage companies, and perhaps it’s actually more important, to make sure that you bring on the FP&A (financial planning and analysis) skillsets to instill financial responsibility throughout the organization. Because from the all competitive landscape is changing. Our lead target is designed for the treatment of chronic HBV (hepatitis B virus), which is a highly competitive area following the recent success with the treatment of HCV. That said, we are strong believers that the HBV treatment paradigm will be a multi-mechanism approach that will require collaboration working with other companies and their mechanisms.

“It’s not only staying on top of our internal developments with our own science and our own clinical trials, but really continuing to monitor the overall landscape.”

—**JON FREVE, SPRING BANK PHARMACEUTICALS**

Not only do I need to currently monitor our internal progress and clinical developments, but I’m regularly looking at the emerging issues and/or developments in the overall HBV space.

With regards to our lesser-known programs, we’re continuously monitoring organizations that develop targets or focus on indications where we are active. For example, our secondary program, a STING agonist for the potential treatment of certain cancers, is also

**Roundtable Participants**

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Kim Cammarata, Controller, Virtus Pharmaceuticals
Rhonda Chicko, CFO, Scholar Rock, Inc.
Amy Dieder, VP of Finance and CFO, Chiesi USA
Nancy Dong, VP Finance & Administration, ContraFect Corp.
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Lisa Henderson (moderator), Editorial Director, Pharmaceutical Executive
in a very competitive space. There are many companies that have started to enter the clinic, but we have not yet done so. This timing allows us to look for their data to guide us a little bit.

It’s not only staying on top of our internal developments with our own science and our own clinical trials, but really continuing to monitor the overall landscape. That’s important as we make decisions as to where we’re going to look to spend our funds over the course of the next 18 to 24 months.

**PE:** How important is your company’s narrative—your story when you speak to investors, stakeholders, or potential business partners?

**FREVE:** It’s critical. We have a complex story, so it takes a little longer to tell. Sometimes I’d prefer to have a 60-second elevator pitch, but we are a dynamic, young company with a platform of targets, and we have to explain it well—and that’s not as straightforward as you might think. It’s not, “we’ve got one pill that’s going to cure it all.” It’s, “we’ve got to work with multiple mechanisms and collaborate with a few other companies and hopefully we’ll be the backbone therapy in those mixes that eventually move forward and provide a functional cure.” When we’re out on the road—whether it’s non-deal road shows or actively raising funds—that’s how we attract new investors. And if we can’t communicate that story effectively, we’re not going to be able to bring in the additional funds necessary.

Similarly, with existing investors, we have to continue to keep them engaged and show them where their investment is going, which allows us to continue to progress the science. And if we’re not telling that story in an effective way, we’re going to lose certain investors if they’re not happy with either the speed of development or the direction that we’re taking the development plan. Also, if you have delays, be able to explain those delays. Talk about challenges and how you’re overcoming them and moving the programs forward.

The story is critical. I’m on the road quite often and that story evolves regularly. That’s a big part of being able to continue to stay ahead of things; adapt your story to where not only the competitive landscape is going but where your company is going. I think that’s important for all of us as financial executives—being able to tell that story effectively.

**GARBacz:** The company’s story is very important. The investors are interested in the science and the portfolio’s prospects. They are also interested in the company’s cash position and runway, and if the company’s financing strategy can credibly support its narrative.

**AMY DIELBER, Chiesi USA:** The Chiesi story is a very important component of these conversations, because most are focused on collaboration and partnership, where trust is at the center.

Chiesi is a family-owned company, and after more than 80 years, the second and third generations of the family still work here. So, when we’re talking about acquiring assets, or collaborating with a biotech company on its R&D, or becoming its commercial partner, our history of financial stability and commitment to the science are front and center.

The fact that our owners want to see medical innovation advanced to the next level is meaningful. In fact, more often than not, our story is the reason we are chosen as partner.

**DONG:** We have a deck our team uses, but every so often we present it to the entire company so that our team inside knows what we’re saying out there in the market. Our teams on the inside really enjoy them, especially our team in the lab. They used to say, “Oh, the corporate world does a lot of things that we don’t know about. We don’t even know if they know the science.” But now they know and it’s a lot of fun because we have received very particular questions about the science from our internal staff.

**BEN STEIN, Ovid Therapeutics:** Because we’re in the rare orphan disease area, engagement with the patient community is an area that we really strive to be a part of. That provides something to the employees as well.

“If we’re going to develop therapeutic solutions that improve the quality of life and health of people, we’ve got to be financially and operationally healthy as well.”

—AMY DIELBER, CHIESI USA
We try to communicate with the staff as often as we can about what’s going on with the clinical trials and get them involved with the patient community as well. Whether that’s through Facebook, LinkedIn, online, other social media—getting them involved is something that strengthens the engagement and the story.

GARBACZ: I think one of the great advantages of a small company is that everybody is a bit closer to the science. And a lot of the scientists and research people love explaining what they’re doing and what’s going on, almost like a professor. Actually, some of them are professors.

JOHN LANZA, RSM: All of your companies are different. I’ve always wondered, if the founder is the scientist, do they talk differently versus a non-founder scientist? About numbers, cash, runway and spend, and all that?

DIEBLER: Our founder, Giaccomo Chiesi, was a scientist, and the second generation of the family has scientists among them as well. But what’s interesting about Chiesi is that the family has always been visionary about how to achieve their ultimate goal. If we’re going to develop therapeutic solutions that improve the quality of life and health of people, we’ve got to be financially and operationally healthy as well.

As the company grew, Chiesi hired non-family members in senior leadership to provide additional business acumen and balance. They knew they needed to have a CEO and CFO who would pursue the scientific vision from a business point of view.

GARBACZ: What is interesting is that a lot of the security analysts now have PhDs and know the science inside out, so you need someone that is respected and can credibly talk with this community.

CHICKO: We also have a couple different approaches and a couple different levels of our story. One of the things we do is assess to make sure we know our audience. Who are we talking to? Sometimes even before we start, we have a conversation about their backgrounds and ask them about their level of scientific knowledge.

Someone mentioned during the conference that you have to be careful whenever you speak. If you start throwing out medical buzzwords, be careful in case someone calls you on it. Our CEO is excellent at making sure he understands the scientific background for whomever he is speaking with and making sure the person is responding—and, if not, then take a different approach. Having a couple of different pitches works well.

FREVE: What leadership skills do you draw on most in your role?

PE: For me, it’s adaptability. It’s critical. We’re a small company, 25+ employees. I wear many different hats, whether it’s finance, HR, investor relations, IT, facilities. Just recently we built out a new lab space and a new office space where we were moving two locations, consolidating into one; that entailed building out a 10,000 square foot lab facility.

Although the scientists are the most knowledgeable about lab requirements and where they want to build certain aspects of the chemistry lab versus the biology lab versus the NMR (nuclear magnetic resonance) room, etc., it was ultimately me that ended up participating in all the meetings, helping transition everything over to our contractor in working through the build-out.

I learned more about the lab than I ever thought I would, which was great, but I think it’s having the ability to really just put on that different hat and focus on the task at hand and do what needs to get done. That’s critical, especially in a small company world. It’s being able to adapt and work with whatever aspect is coming my way that particular day and take that challenge head on.

GARBACZ: I’ve had the same experience early on in the company—needing somebody that can do just about everything and be comfortable with the uncertainty that comes with biotech. And the organization evolves over time. First, the company is a relatively simple early stage research organization. So you initially focus on...
accounts payable and managing expenses. Next, you may have collaborations or government grants, and then financial reporting becomes more complex because you have to work with your collaboration partner and provide them with timely planning and reporting.

If successful, the company will then progress into clinical trials and the early stage research may become relatively less important. At this point, the company will need to begin hiring clinical and regulatory people that have a different skillset. That changes the culture as well because the company becomes a little more organized and regulatory oriented, and a little bit less academic. Once the company approaches commercialization, you will then need to bring in a salesforce, which is an entirely different group with a new personality.

I think successful senior executives must be able to provide effective leadership across that continuum, adapt to each new phase of the business, and recruit people that embody the company’s core culture.

“Some of the bigger mistakes that companies make is they assume that when work is contracted out, it happens seamlessly. …It’s not easy. It’s a balancing act.”

— RHONDA CHICKO, SCHOLAR ROCK

PE: How much do you outsource to third-party providers and how does that experience go?

FREVE: CROs are our largest source of cost and it’s very difficult to manage the costs. As a smaller player, we don’t have the same leverage as a large pharma. We bid all our CRO work through a competitive proposal process. It’s a challenge to keep them on budget as they’re rarely incentivized for efficiency. It’s definitely an area that will be difficult to improve on as a small player in the space.

