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COVID-19 Ripple Effects for Pharma

THERE IS NO SHORTAGE OF IDEAS on which to write for this month’s column. I’m talking about COVID-19 and what it meant to industry in the month of March, which is coming to an end as I write this. I’m sure most of us had a similar experience ending February and into early March. What we first thought was in our control, was not. And for CEOs and executives answering to the needs of their local, state, or country governments, as well as the well-being of their employees, it has been nothing but crisis management since mid-March.

Let’s take a look at some highlights. As of this writing, biotech and pharma analysts were reporting that the impacts of COVID-19 were still materializing, but maybe more noticeable in some areas vs. others. For example, manufacturing was reportedly stable as most staff were deemed essential personnel. On a smaller R&D scale, one biotech executive told me his lab staff were working in shifts of four around the clock, observing all distance and safety precautions.

The FDA had not lost steam in reviewing applications as of press time. In fact, the agency announced the approval of Bristol-Myers Squibb’s Zeposia (ozanimod), for relapsing forms of MS on March 26, however, it was also reported that the company was going to delay the drug’s launch due to the pandemic. It will determine new launch timing in conjunction with discussions with the neurology community.

For sales representatives, those in the US and globally are working from home and using virtual interaction. This is par for the course for medical professional outreach, however, it’s usually only part of the outreach, not the sole means of communication. In early March, Veeva Systems offered its CRM users a free six-month access to the company’s research labs to analyze samples taken in Indiana healthcare facilities, such as nursing homes and emergency rooms. Response Fund. And Eli Lilly increased its COVID-19 testing capability in its home state, using the company’s research labs to analyze samples taken in Indiana healthcare facilities, such as nursing homes and emergency rooms.

So, what does the future hold for pharma post-COVID?

Speculation that the industry could turn its negative image around and stress the scientific advances it brings to the quality of human life in the background of COVID is not a reach. Will clinical trials truly turn the tide in trial monitoring so that it is more efficient and patient-directed? Will digital tools and platforms begin to be used more freely in organizations by both internal teams and external HCP communications?

As for clinical trials, that area is affected in different levels across the board. Pfizer, Merck, and BMS have temporarily suspended screening and enrollment in some studies. Others, including Vertex, are exploring virtual visits for trial participants. And, in what looks to becoming a rallying cry for implementing decentralized and remote clinical trials and bringing studies into the 21st century, many companies are offering services and software to address clinical trial needs during COVID-19.

Besides the necessity to quickly develop a vaccine, as well as tests, biopharma has stepped up. Merck donated 500,000 personal protective masks to New York City Emergency Management; Horizon Therapeutics, in partnership with Illinois Gov. JB Pritzker, provided $500,000 to the Illinois COVID-19 Response Fund and $500,000 to the Illinois Biotechnology Innovation Organization (iBIO) Institute’s COVID-19 Response Fund. And Eli Lilly increased its COVID-19 testing capability in its home state, using the company’s research labs to analyze samples taken in Indiana healthcare facilities, such as nursing homes and emergency rooms.

Meanwhile, another event, the 5th Annual School of Gastrointestinal Oncology (SOGO) multidisciplinary education conference was converted from a live, in-person event into a virtual meeting*. Companies continue to explore other ways to inform the medical community of scientific data.

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Only time will tell. As much as we all want to get back to life as it was, there may be some things that will never be the same, and some things that could get even better.

*Editor’s Note: This live-to-virtual meeting was produced by our parent company, MJH Life Sciences.
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2011 NEAL AWARD WINNER FOR “BEST COMMENTARY”
2020 HBA WOTY: A Motivated Mission
Elaine Quilici, Senior Editor

Pharm Exec profiles Sandra Horning, this year’s selection as the Healthcare Businesswomen’s Association Woman of the Year. Rooted as a practicing oncologist, researcher, and professor, harnessing the patient experience has been the driving force in Horning’s career-spanning and service-focused mission to fight disease and accelerate the delivery of new medicines.

Workforce Development
Doubling Down on D&I
Julian Upton, European and Online Editor

Recognizing that diversity and inclusion is no longer simply a “nice to have,” pharma has boosted efforts in this area, from hiring policies to media campaigns. But just how far are new industry initiatives moving the needle on D&I?

Access and Reimbursement
Gene Therapy: Payer Perspectives
By Jane F. Barlow, Matt W. Courthey, and Mark Trusheim

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By Janardhan Vellore and Daniel Wetherill
A great article that articulates the facts around current challenges with canceled conferences, but also shows the light at the end of the tunnel. There are indeed great options today, with modern technology, to facilitate the exchange and interaction between peers, even when conferences get canceled.