CHICKO: It is hard. And we are challenged, as you said, because we’re small companies. We don’t have all the resources and the contractor who works a couple days a week on quality or even in the finance department, can make sense for an early stage company. And for Spero, as we go back to the discussion about how the company evolves over time, our decision to have outsourced a lot of the early stage research, as opposed to bringing it in-house, was beneficial, because we subsequently shifted our focus to clinical development and reduced our need for early stage research.

Rhonda is right, you really need to closely manage third-party providers and stay on top of them.

PE: You all have different pathways to your current role in a smaller biopharma. How do you feel about working in this industry?

GARBACZ: I really enjoy my job and really love working in biotech. Cambridge is like the center of the universe in that respect—there’s so many opportunities and interesting things to do, and great people as well. Everyone that you work with and meet with are intelligent, nice people.

FREVE: Biotech, from my perspective, is by far the most collaborative industry out there. We’re all rooting for each other, because it’s for the right reason.

Even though we may be competitive in a certain space, we want to see others succeed, because the outcome obviously benefits a broader group. From that perspective, it’s exciting to be a part of the entire biotech space.

GARBACZ: You’re doing something that benefits people.

DIEBLER: Our work is important and rewarding. We’re changing lives.
Traditional co-pay programs are no longer an effective strategy for ensuring patient access and adherence to your branded medications. New and evolving payer controls such as accumulator and variable co-pay program (maximizer) designs, combined with higher patient cost-sharing are driving the need for innovation.

The complexity of this new market environment is also driving the need to expand the functional oversight of patient assistance programs to effectively reduce risk and ensure the maximum value of program investments. Designed and implemented successfully, Patient Assistance Centers of Excellence have the capacity to meet the needs of providing patient support in today’s challenging environment.

Join experts from IQVIA’s US Market Access Strategy Consulting and Patient Access & Affordability teams as they examine:

- The challenges to patient support programs and Centers of Excellence
- What can be learned from the common of CoEs that fail
- And the best practices and characteristics of a successful CoE
Securing a Winning Strategy for Real-World Evidence

While the increasing importance of real-world evidence (RWE) is widely acknowledged, the dramatic shift required by pharma to embed meaningful and holistic benefits from this capability is still a work in progress.

By Julian Upton

In Deloitte’s Second Annual Real-World Evidence (RWE) Benchmarking Survey, published earlier this year, the authors highlighted how the proliferation of healthcare data, advancing technology and analytics capabilities, and an increased regulatory/pricing focus on value showed how “the use and importance” of RWE in the life sciences industry had evolved in just 12 months. More specifically, this year’s report pointed to how “RWE initiatives are increasing at the executive level,” not just in regard to generating evidence, but also in supporting other research, corporate, and commercial objectives. Almost all (90%) of the survey’s respondents, Deloitte reported, “have either established or are currently investing in building RWE capability for use across the entire product life cycle,” with 70% building or increasing their internal RWE capabilities. “As a result,” the authors wrote, “RWE spending on talent and technology in the future is anticipated to increase.”

Culture shift

While the increasing use and importance of RWE is widely acknowledged, the shift required by biopharma companies to embed and secure an RWE capability effectively across the organization is still a work in progress. For Qin Ye, associate principal and global RWE lead at ZS Associates, in developing effective strategies, pharma companies still face a big hurdle “to overcome product-development-centric business models and be a lot more connected with the value they’re trying to bring to market and the problem they’re trying to solve for their stakeholders.”

Addressing this, he says, involves a fundamental culture shift. Companies tend to have a need to compartmentalize their functions to gain the necessary focus and efficiency, but that is at the expense of a more holistic approach. While pharma companies need talented teams with specialized training and knowledge, these highly skilled teams tend to base a lot of their decision-making on their experiences in the past. “It can be difficult to change that mindset to one that is more data-driven,” says Ye.

In helping companies implement an effective RWE strategy, ZS sees “the change management of culture shift as the key priority.” A lot of pharma companies currently have a siloed approach to gaining access to and leveraging data. “Looking end to end,” says Ye, “the question should be, how do you leverage data to help you to make decisions and better position your product from the very beginning of the development process?”

This view is echoed by Saama Technologies, a clinical data integration platform company, who this year partnered with Informa Pharma Intelligence’s Citeline to bridge clinical trial information to RWE. Nekzad Shroff, Saama’s VP of field prod-
uct management, told *Pharm Exec*, “The adoption of RWE by pharma leaders is a different problem from developing the actual capabilities involved. Adoption requires a shift to an exploratory mindset. There needs to be a tolerance of ambiguity and imperfect data to be able to actually interpret and gain insights in spite of data gaps.” He says that, with challenges and fears around data quality and security and the constraints of data sharing, “some companies would rather have siloed data than shared data.”

But Shroff believes the situation is slowly being overcome, as pharma organizations are building standards around how real-world data (RWD) is generated as well as interpreted. Similarly, the days when decision-making based on RWE “required an advanced understanding of mathematical statistical techniques as well as computer programming” are also changing slowly. With increasing access to the “democratization” of these data sources and platforms providing actionable insight, Shroff says, companies “are now getting used to relying on these metrics to enhance their decision-making.”

Once that happens, this mindset starts to become pervasive and embedded into day-to-day business processes. And when that culture shift begins, “analytics tools can definitely help,” Shroff explains, “with IT playing a big part in providing a platform with the right set of capabilities, access, and security levels.”

### New (and old) talent

Many large pharma has identified the gaps in their real-world data and evidence capabilities and have the funding to fill them, bringing more data scientists into the talent pool. But, Ye warns, even when a company recruits talented data scientists, it can be difficult to leverage that talent, and equally difficult for the newcomers to gain an understanding of the business process. “It’s a merger of old and new, of two different scientific capabilities,” he says, “and that can be challenging if you don’t focus also on building the process transformation and enabling technologies. Just having new talent come into your team structure in a people-only approach will not solve the problems.”

Karim Damji, Saama Technologies’ senior vice president of marketing, has seen big pharma “running into talent issues.” For companies trying to expand their in-house data-analytics capabilities, there are questions around the talent associated with achieving this the right way, he says. “Pharma companies have been doing it the hard way for a very long time. The tools and technologies that are available to other industries have not been widely adopted by pharma, simply because of a fear of taking that legal risk associated with doing something novel.”

In the meantime, Damji adds, companies like Google, Apple, Facebook, Amazon, and Netflix have been “gobbling up all the talent that pharma needs in terms of expanding internally.” That may be changing, as some firms are making high-profile moves to draw that talent into the pharma realm. Ye welcomes this, as such recruits are used to “thinking outside the box, have lots of experience dealing with a wide variety of data, and transforming how that data can be leveraged.” What’s more, he says, when these “outsiders” bring with them a “very humble mentality to engage with and learn life sciences, it helps to create a more impactful collaboration.” However, says Shroff, for pharma, it is a question of solving the issue “non-traditionally.” Instead of pushing to hire more data scientists, Saama also looks at “the other side of the equation.” That is, “How can we enable the existing employees and executives of companies to start to make sense of some of this complexity without needing an advanced degree in data science?” He adds, “That’s not easy, because sometimes there’s a reluctance to take insights from a system that you don’t understand in and of itself.”

### Data sources

While pharma grapples with these organizational questions, there is also the issue of navigating the myriad—and growing—data sources available for lever-
Real-World Evidence

The industry should avoid simply “rushing in and buying data from the biggest data set in the market,” says Ye. “Companies should start from their business need and their questions. While they do need some baseline data for the area they are focused on, they should have a more comprehensive view of the things they want to transform, and from that they can decide what specific data sources are needed.”

For Damji, amid the myriad data sources, a key challenge for pharma is around longitudinal information and patient centricity. But it’s not just about looking at all the RWD sources and creating an intra-longitudinal view of the patient from a data source. “Creating inter-longitudinal views of the information is a bigger challenge,” Damji says. “And this is where I think modern aspects are being developed outside of pharma. Technology assets, to be precise, will significantly help mine that and stitch it together.” (See graphic above).

Although Shroff points to a current lack of standards covering the use of RWE, he sees more standards being created around the interchangeability and exchangeability of data sets as the space evolves. “Once we standardize how patient records are used for insight generation and how technologies like blockchain can store that kind of data in a secure way, we start to get more consistent around generation, use, and interpretation,” he says. Further FDA involvement will see data sets become much more streamlined, Shroff notes, “as opposed to right now, where companies are looking at anything they can get their hands on.”