Anonymous
“Navigating Medical Conferences in the COVID-19 Era”
bit.ly/2yjcsG2
New drug launches face more marketplace scrutiny today than a decade ago. Brand success requires not only communicating the clinical value to providers and patients, but also demonstrating the cost-effectiveness to payers in a real-world setting.

This can be particularly challenging when these three stakeholders have different perceptions of the value of the therapy. To navigate these complex realities, manufacturers must focus on market access—a very broad term that encompasses everything from distribution to formulary status to contracting, across benefit designs and within specialty and traditional drug therapies.

Many times, market access isn’t thought about until the therapy goes to market. This needs to change. It must be discussed as early as possible in a drug’s life cycle, which means during clinical development or before.

Roshawn Blunt, managing director of 1798, a Fingerpaint company, and Andy Pyfer, partner at Fingerpaint, shared insights into the current market access landscape and explained why it’s important to have a clear understanding of the topic.

What are the top two access pain points when it comes to launching a new drug, and how do companies overcome them?

ROSHAWN BLUNT: There are two market access pain points applicable to most, if not all, new drug launches. The first is achieving the broadest payer access at the best possible margin. The manufacturers must establish a price that reflects the drug’s value and achieves shareholders’ expectations. Payers have a variety of tools that can be implemented to control therapy utilization; they employ those tools if the net cost does not reflect the value.

To mitigate the risk of pricing incorrectly, manufacturers must understand the amount of control payers exert on a drug’s market basket—as well as the prescriber specialty’s willingness to navigate those controls to obtain access for patients. A drug could have a strong therapeutic effect, but if providers are unwilling to take the necessary steps to qualify patients for therapy, the value of the drug is lower.

Predicting how payers and providers will behave in a real-world setting ensures that the cost is based on the perceived value—measured not only by providers, but also by payers. This is the first step toward access.

Further, those data allow pharmaceutical manufacturers to develop the gross-to-net analytics needed to calculate the return on investment.

The second is patient affordability. Even patients with insurance might not be able to afford the copays for medication. At a drug’s launch, it’s critical to ensure that a patient’s inability or unwillingness to manage out-of-pocket costs will not affect a physician’s decision to prescribe a certain brand.

Moreover, it’s critical to ensure that cost does not impact a patient’s ability to remain compliant with that brand’s use. Manufacturers must have a clear understanding of patients’ total cost exposure—for both the newly launched medication and any concomitant medications—in order to inform price as well as any investment in copayment assistance and free drug support.

Why is it important for an agency brand team to have a thorough understanding of these pain points?

ANDY PYFER: When you are developing a commercialization plan, you have to deeply understand all of the factors that will impact a therapy’s success in the market, and that includes access issues. An agency can develop the most creative work imaginable, but if there are access barriers faced by the healthcare provider, or a patient can’t go to their pharmacy and get their prescription filled because it’s not covered or not affordable, then we have failed.

Working closely with access experts, like 1798, and grasping the pain points can also help creative teams develop materials. We can develop solutions to help move the conversations by being aware of those pain points and leveraging them. In some cases, it might mean addressing them head on in marketing materials or developing resources that move the needle in the access discussion.

By working together and creating a comprehensive and cohesive commercialization plan, it can ensure success for all stakeholders.
COVID-19 Pandemic Thrusts Biopharma R&D to Forefront

Emergency funds for research, public health, and economic disaster raise access issues

The rush to develop viable therapies and vaccines to combat the spread of the novel coronavirus outbreak continues to focus public attention on the importance of access to vital medicines and diagnostics. FDA and health agencies struggled last month to expand COVID-19 testing after weeks of delay, while Washington policymakers approved measures to fund biomedical research and to support free coronavirus testing plus a safety net for families and workers hurt by closed schools and job losses. Congress also sought to allay spreading fears of an economic recession with a trillion-dollar emergency coronavirus aid package to send financial assistance to hard-hit companies and cash payments to individuals.

Meanwhile, dozens of biopharmaceutical companies outlined plans for developing effective treatments, as marketers grounded sales forces and halted travel to avoid in-person interactions. The industry also sought to address fears about biopharma organizations potentially making millions from the healthcare crisis and the need to ensure access to critical new treatments.

The pandemic provides the life sciences industry with an historic opportunity to demonstrate how its extensive R&D resources, rapid screening techniques, manufacturing capacity, and willingness to share research findings and clinical trial data can help resolve this global health crisis.

Advancing research

This imperative to halt the COVID-19 outbreak has spurred public and private labs to action. Scientists have identified some 50 existing drugs that may help treat infected individuals, and results from early efficacy trials of repurposed agents were expected to begin rolling out this month.

These projects include clinical studies on antivirals, malaria drugs, vasodilators, corticosteroid agents, immune modulators, and some cell therapies.

A viable vaccine is predicted to take more than a year to emerge, even with accelerated development platforms and advanced production methods. In the middle of last month, the National Institute of Allergy and Infectious Diseases (NIAID) announced the launch of a Phase I trial in record time to evaluate the safety and potential benefits of a much-discussed Moderna vaccine candidate, which has already been studied for SARS and MERS. Pfizer and BioNTech anticipate clinical trials this month for an mRNA vaccine. Johnson & Johnson’s Janssen unit has teamed up with the US Biomedical Advanced Research and Development Authority (BARDA) to evaluate its Ebola vaccine candidate, with hopes of launching clinical studies in November.

Meanwhile, German officials blasted alleged efforts by the Trump administration to shift to the US a vaccine development program by the German biotech firm CureVac, ostensibly to ensure that Americans get first access to any resulting coronavirus vaccine. All sides denied the charges, but the dispute indicates ongoing concerns at home about shortages of vital therapies made overseas.

Continuing cost debate

This reliance on foreign-made medicines has fed fears about drug supply reliability as well as product costs. In the March debate between the two remaining Democratic presidential hopefuls, Sen. Bernie Sanders charged drug companies with looking to profit from the pandemic, as part of his continuing call for a government-run single-payer healthcare system and drug price controls.

Such statements reflect fears that companies developing new treatments for COVID-19 would reap excessive profits, as emerged in the debate in Congress over the initial $8.3 billion emergency coronavirus funding package enacted March 5. Democrats initially sought language to ensure that the government doesn’t overpay for new pandemic treatments and to allow the feds to take over a company’s intellectual property on a vaccine or therapy deemed too expensive.

Pharma organizations worked with Republicans to eliminate such constraints, claiming a threat to industry R&D investment. Although the final legislation calls for HHS to ensure that new vaccines, drugs, or diagnostics developed from added public funds are “affordable in the commercial market,” it also states that HHS can’t delay the development of new therapies in order to “maintain affordable prices.”

JILL WECHSLER is Pharmaceutical Executive’s Washington Correspondent. She can be reached at jillwechsler7@gmail.com
The measure further provides substantial support for new R&D—some $3 billion for developing new vaccines and therapies and $2.2 billion to help expand state and local testing and infection control programs. FDA gains $61 million to support speedy review of new therapies and to deal with shortages due to supply disruptions from China and elsewhere, while NIAID gains $826 million to develop coronavirus vaccines, treatments, and tests. The measure also subsidizes loans to small businesses and supports international efforts to battle the spread of COVID-19 overseas.

HHS also addressed industry liability concerns, which were dropped from that initial bill, by issuing a declaration granting manufacturers protection from liability for medical and security countermeasures against COVID-19. The protection applies to FDA-approved drugs, biologics, and medical devices used to diagnose, prevent, treat, or mitigate a pandemic disease or any serious condition linked to new countermeasures.

Meanwhile, drug costs have remained on the political radar screen. Before the severity of the pandemic fully emerged last month, the Trump administration offered another drug cost-cutting strategy for limiting what Medicare beneficiaries pay for insulin. A voluntary demonstration program for Medicare Part D drug plans would provide insulin products at $35 a month to some 3.3 million diabetic seniors, a big drop from over $400 a month average for such supplies. Sanofi, Eli Lilly, and Novo Nordisk indicated willingness to participate in the demo, while patient advocates complained it doesn’t go far enough: it applies only to more expensive, and not basic, Part D plans and doesn’t actually cut drug prices, but lets the Centers for Medicare & Medicaid Services (CMS) waive certain restrictions so that plan sponsors can negotiate rates with manufacturers and reduce copayments—strategies that critics say merely bolster list prices.

Leaders of the Senate Finance Committee also look to the pandemic to spur Congressional action on its drug pricing legislation, which was recently revised to boost estimated savings to $95 billion over 10 years. The bipartisan bill, which would limit price increases for drugs covered by Medicare and cap out-of-pocket spending by seniors, was approved by the committee months ago, but failed to gain sufficient Republican support. Its backers hope that efforts to ensure access to drugs developed to treat the coronavirus pandemic will increase support for policies promoting affordable drugs for Medicare and other health programs.

Although the final legislation calls for HHS to ensure that new vaccines, drugs, or diagnostics developed from added public funds are “affordable,” it also states that HHS can’t delay the development of new therapies in order to “maintain affordable prices.”