As the rise of patient data continues apace, with more wearable devices continuously measuring the attributes that pharma companies are interested in, Shroff believes that companies will start to go straight to patients for an increasing number of data points, rather than going through the traditional trial route. “A lot of pharma’s questions are answerable through observational studies and through custom data sets that they’re able to create. So, there will need to be creativity on how to get to the patients and how to get patients to agree to share their data.”

There is work already being done in this area. Shroff points to California, where, by 2020, patients will be able to consent to have their own data used for a specific purpose without actually going through a third-party consent mechanism. Shroff predicts there will be a consolida-
Five Steps to Developing a Successful RWE Capability

1. Develop a strategy and assess your processes. Before gaining leadership’s buy-in, it’s important to develop a compelling strategy. Start by thinking beyond products, which requires reframing your understanding of your company as a solution company—a value-offering company—rather than an organization that produces a certain number of new drugs per year.

Next, look at the most important components of all, your current product life cycle processes, and identify critical decision-making points. How are teams coming to conclusions, and how are these conclusions being implemented and then evaluated? How can you transform those key decision-making processes into data-driven decisions? Do you have the data and analytical expertise to fill these gaps?

2. Get leadership’s buy-in. A real-world evidence (RWE) transformation isn’t just about generating evidence or adopting new inputs. You need leadership’s buy-in from the start to avoid siloed thinking, duplicative investment, and short-lived success.

A good strategy is to develop an RWE steering committee made up of leadership from each functional area that’s impacted by RWE, as well as technology leaders—and those who join the committee should understand that it won’t be formed for a one-off transformation, but will need to stay in place to encourage change management and continued innovation. The committee can also help further refine your proposed strategy, establish the right key performance indicators (KPIs) and metrics, and help define the organization’s core values and beliefs about RWE.

3. Establish a federated operating model. Most organizations pursue either a siloed or centralized RWE capability. A federated model is a third option that offers the benefits of each while avoiding the pitfalls by balancing centralization with autonomy. It includes a central hub that contains sharable capabilities that support research and business functions that can independently leverage real-world data (RWD) for their use cases. These capabilities provide flexibility to functional groups, which allows them to perform independent analytics using their own tools. The key role of the hub is to enable these functions’ use of RWD and encourage innovation. Data management, data acquisition, and curation are all centralized. Meanwhile, flexible and reusable solutions allow data scientists to efficiently support the many functional areas.

4. Build a global RWD strategy to fuel your RWE needs. Companies need to develop a comprehensive RWD strategy to understand what data resources they already have. From there, they can make investments in the development of new data sources to meet future needs. Many companies only focus on commercially available RWD sources. True competitive advantage comes from long-term investment in developing proprietary data sources.

Consider patient registries and digital devices as well as more strategic arrangements such as RWD partnerships, collaborations, and acquisitions. Also, proactively planning for your evidence needs and investing in data well in advance of these needs is essential to differentiating your RWE capability.

5. Examine your technology capability. RWE models involve sharing data with non-data-savvy business users in a way that’s understandable and useful to them. One example of successful RWE technology is an internal, marketplace-like portal, which allows teams to explore data sources, answer population or patient-level questions, respond quickly to changing regulatory needs and standards, and confirm whether the right data is being leveraged for the right question. Be sure to involve your IT team when evaluating your technology needs for RWE to ensure that you have a scalable platform and the right level of user support.

— Qin Ye and Abhay Jha, ZS Associates

Real-World Evidence

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REFERENCE

Answering Questions in Rare Disease

Founded in 2002, Alnylam is a biopharmaceutical company committed to the translation of transformative medicines based on RNA interference (RNAi) technology. The company’s discovery platform and pipeline of investigational medicines include three programs in late-stage clinical development. With the use of real-world evidence (RWE) key to the development and commercialization of Alnylam’s products, Sonalee Agarwal, head of value and evidence strategy, tells Pharm Exec about RWE’s central role in the organization and its application to the rare and ultra-rare disease space.

**PE:** Can you outline the role of real-world evidence in Alnylam’s activities and how has it evolved over the years?

**SONALEE AGARWAL:** Given that our first three Alnylam-driven products—ONPATTRO (patisiran) for hATTR amyloidosis, givosiran for acute hepatic porphyrias, and lumasiran in primary hyperoxaluria 1—are in rare or ultra-rare diseases, we are currently using RWE to understand the unmet need, the disease burden, and the natural history of the disease, and we’ll be using it to see how the products perform in the real world.

We tend to think about RWE as this new thing, but when I joined Alnylam two-and-a-half years ago, it was already an integral part of the organization. On our website, for example, we have a natural history study in acute hepatic porphyrias; this is an observational study that has patients documenting their experience with the disease, which is real-world data.

RWE has long been at the core of our product strategy, all the way from Phase I, where possible, and certainly from Phase III clinical trials. In recent years, of course, the volume of medical information collected in digital formats has increased tremendously. The evolution of technological and statistical methods have given us a greater confidence in the inferences we make from these data sets. We are not doing causative studies with these data sets, but correlational studies. They give us hypotheses and an understanding of what is happening in the real world.

**PE:** What do you look for in data scientists and analysts at Alnylam?

**AGARWAL:** We look for people who are urgent, passionate, technically strong, and effective communicators. We are a science-based organization and we like to think about hypotheses, so not only do our analysts need to understand the data sets, but also understand the biases within them and effectively communicate what we are controlling for versus what we are not controlling for. In general, having past experience with big data sets is important and can help to establish clarity as we answer research questions.

In my own experience, I worked with a large database, and I gained an understanding, for example, of what is clean data and what is not. I think that experience helps you understand the biases of the data sets and then overcome those biases to answer the right research questions.

**PE:** What challenges remain for Alnylam in analyzing and understanding data?

**AGARWAL:** The biggest challenge in answering research questions in rare diseases is that we need to find sufficient number of patients and the right data sources to answer the critical questions. On the technical and the statistical side, we have the tools for this, and if not, we collaborate with people who do. We use a variety of data sources, including patient surveys, claims data, electronic medical records (EMRs), and observational studies. It can be difficult because claims databases, for example, give us the volume, but not the granularity of data that we need. EMRs give us more of the information we’re looking for, but not the volume. So, it’s not one-size-fits-all. It all depends on the disease, how robust the data is, and the questions you’re trying to answer.

**PE:** How do you see Alnylam’s RWE strategy evolving over the next three to four years?

**AGARWAL:** We’ll be using RWE throughout the product life cycle. Earlier in the life cycle, we use RWE to focus on defining unmet need and the burden of the disease, as well as design effective clinical trials. Post-approval, we will be using RWE data to better understand how the product performs in the real world and to understand the value of these medicines. RWE will continue to be a very important part of our core product strategy.
Measuring the Probability of Pricing and Access Success

Many reports have chronicled the increasingly restrictive market access landscape and pricing pressure in the US and EU, but there is still no structured approach for assessing pricing and access risk. Presented here is a straightforward method for integrating pricing and access risk into portfolio planning and decision-making.

By Jonathan Chee, Betty Pio, Julia Ehrhardt, and Evelyn Siu

In 2014, mergers and acquisitions (M&A) among pharmaceutical and biotechnology companies hit a record-high deal value of ~$219 billion, with ~220 deals completed. Yet in 2016, total deal value dropped to half that number, with $104 billion in total value and 200 agreements completed. With passage of US tax reform legislation, which generated tax savings and lowered barriers to repatriation of overseas cash reserves, 2018 was anticipated to be the return of major deal activity. However, halfway into 2018, M&A activity still looked relatively modest compared to 2014.

This reluctance for major spending is not surprising given that biotech/pharma asset valuation is higher today. From 2013–2015, biotech companies benefited from an IPO boom, with high valuations and soaring stock prices. In 2015 and 2017, venture capital funding and follow-on public offerings reached new highs, providing additional sources of funding. Altogether, biotech companies now have many more profitable alternatives to raise capital than selling or licensing assets, which means companies looking to acquire or license assets can expect to see a higher price tag. With this shifting landscape, portfolio decision-makers require additional tools to identify overvaluation risk.

Key valuation risks are technical and regulatory success. PTRS (probability of technical and regulatory success) is defined as the probability that a new drug will successfully navigate the development stages and receive regulatory approval. This is a crucial factor in determining the potential market size and profitability of a new drug. A high PTRS is associated with higher pricing and access success, as it increases the likelihood of reimbursement. Conversely, a low PTRS can lead to lower pricing and access success, as it decreases the likelihood of reimbursement.

In the next section, we will discuss how to quantify PTRS and integrate it into portfolio planning and decision-making.
latory success) is often used to approximate the potential risks of pre-market factors, ranging from likelihood of asset failure in clinical trials to likelihood of regulatory approval. A composite factor is generated based on the level of risk in areas such as clinical evidence (clinical target validation, drug class, mode of action novelty); trial robustness (including patient population used, selection strategy); and trial design (trial complexity). Notably, the risks according to these factors are highly variable between therapeutic areas.

Ultimately, PTRS can be integrated into an asset’s valuation to understand the impact of this risk on net present value (NPV), and create a more risk-adjusted view of an asset’s worth. How-

<table>
<thead>
<tr>
<th>Country (assessments)</th>
<th>Clear benefit over competitor (all patients)</th>
<th>Subgroup benefit only</th>
<th>Similar benefit to comparator</th>
<th>Less benefit than comparator</th>
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<tbody>
<tr>
<td>UK (NICE)</td>
<td>55%</td>
<td>25%</td>
<td>*</td>
<td>20%</td>
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<tr>
<td>Germany (G-BA)</td>
<td>40%</td>
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<tr>
<td>France (TCI)</td>
<td>35%</td>
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Most likely reimbursement outcome

- Premium to comparator possible
- Premium over comparator in subgroup where benefit achieved / uncertain reimbursement in other patients
- Parity to comparator at best
- No reimbursement / discount to comparator required for access to market

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<tr>
<th>Source:</th>
<th>Based on publicly available assessments from NICE (2000-2018), G-BA (2011-2016), and HAS (2010-2018) and Simon-Kucher analysis.</th>
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<td>*</td>
<td>Data unavailable due to lack of equivalent assessment category, manufacturer decision to withdraw from the market, or only occurs in a small number of assessments</td>
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<td>NICEdesigned from left to right: “Recommended for funding,” “Continued/indications restriction,” and “not recommended for funding.” Recommendations are driven by evaluations of clinical data and cost-effectiveness.</td>
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<td>Source:</td>
<td>Depending on the benefit demonstrated in other patients, product may require a price parity to comparators, price discount to comparator, or may be reimbursed</td>
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Figure 1. Approximately 50% of innovative products assessed in the EU-3 are likely to achieve a premium above comparators in all addressable patients (assessed as showing “clear benefit over comparator”).

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European markets are difficult environments, where payer assessments can dramatically impact pricing and access (see Figure 1). In Europe, new products often face long negotiations to gain reimbursement, significant discounts to US prices, and scrutiny of clinical results. A Simon-Kucher & Partners EU oncology-reimbursement study found that after European Medicines Agency (EMA) marketing approval, products can face up to two years of price negotiations with payers before gaining reimbursement in certain markets.

Through the course of negotiations, new products may require discounts as high as 70% to 85% off the US list price. Additionally, some payer agencies will delive into clinical trial data and restrict reimbursement to subgroups not intended by the manufacturers, further cutting into revenue (see Figure 1 “Subgroup benefit only” column).

Although US payer scrutiny is not at the level of Europe and other markets, payer pressure in the US is increasing as payers consolidate and exert their influence. Currently, the four largest national payers (Anthem Blue Cross and Blue Shield, UnitedHealthcare, Aetna, and Cigna) reportedly cover at least 85% of the US market, vs. 74% in 2006. Payer dominance is even greater on a local level. According to a report by Community Catalyst, citing data from the Kaiser Family Foundation, Blue Cross Blue Shield controls 93% of the commercial health insurance market in Rhode Island, 91% in Alabama, and 89% in Vermont. This gives the payer virtual monopoly power over the health
insurance markets in each of these states. There is no signal that payer consolidation will stop anytime soon. In fact, the recent mergers of four of the largest US payers (CVS/Aetna and Cigna/ESI) indicate that consolidation will likely increase. With this new level of influence, payers request more concessions from manufacturers to gain market access, especially if a new product triggers budget impact concerns (e.g., PCSK9 inhibitors).

Reaching the C-suite
With these shifts in the payer landscape, pricing and market access are now C-level topics. In 2016, when the Tufts Center for the Study of Drug Development released R&D cost estimates, the authors acknowledged “efforts to gather health technology assessment (HTA) information” as a key factor driving costs associated with bringing drugs to market.

The 2017 Global Life Sciences Study by Simon-Kucher found that in the most commercially successful companies (classified based on several key performance indicators, including EBITDA, hit rate of innovations, etc.), the C-suite provides clear guidance and direction on pricing decisions. Additionally, these companies more often have defined pricing roles and responsibilities, and dedicated processes, than other organizations surveyed in the study. Highly profitable companies (defined as EBITDA >20%) are also more likely to consider payers’ decisions and needs in early product development, compared to less profitable companies.

The study also found that involving the pricing and market access team in business development opportunity assessments, such as partnerships and licensing, was of high importance. However, only 32% of companies currently do so. The key reason that most fail to do so is a lack of alignment between senior management and other departments on the importance of pricing and market access, and low awareness of the internal resources and capabilities.

Thus, similar to PTRS, pricing and market access risk should be considered a key step in portfolio decision-making. A lack of pricing power, failure to gain coverage with a payer, or access in hospitals can lead to millions of dollars lost in potential sales for the company (see case study example below). To assess pricing and market access risk, we propose integrating a new metric, PPAS (probability of pricing and access success) into portfolio planning and decision-making.

Pinpoint valuations to assess risk
PPAS is a measurement of the asset’s pricing and market access risk, considering therapeutic area, region, and expected launch timing. Use of PPAS helps to minimize risk of over- or under-valuation, by directly vetting an asset’s likelihood and level of success. PPAS can also help to structure appraisal of assets in markets previously believed to have insurmountable pricing and access risk, but where that risk had never been quantified (only assumed based on anecdotes).

Instead of solely relying on past market experience, PPAS provides a more focused approach to assess the opportunity.

PPAS should be determined as

Case Study: Pricing and Access Challenges Complicate Licensing Deal
A mid-sized pharmaceutical company in-licensed a novel pain medication that was intended for use in the hospital segment. Despite an attractive clinical value proposition, a post-deal assessment concluded that the pricing, access, and uptake barriers the product would face were significant. These included:

a) Low price benchmark. Without a clinical advantage (e.g., proven in head-to-head pivotal trials) over generic morphine, hospitals were reluctant to pay a premium.

b) Appropriate patient population. In cases where hospitals were willing to pay a premium, resulting patient populations were very small.

c) Uptake barriers. The novel delivery device was too large to fit into standard automated dispensing cabinets and thus would need to be stored in the hospital pharmacy, introducing another barrier to uptake vs. generic morphine.

Based on these hurdles, the pharmaceutical company returned the rights of the drug to the originator one year later.
### PPAS Checklist

<table>
<thead>
<tr>
<th>Topic</th>
<th>Performance Criteria</th>
<th>Example answers for a hypothetical rheumatoid arthritis asset (Scale: high risk, moderate risk, or low risk)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Payer area priority</strong></td>
<td>What is the payer perception of unmet need?</td>
<td>Moderate: Many effective treatments but no cure</td>
</tr>
<tr>
<td></td>
<td>What is the budget impact of this therapeutic area?</td>
<td>High: One of the highest budget impact areas</td>
</tr>
<tr>
<td><strong>Competitive environment</strong></td>
<td>What is the level of branded competition?</td>
<td>High: Anti-TNF mainstays and a range of other MOAs</td>
</tr>
<tr>
<td></td>
<td>What is the level of generic competition?</td>
<td>Moderate: Biosimilars exist but do not compete cross-MOA</td>
</tr>
<tr>
<td></td>
<td>What is the level of non-drug competition (e.g., procedures)?</td>
<td>Low: No real non-drug competitors</td>
</tr>
<tr>
<td><strong>Asset differentiation</strong></td>
<td>What is the level of additional clinical benefit from this asset vs. other options (e.g., safety and efficacy)?</td>
<td>TBD: However, new entrants have typically been perceived to be incrementally beneficial</td>
</tr>
<tr>
<td></td>
<td>What is the level of additional non-clinical benefit from this asset vs. other options (e.g., dosing, route of administration, etc.)?</td>
<td>TBD: However, a range of routes of administration and dosing frequencies already exist</td>
</tr>
<tr>
<td><strong>Therapeutic area, region-specific or asset-specific issues</strong></td>
<td>Other situation-specific criteria such as budget impact predictability (e.g., is there medical need for more treatment options, due to finite durability of response or resistance mechanisms?)</td>
<td>Low: RA drugs are used chronically and may decline in efficacy after year one, thus payers recognize the need for more options.</td>
</tr>
<tr>
<td><strong>Price potential</strong></td>
<td>What is the level of attainable price for this asset relative to comparable assets?</td>
<td>Moderate: Due to the competitive nature of RA, premiums are rare</td>
</tr>
<tr>
<td></td>
<td>What is the prevalence of discounting/contracting in this therapeutic area?</td>
<td>High: Significant discounts and contracts are common</td>
</tr>
<tr>
<td><strong>Market access environment</strong></td>
<td>Is there active payer management in this therapeutic area?</td>
<td>High: Payers actively manage the category</td>
</tr>
<tr>
<td></td>
<td>How challenging is the pricing/reimbursement process in this therapeutic area?</td>
<td>High: Expect tough negotiations</td>
</tr>
<tr>
<td><strong>Overall</strong></td>
<td>What is the level of pricing and market access risk for this asset?</td>
<td>High: If you are competing against 1st- or 2nd-line anti-TNFs; Moderate if you are competing for a post-TNF process</td>
</tr>
</tbody>
</table>

Table 1. The six criteria essential in determining pricing and market access success.

PPAS is determined based on how the asset is expected to perform in the six criteria. These criteria are dependent on the market landscape (competitive environment and market access environment), the asset itself (disease area payer priority and level of differentiation), the asset’s price potential, and an overall assessment, including miscellaneous factors such as internal resources and therapeutic area expertise. Within these criteria are more specific factors, such as the relevant patient population size and the magnitude of improvements in efficacy and safety used to generate the overall rating.

Each of these questions can be answered with a response of high, medium, or low. Finally, the ratings from the individual questions are then aggregated into an overall performance rating, with a high risk assessment indicating many expected hurdles and a low risk assessment indicating less expected barriers for an asset.

In a prior Simon-Kucher analysis of existing products in a particular therapeutic area, over 200 data points were gathered by surveying the formulary coverage of 16 hospital analogs across 15 hospital systems in the US. We saw that products that scored “low risk” on PPAS overall had a 70% chance of maintaining a price premium with formulary access, products that scored “moderate risk” had a 50% chance of maintaining price parity or a premium with formulary access, and products that scored “high risk” overall had only a 20% chance of formulary access (e.g., 80% chance of non-coverage). (See Figure 2 on facing page). The same analysis conducted for analogs in the EU yielded similar results, although a score considered moderate in the US may be considered low in the EU due to different definitions of acceptable risk.

**Integrating PPAS into diligence**

PPAS can be integrated into diligence based on a company’s internal processes. For example, PPAS can be integrated into an asset’s revenue forecast in two ways: 1) Reflect pricing risk through a weighted average gross-to-net calculation, or 2) Risk adjust the addressable patient population. Additionally,
PPAS can also be used to facilitate focused discussions between the portfolio decision-makers and pricing and access teams.

1) In the first method, once a PPAS score is generated, the internal pricing and access team can be consulted to determine how pricing risk should be accounted for. A risk to pricing can be expressed in the forecast in different ways, such as a lower launch price, increased discounts or rebates, or extremely limited price increases in the US. The pricing team should provide input on how it believes the level of pricing risk will translate to an impact on price. Based on the input, the price and gross-to-net assumptions used in the forecast should be modified accordingly.

2) In the second method, the pricing and market access team can be consulted to determine how access risk should be accounted for. A risk to access can be accounted for with varying levels of complexity, such as a smaller overall patient population, or a smaller first-line population but a larger second-line population. The market access team should provide input on how it believes the PPAS score will translate to an expected patient population size. Based on the input, the addressable patient population size assumptions used in the forecast should be modified accordingly.

PPAS can also be used to spur collaboration between the portfolio decision-makers and pricing and access teams. Internal pricing and access teams can be leveraged as internal experts in how to best proceed with the asset and what steps can be taken to reduce PPAS risk, particularly if existing market access competencies are expected to be able to overcome hurdles. Pre- and post-launch planning strategies could help mitigate pricing and access risk, without the need to adapt forecasts. Internal pricing and access teams would be in the best position to determine if there are strategies that would feasibly protect pricing and access integrity.

Moving forward
The importance of pricing and market access will continue to grow as payers scrutinize new products. Integrating PPAS early into an asset’s valuation is a key step to ensuring that value is captured as comprehensively as possible. It also has the added benefit of highlighting the additional work required (asset- or market-related initiatives) to create a more positive pricing and access environment for the product. Investing slightly more time upfront will result in a fuller picture of an asset’s likelihood of success in an increasingly strict payer marketplace.

Take a look at the checklist and think about a recent acquisition or licensing deal that you or your company completed. Based on the asset’s performance in the criteria listed, would thinking about these pricing and market access factors be helpful to you when considering a deal?

REFERENCES

JONATHAN CHEE is a consultant; BETTYPIO is a partner; JULIA EHRHARDT is a director; EVELYN SIU is a consultant; all with Simon-Kucher & Partners
Lessons for Pharma from the Merck Cyber Attack

Almost a year and a half later, key understandings have emerged to help companies better combat future data-breach attempts.

If you had to make a list of some of the most pressing issues that we’re facing as a society, cybersecurity would undoubtedly be right at the top. Cybersecurity is a critical and closely watched issue for pharmaceutical businesses in particular, for a number of reasons.

In 2017, a study conducted by Ponemon Institute revealed that about 54% of companies experienced one or more successful attacks that compromised data and/or their larger IT infrastructure at some point in the year. A massive 77% of those attacks utilized file-less techniques—meaning that instead of tricking someone into downloading and installing a virus, the attacks were executed using vulnerabilities that were already there.

According to another study conducted by Deloitte, the pharmaceutical industry is regularly the number one target of cyber criminals around the world—particularly when it comes to stealing intellectual property (IP). In the UK, for example, damages from IP theft totaled 9.2 billion GBP during 2017. A significant 1.8 billion of that was attributed to pharmaceutical, biotechnology, and healthcare organizations.

One of the biggest such attacks in recent memory struck Merck & Co. All told, the company employs more than 69,000 people and reportedly had an operating income of about $6.52 billion in 2017 alone. If this type of attack can hit a company as large and as old as Merck, it can happen to anyone—which is why learning from situations like these is always of paramount importance.

What actually happened?

In June 2017, word first broke that Merck was just one of dozens of businesses that were hit with a massive ransomware attack that ultimately ended up affecting organizations all over the world. On the morning of June 27, Merck employees arrived in the company’s offices across the globe to find a ransomware message on their computers. There was not a single location within the company that managed to get by unscathed, according to published reports at the time.

When the incident was said and done, the pharma giant suffered a total worldwide disruption of its operations, forcing a halt on the production of new drugs, which ultimately impacted the company’s revenue for the year.

Merck, of course, wasn’t the only entity affected by the cyber attack, which reportedly began in Ukraine, then spread quickly through corporate networks of multinationals with operations or suppliers in Eastern Europe. Nevertheless, according to published reports four months later, it was estimated that insurers could pay out as much as $275 million to cover the insured portion of Merck’s loss from the ransomware attack.

What have we learned?

To the industry’s credit, organizations do seem to have learned a great deal from the Merck incident—as evidenced by another high-profile intrusion attempt in July 2018, this time against North Carolina-based LabCorp.

Fortunately, LabCorp officials were able to detect suspicious activity almost immediately—far sooner than the 206-day average. The medical testing company took 50 minutes to contain the damage, thus mitigating the major ramifications moving forward.

During that 50-minute window, some 7,000 LabCorp computers were affected—along with other resources, such as 300 production servers. The company says that it had 90% of those assets back online seven days after the attack.

LabCorp had a detailed response plan that it was able to act on after the attack began. This helped the company contain and minimize the impact of the breach, and its own CEO cites this level of preparation as a big part of what saved the organization. As a preemptive measure, it also instantly shut down certain strategic services in an effort to protect the confidentiality of its data.

All told, what happened in the aftermath of LabCorp’s attack looked far different than what immediately followed Merck’s. But how does a biopharma or life sciences organization make sure that its own cybersecurity situation can be contained?
with hopefully limited fallout? That, of course, requires one to keep a few key things in mind.

**What do we do moving forward?**

In an effort to help mitigate risk from these types of cyber attacks in the future, pharmaceutical companies need to be willing to learn from each other’s mistakes and respond accordingly. This isn’t something that affects one organization more than others based on size or location—this type of data breach can hit any company at any time, and, collectively, everyone needs to be ready.

Organizations must also be accountable, too. A company’s cybersecurity posture cannot singularly be dependent on an IT department. All employees and key stakeholders must take the situation equally seriously and they must engage in cybersecurity best practices every day to help the organization as a whole avoid these types of incidents in the future.

First, it’s important to understand the industry-specific consequences that such a breach might entail. As the Merck case showed, a total disruption of an entire business is likely if you become the target of this type of significant breach—but that’s not the end of the story. Addi-
With a reputation as a reliable, if somewhat unspectacular, marketplace, Malaysia has long appealed to life science investors lured in by the prospect of generating consistent returns. “The country has enjoyed a stable business environment for a number of decades; its institutions are well-established and have proven to be adequately resilient while, historically, business and the government have managed to forge a highly open, amicable and fruitful relationship,” observes Siobhan Das, executive director of the American Malaysian Chamber of Commerce.

Indeed, few can deny the strong underlying credentials of a country that ranked 24th in the World Bank’s ease of doing business this year, comes third within the ASEAN region for GDP per capita, and possesses a fully functional IP framework. “Malaysia has a straightforward operating environment whereby, post-registration of a therapy, there are few entry barriers as long as physicians are convinced of the efficacy of your drugs and patients are ready to pay for them,” confides Wong Kin Sang, country manager of Lundbeck. “It is precisely for this reason that, despite its population size of a mere 32 million people, Malaysia is one of our best performing affiliates within Southeast Asia,” he adds.

Right now, however, the former British colony can be said to be especially alluring. For one, the overall value of its life sciences sector continues to soar: AffinHwang Capital, for instance, estimates that pharma sales have been expanding steadily at a ten-year CAGR of eight to ten percent, reaching as much as USD 2.2 billion in 2017. Business Monitor International meanwhile reports the local medical device segment as also continuing to thrive and forecasts a CAGR of 9.7 percent up to 2021.
Moreover, in the wake of the shock electoral victory of Pakatan Harapan (PH) in May after some 61 years of uninterrupted rule by a single party, the life sciences space has been receiving renewed political focus. Healthcare is now officially designated as one of the National Key Economic Areas under the Economic Transformation Programme (ETP) that strives to steer Malaysia to high-income status by 2020.

“Right now, Malaysia offers the best value for money among its neighbors to an investor looking to enter the region. Not only is it quick and easy to set up a limited liability company, but the infrastructure, tax breaks, talent pool and political backing are all in place for making R&D-related capital investments,” ventures Roberto Benetello, CEO of the EU-Malaysia Chamber.

Certainly, an increasing number of international pharma firms appear to be eyeing Malaysia up as a decent destination to situate their regional hubs including for non-manufacturing activities such as R&D and business processing operations.

One example has been the establishment of the Roche APAC Shared Service Center (SSC). “Malaysia stands as one of only three such operations worldwide and provides finance, procurement, and IT services to 15 countries across the APAC region. Today, it employs some 350 personnel and we actually have ongoing plans to expand out its services to support additional countries,” explains the company’s general manager, Lance Duan.

Novartis, meanwhile, appears to have pursued a very similar logic. “We identified great potential to harness the availability of diverse talent and strong market forces so set about establishing Kuala Lumpur as one of the five Novartis Global Service Centers,” recounts Sandoz’s country head Fabio Sperandei.

CULTIVATING A MANUFACTURING BASE

At the same time, Malaysia is starting to witness the maturity of a local manufacturing base and the simultaneous take-off of a fledgling pharmaceuticals export industry. Aside from a handful of notable exceptions – such as Ranbaxy, Biocon and SM Pharma – the bulk of pharma manufacturing in Malaysia has always been conducted by indigenous companies, with MNCs instead preferring to import their products or harness the services of local contract manufacturers like Xepa-Soul Pattinson.

“We worked for more than ten years with Taisho Pharmaceuticals, the number one OTC company in Japan, and helped them enter Malaysia by developing and producing their cough syrup products. We have also acted as Sanofi’s approved GMP site since 2016 and have been developing eye drop products for SNEC since 2014, all of which has furnished us with valuable expertise,” explains the company’s executive director, Ch’ng Kien Peng.

Nowadays, having learned from these partnerships, companies like Xepa-Soul Pattinson are beginning to ramp up their own export strategies. “Already we are managing to source some 25 percent of our revenues from export activity courtesy of a strong presence in Myanmar and are now in the process of entering Vietnam as well. We want to keep expanding and growing in South-East Asia but will simultaneously be seeking to deepen our presence in African countries,” asserts Peng. “We aspire to place ourselves on the world map and to realize that goal we have to expand our solid base production capacity and secure the requisite accreditations,” he adds.
Many industry insiders credit the National Pharmaceutical Regulatory Agency (NPRA) with raising the standards of Malaysian manufacturing and rendering locally produced pharmaceuticals fit-for-export. “The NPRA has been upgrading its regulatory and compliance standards in line with international norms with the unequivocal aim of rendering the Malaysian pharmaceutical industry as a leader in the regulatory field within ASEAN. This has been useful to our members because it furnishes their exports with a competitive advantage,” muses Billy Urudra, president of the Malaysian Organization of Pharmaceutical Industries (MOPI).

“Another big advantage has been that Malaysia is now a member of the Pharmaceutical Inspection Co-operation Scheme (PICS),” notes Javed Ghulam Mohammad, CEO of AJ Research & Pharma. “This is highly beneficial for companies looking to later distribute their products in any regulated market around the world, because the manufacturing processes will have already gone through very stringent guidelines.”

Moreover, the regulator is also more stringent in its testing of local as opposed to foreign companies,” reflects Saravanan G., managing director of Biocare Group. “While MNCs get audited every five years, Malaysian companies are audited every year so there is absolutely no compromise made on quality. This ultimately provides greater opportunities for local companies to export to the 54 PICS member countries,” he reasons.

“Malaysia relies disproportionately on pharmaceutical imports with almost 60 of the therapies consumed coming through that channel. We are therefore doing all we can to support the local pharmaceutical industry and, in tandem, render Malaysia more self-sustainable,” affirms NPRA director, Dr. Ramli Zainal. “It is testament to these efforts that Malaysia can lay claim to being one of the very first ASEAN markets to gain the PICS qualification and we are therefore a benchmark and role model for others,” he declares.

A SURGE IN GENERICS & BIOSIMILARS

One slice of the local market that has been registering particularly strong growth is generics. “Only a decade ago, innovator drug developers used to possess a strong foothold with originator products accounting for over 80 percent of market supply, but nowadays generic penetration is reaching 45 to 50 percent and this is only set to rise further as other top brands reach the patent cliff,” reasons MOPI’s Billy Urudra.

The segment remains, nonetheless, a crowded field where competition is notoriously stiff. “Many more companies now understand the merits of the Malaysian market and are angling for a piece of the action and therefore it pays off to be able to deliver up a differentiated offering,” counsels Sandoz’s Sperandei. “The main risk for a generics player is to enter a large number of therapeutic areas without any real focus. We have therefore made it our strategy to prioritize three to four therapeutic areas where we know we can be dominant,” he adds.

Local actors, meanwhile, have had to come up with their own distinctive pathways. “We are not able to compete head to head with large generics players from India or China that possess a heavyweight domestic market to fall back on,” frankly acknowledges David Ho, managing director of Hovid. “Our strategy has thus been to bring innovations in niche segments. We focused on developing different controlled release delivery systems and combination products,” he elaborates.

Interestingly, discernable momentum can equally be witnessed in the biosimilars field. “The Malaysian authorities are particularly keen to encourage and incentivize investment into biosimilars and any firm entering this market is being welcomed with open arms... already we have introduced two such items into the local market: namely the oncology product Filgrastim, and Epoetin Alfa, used by nephrologists and dialysis centers,” recounts Sperandei. Similarly, last year, the
Indian outfit, Biocon, was awarded a three-year contract by the Ministry of Health to supply recombinant human insulin (rh-Insulin) formulations manufactured at its biopharmaceutical facility in Johor.

Indigenous firms have also been quick to clamber aboard the biosimilars bandwagon. Korea’s Alteogen, this year, signed a memorandum of understanding with the Malaysian state-funded corporation, Inno Bio Ventures, to establish a joint venture for the development, clinical research, production and marketing of biosimilar drugs. “The idea is partly for us to utilize our connections to the Muslim world. The South Korean entity is seeking to deploy Malaysia as a hub to penetrate Middle Eastern markets,” elaborates Tan Sri Rahman Mamat, chairman of Inno Bio Ventures.

When it comes to the healthcare sphere, Malaysia certainly seems to have scored some memorable wins. “Notwithstanding the ongoing challenges that we face, the performance and quality of healthcare in Malaysia remains robust; we have recently become the first western pacific state to eliminate what is known as mother-child transmission of HIV and syphilis, the culmination of work that is a decade in the making,” enthuses Minister of Health, Dr. Haji Dzulkefly bin Ahmad.

What’s more the country has managed to assert itself as a prominent leader in health tourism. Malaysia began developing the healthcare travel scene in the early 2000s. Seeing its potential to contribute to the nation’s GDP, the government set...
Malaysia has great potential as a clinical trials destination within Southeast Asia. As Dr. Akhmal Yusof, CEO of Clinical Research Malaysia (CRM) – the organization tasked by the Malaysian Ministry of Health with increasing the level of clinical research in the country – notes, "We have a population of 32 million which represents a third of the world’s genomics."

Dr. Goh Pik Pin, director of Malaysia’s Clinical Research Center (CRC) adds, “We have a wide demographic and genetic pool, which is important for the testing of new drugs which may have different effects on different genetic makeups. In this regard, clinical trials conducted with the multi-ethnic Malaysian population can root out different responses to different drugs.”

Dr. Yusof also highlights the fact that “Kuala Lumpur is home to the biggest hospital in Southeast Asia – Hospital Kuala Lumpur – and the country has several hospitals of a similar size. Hence, there is a huge pool of patients within a diverse range of therapeutic areas.”

Dr. Yusof continues, “Moreover, we have a good command of English so there is no need for documents to be translated. Also, the common diseases in Malaysia are similar to those seen in western countries. Finally, we work very hard in maintaining timelines. The timeline to review clinical research (Regulatory Authority and Medical Research Ethics Committee) in the country has been reduced tremendously ... Malaysia is now one of the most efficient places in the region to conduct research.”

Despite these appealing factors, Dr. Yusof feels that Malaysia does not yet have the reputation it deserves as a clinical trials destination. "Malaysia lacks exposure on the world stage for its involvement in early stage research, as the number of trials we conduct is still small. Therefore, we are working with centers in Malaysia to develop their capacity for phase I and pre-clinical research ... In conjunction with this, we are ensuring that our researchers have the appropriate training to conduct early stage research. We are sending medical professionals on scholarships to train in centers that have conducted a significant amount of early phase research such as King’s College London and The Christie in Manchester, UK.”

Dr. Yusof concludes, “Taiwan and Hong Kong are currently conducting three to four times more sponsored clinical research per capita than Malaysia. Therefore, we have significant scope for growth!”

A Clinical Trials Hub for the Region?

Dr. Akhmal Yusof, CEO, CRM

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Find out how the Government of Malaysia supports the clinical research industry through Clinical Research Malaysia.

www.clinicalresearch.my
Southeast Asia’s First Medical Device Refurbishment Park

One hidden cost of the healthcare value chain is the refurbishment and repair of medical devices which can no longer be used effectively. With some 60,000 pieces of medical equipment currently in disrepair in Malaysia, a plan to invest MYR 400 million (USD 95 million) in a medical device refurbishment and innovation park – the ASEAN Medtech Innovation Park (AMTOP) – was put forward in 2016, with construction slated to be completed within 2018.

With funding from the investment arm of Yayasan Bina Upaya Darul Ridzuan (YBUDR) – a non-profit foundation set up to assist the Malaysian government to address poverty issues – the project will be jointly developed by YBUDR and the privately-held healthcare group, Chulia Life Sciences.

Assessing the potentially wide-ranging impact of the park – the first in the region – on the Malaysian healthcare industry, Chulia’s Jamaludin Elis asserts that “AMTOP will be the first medical device refurbishment and innovation park in the region covering four main areas: medical equipment upgrading and repair, education and skills training, research and development, conformance testing, prototyping and manufacturing. The park will address the gaps in the medical device industry and help save costs to reduce rising healthcare expenses in Malaysia."

Elis feels that AMTOP also has the ability to bolster the economy of Malaysia as well as those of its regional neighbours. He elaborates, “This park will be a complete ecosystem of medical technology and innovation in ASEAN which can be replicated across the region. In the future, we want to expand into other markets, creating specializations unique to each location. For example, Thailand could be the center for X-ray machines and Vietnam for ultrasound technology with the Malaysian park acting as central hub. This can be a platform to exchange knowledge and experiences which will create a strong base of collaboration across ASEAN.”

However, some systemic problems lie bubbling beneath the surface. Firstly, there is recognition that the country has been skimping on health expenditure and that there is a lot of catching up to do for the public health apparatus to become properly capitalized. “As an upper middle-income economy, we have simply underspent on healthcare. While the average spending on healthcare for an economy of our size is around six to seven percent of GDP, we are only spending 4.5 percent on healthcare” candidly admits Minister Dzulkefly bin Ahmad. “Consequently, I am determined to oversee an incremental increase of budgetary allocation towards healthcare over the next five years in this parliamentary session so that we can address pressing infrastructure issues, such as aging facilities and equipment,” he pledges, noting that of Malaysia’s 145 public hospitals, 45 are over 100 years old!

Secondly, the country’s transition towards a high-income status is adversely impacting Malaysia’s epidemiological profile. In recent decades, Malaysia’s population has witnessed a dramatic surge of non-communicable diseases (NCDs) due to the prevalence of unhealthy diets and lifestyles. NCDs currently account for 70 percent of all deaths in the country and, in 2017, the country had the ignominy of having the highest rate of diabetes in the Asia-Pacific, with almost one-fifth of its population living with the disease. “The incidence of diabetes has increased from 6.6 to 17.5 percent of the population. Hypertension is now at around 30 percent, and 45 percent of the population is now overweight. Half of those with diabetes are undiagnosed and, amongst those diagnosed, 50 percent are poorly controlling the condition,” laments Dr Noor Hisham bin Abdullah, director general at the Ministry of Health.

Naturally this is, in turn, placing great strain on the finances of the social security apparatus known as PERKESO. “NCDs are jeopardizing our financial viability; we receive around 14,000 new claims for invalidity pension or survivors’ pension due to NCDs each year and this now accounts for some 50 percent of all invalidity claims,” reveals the social security system’s CEO, Mohammed Azman.
Unfortunately the existing toolbox for competently dealing with these problems is inadequate. “The snag at the moment is that the treatment of NCDs is based on a comparatively expensive curative and therapeutic approach with no guarantee of success. Healthcare professionals are treating diseases without necessarily looking at preventive measures. Therefore, the government has to focus on investing more resources into prevention – the only way to change stakeholders’ mindsets and ultimately reverse the situation,” analyses Dr. Azrul Mohd Khalib, chief executive of the Galen Center for Health and Social Policy.

Nor are all potential options being explored. “The time is ripe to start looking externally for inspiration and begin coopting the private sector through proper PPPs. The government needs to recognize private enterprise as a solution which can drive healthcare forward and alleviate the burden, rather than merely viewing it as a commercial entity,” opines Chulia’s Jamaludin Elis.

**GREAT EXPECTATIONS**

Many will, no doubt, be vesting their hopes in the surprise return to power of Mahathir Mohamad, a nonagenarian former 5-term prime minister credited with liberalizing healthcare in the 1980s, who now spearheads a populist coalition whose unorthodox policies include, among others, monopoly busting in life sciences procurement, free medical insurance for the poor, and lavish spending on public healthcare.

“The newly elected government has seemingly embarked upon a decent reform trajectory by announcing their intention to double the budget allocation for healthcare, but we are still in the dark about how they actually intend to reach this unprecedented target,” frets Kheng Huat Ewe, executive director of PhAMA, the Malaysian association for innovative companies.

“The new regime has been voted in for their progressive thinking and it’s the very first time that such a laudable program dedicated to supporting the health needs of the poor has been mooted, but, to date, no concrete financial plan has been established to buttress this initiative,” agrees Antah’s Tunku Naquiyuddin.
As the Malaysian pharmaceutical industry expands and develops greater links with its Southeast Asian neighbors, a significant need has emerged for a space where key stakeholders can meet, interact and promote their business offerings.

One organization aiming to fulfill this role is the Malaysia International Trade and Exhibition Center (MITEC), established in 2017, which is branding itself as ‘The Venue of Choice for Medical Exhibitions.’

CEO Günther Beissel explains that MITEC is “the largest trade and exhibition venue in Malaysia with 45,000 sqm of space. MITEC is strategically located less than 7.5 km away from Kuala Lumpur city center, less than 65 km from the Kuala Lumpur International Airport and is being positioned as an economic catalyst and gateway to Southeast Asia.”

Beissel is keen to highlight MITEC’s strong governmental ties and its role in helping achieve national goals. He points out that, “MITEC is owned by the Malaysia External Trade Development Corporation (MATRADE), a national trade promotion agency under the Ministry of International Trade and Industry (MITI). This synergy brings many advantages to the venue … our ultimate vision is to see Malaysia becoming the leading Meetings, Incentives, Conferences and Exhibitions (MICE) destination in Southeast Asia.”

Having already played host to two major medical events in its short history – the Malaysia Medical Device Expo 2018 and the Malaysian International Scientific Congress of Obstetrics and Gynecology 2018 – Beissel feels that MITEC can continue to capitalize on the dynamic Malaysian and Southeast Asian healthcare industries. He concludes, “MITEC is a game changer in the business events industry, giving Malaysia the capability to compete and meet demand in emerging markets, including for regional and international exhibitions, particularly for ‘mega exhibitions,’ which attract over 100,000 visitors.”
Gaining the level of visibility one would need to adequately secure these resources is an ongoing and reactive process that requires the coordination of a company’s vendors, operational methodologies, and culture and sensitive data during an intrusion attempt. This includes elements like clinical data, IP, formulas for compounds, and, in some cases, patient or employee personal data. The amount of money that a hacker can get for a stolen proprietary formula on the black market significantly eclipses what they might be able to get for something like stolen credit card information. One study from the Security Strategy Risk & Compliance Division at IBM, for example, revealed that a stolen electronic medical record (EMR) by itself can be sold for up to $350 on the dark web.

With 3.15 million records being exposed across 142 industry breaches in Q2 of 2018 alone, according to data cited by Health IT Security, a network of Xtelligent Healthcare Media, one can quickly see how it can add up. The amount of money that people can make using health information to blackmail individuals is even higher. Therefore, it’s far more likely that hackers will target industries that yield bigger payouts than they would get by going after a private citizen via identity theft, for example.

It’s also important for drug manufacturers to apply learnings from past cases in the industry, all of which involved systems, partners, contractors, and subcontractors. “Pharmaceutical businesses in particular need to understand that all of these systems are connected,” says Kenneth Sprague, senior security engineer at Technical Support International (TSI). “If any link in the chain is broken, the entire chain becomes compromised. You need to be on the ball. Yes, security and patching are an ongoing battle, especially when you consider the changing threat environment we’re dealing with. But it’s something you have to do in order to survive.”

One of the issues with big pharma from an IT perspective is that oftentimes organizations are dealing with infrastructures that are a collection of legacy systems, multiple systems that are difficult to properly integrate (and secure), Excel spreadsheets, purpose-built cloud systems, and more. Gaining the level of visibility one would need to adequately secure these resources is an ongoing and reactive process that requires the coordination of a company’s vendors, operational methodologies, and culture. Challenges can arise when IT functions are siloed. Legacy systems, for example, often lack the vendor support needed to update them against the latest threats. That alone can leave an organization exposed, regardless of how large it is.

This is a pressing issue for smaller pharma companies as well. Often, these organizations fail to believe that IT and planning for growth should be an area of immediate focus; in reality, it couldn’t be more important. IT can help empower the growth of an organization if properly built for agility and aligned with long-term goals.

**Think like them**

In the end, the most important thing for pharmaceutical companies, large or small, to understand is that getting hit with this type of cyber attack is no longer a question of “if,” but “when?” A company can invest in all of the cybersecurity measures that it wants—it still won’t prevent it from one day becoming the target of hackers with malicious intentions.

But if an organization knows what someone is after, the good news is that it’s now in a much better position to mount the specific defense needed to protect it. That insight will act as a company’s first line of defense against these types of cyber criminals in the future.
Artificial intelligence (AI) has become ubiquitous in all industries, including the life sciences, and is often billed as the technology needed to forge ahead with innovation. Yet, AI is also attracting some concerns—particularly around job losses, the ethics of AI, and more broadly, how successful it really is. In a recent survey, we found 69% of companies are using AI, machine learning, deep learning, and chatbots, yet only a fifth (21%) of those that adopted AI felt their projects were providing meaningful outcomes.

As the dust settles after the initial rapid adoption of AI, more firms are now viewing their investments objectively, noting that not all results are positive. To ensure AI pays dividends, companies will need to overcome several barriers.

1. **Skills shortage hits AI hard**
One of the biggest issues is a shortage of adequately qualified workers with the right technical skills. Life sciences companies typically don’t find it easy to attract digital “natives”; there is often a pay discrepancy between the science and technology industries, and pharma has not typically been recognized as leading from the front when it comes to digital innovation. More recently, pharma companies have also garnered a reputation for “hire and fire” within the tech community, as more people unfamiliar with the environment join the industry. Upskilling those already in the industry will be a key factor in improving AI, as well as altering job-seekers’ impressions to attract skilled data scientists to roles in life sciences.

2. **Poor data affects outcomes**
Limited access to quality data is also affecting the results AI can currently yield. In AI, the “garbage in, garbage out” concept is critical when building algorithms, and even the most experienced technology companies can get it wrong. For example, in 2016, Microsoft’s AI-driven Twitter chatbot, Tay, went completely rogue when attempting to use language patterns of its 18-24 demographic. Tay was said to have found herself “in the wrong crowd”—and while this example likely didn’t result in physical harm to anyone, it highlights that when AI is making decisions about people’s health, the need for a correct, impartial response is paramount.

3. **Lack of data standards**
As well as a challenge in accessing patient data, there are currently no industry-wide data standards. These standards need to include patient data in the broadest possible sense and from a wide range of sources, including mobile devices, wearables, and more. As a result, significant time and resources are required to integrate data into corporate systems and make it usable. Standardized data formats would tackle this issue but will require much greater collaboration between pharma and biotech organizations and data and technology firms. Currently, there are guidelines that promote data sharing, such as the FAIR principles (Findable, Accessible, Interoperable, Reusable), but these need to be further encouraged to help maximize the usability of data.

4. **Anxiety limits progress**
The progress of AI has also been hindered by anxiety over change, such as the ethics of AI, and employee concerns over potential job losses. But fears of robots taking our jobs are misplaced; AI will augment researchers by helping to tackle repetitive, time-consuming work, allowing them to be more creative and follow different paths to enable fruitful research.

On the other hand, reservations over how “biased” or “unethical” AI might be will need to be addressed. In clinical trials, for example, worries have been expressed that recruitment is not truly representative of demographics. This is a problem given that age, race, sex, genetic factors, other drugs being taken, and more can play a vital role in a person’s response to a drug or intervention. The diversity of clinical trial recruitment must be improved to ensure we are building AI algorithms that will provide the best recommendations for all groups.

A collaborative approach
Overcoming these barriers to progressing AI will require, first and foremost, a shift toward a collaborative mindset within the life sciences industry. It will be essential in ensuring that AI genuinely helps to boost innovation and delivers accurate, unbiased, and ethically derived results.
Celebrating Years of Pioneering, Together.

It isn’t in our nature to seek the limelight or to sing our own praises. But when you turn 50, well, that’s something pretty special. We don’t want to celebrate alone though, because we know the real power comes from pioneering together. To all of those who share our passion for protecting people and are inspired by science and medical discovery, a heart felt thank you for joining us on our first 50 years of pioneering together!

www.wcgirb.com/50
We received more than 100 nominations, and selected 10 people with the most impressive leadership, knowledge, and skills. These up-and-coming professionals are vital to the future of the pharmaceutical and biotech industry. They’ve proven that they have what it takes to deal with challenges that will continue to face manufacturers in the coming years.

**2018 Emerging Pharma Leaders**

**Christopher Boone**  Vice President, Head of Real World Data & Analytics at Pfizer Inc.

**Hong Cheng**  Head of Research Strategy & Effectiveness, and Interim Head of Asia Pacific Research at Sanofi

**Kathryn Corzo**  Vice President, R&D Global Project Head, Isatuximab at Sanofi

**Grey Griesemer**  Senior Vice President, HR & Communications at G&W

**Liz Lewis**  Chief Counsel & Head, Patient Advocacy at Takeda Oncology

**Dr. Shao-Lee Lin**  Executive Vice President, Head of Research & Development and Chief Scientific Officer at Horizon Pharma

**Bernat Olle**  Chief Executive Officer at Vedanta Biosciences

**Nadeem Rehmat**  Chief Operating Officer at PharmEvo Pvt. Ltd

**Raymond Sanchez M.D.**  Senior Vice President at Otsuka Pharmaceutical Development & Commercialization, Inc.

**Harout Semerjian**  Executive Vice President, Chief Commercial Officer at Ipsen

Read more about them: pharmexec.com/EPL2018
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Years of Pioneering, Together.

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