FDA updates policy to reflect disruptions

As the process for conducting conventional clinical trials becomes more difficult and more risky at a time when the public is advised to shelter at home and healthcare facilities become overwhelmed with caring for seriously ill patients, FDA is offering advice and added flexibility to help sponsors adjust ongoing and planned clinical research programs. Strategies for ensuring the health of participants and site personnel are mapped out, along with processes for maintaining the integrity of the research program if it is altered or discontinued altogether. Several biotech firms recently announced the termination of clinical trials or decisions to delay launching planned studies, and more companies are expected to follow suit. Posted March 18, the new FDA guidance for drugs, biologics, and medical devices recognizes that the COVID-19 pandemic is disrupting research programs, as the risk of infection prevents site visits and testing of participants. Site closures, quarantines, travel limitations, and interruptions in clinical supplies may make it difficult for sponsors and investigators to enroll participants, and patients may not be able to access sites or to spend a day in a clinic.

Such difficulties may require a sponsor to revise or reduce testing procedures, shift to online or remote monitoring, revise protocols, alter informed consent procedures, adopt new data collection practices, and to decide whether to continue a program. The guidance advises sponsors to fully document contingency measures implemented, all study participants affected by altered procedures, and the impact of participant discontinuation and alternative procedures for analyzing study results.

Although the final legislation calls for HHS to ensure that new vaccines, drugs, or diagnostics developed from added public funds are “affordable,” it also states that HHS can’t delay the development of new therapies in order to “maintain affordable prices”
There’s No Easing the Price-vs.-Value Debate

With coronavirus a stark backdrop, European initiative pushes for “fair” drug prices to spur investment in pharma innovation

It may seem inappropriate—not to say tactless—to be discussing a subject as mercantile as the price of medicines in the midst of a crisis of such dimensions as the novel coronavirus pandemic, with its growing human cost right across the world. But conspicuous among the many things that the current health crisis is demonstrating—from the heroism of healthcare professionals to the base cupidity of thefts of protective equipment—is the continuing need for the development of novel and effective medicines. And that, in crude terms, depends on putting investment as well as investigation into the process—investment that springs from the public purse, or from the revenues earned on earlier innovations, or from the expectation of a return on investment made now and in the future.

Who should put the money into research, who should benefit from the results, and who should pay for them are the questions that have underpinned these discussions for decades, and will doubtless continue to do so once the current crisis is resolved. But as the coronavirus began to wreak havoc on the ordered existence that two generations have come to take for granted in the developed world, the questions were thrown into high relief by a challenging initiative from one of the biggest organizations that pays the price for medicines: the Association Internationale de Mutuelles (AIM). AIM is a grouping of mutual benefit societies that spans 30 countries and meets the healthcare bills of a quarter of a billion people and pays out some $300 billion a year.

Much of AIM’s business is in Europe, and it has proposed “a European drug pricing model for fair and transparent prices for accessible pharmaceutical innovations.” The calls for “fair prices” and “access” are well-trodden ground in Europe (although the resulting footprints have tended to leave a confused jumble rather than any clear pattern), but the originality of the AIM approach is to set out an actual plan for how to achieve the objective.

Rethinking R&D

In summary, AIM suggests allowing an initial lump sum of €250 million for the R&D for each new drug, and to determine in advance the amount of R&D for the treatment of a single patient, based on theoretical prevalence of the condition. Adjustments upward could be made for “real” R&D expenses—to a cap of €2.5 billion—and for a smaller target population. For a high-prevalence disease, the model foresees R&D for the treatment of a single patient ranging from €20 to €1,200, according to the amount spent on R&D. For an ultra-rare disease, the allowable R&D for the treatment of one patient could rise to €1 million over the duration of the treatment, and for a life-long treatment, the model would consider a 10-year duration, meaning a cost for R&D around €100,000 per year. Allowances would be made for production and information costs and for a profit of 8%, and an “innovation bonus” of 5% to 40% could be granted in respect of added therapeutic value against available alternatives.

In a recognition of the diversity of national economies in Europe, AIM says the calculation method would deliver only an average fair price that would then be adapted to each country in line with its GDP. For an average price of €10,000 per treatment, actual prices would therefore range from €2,300 in Bulgaria to €20,500 in Ireland and €29,500 in Luxembourg. And that average price could be further adjusted in light of the price for the product in countries beyond Europe with a comparable standard of living and health system, unless the company could demonstrate that the resulting price does not cover the costs.

The formula would result, AIM says, in a hepatitis C medicine that today costs €40,000 being available at a European average price of €845 (and at €195 in Bulgaria and €2,500 in Luxembourg). Similarly, oncology products that today cost more than €50,000 would have been in the range of €5,000 to €10,000.

Policy potential

AIM has been promoting its concept around Europe’s political institutions, arguing that the current pricing system permits “excessive profitability as the final price bears no comparison with the development costs that
are usually used to justify pharmaceutical costs.” It claims that its model would provide “fair prices of medicines to reward what (really) matters.” And it underlines, as a leading payer, that “the fairness toward industry would go together with fairness toward health systems.”

This is sensitive and controversial territory in European policymaking, and the AIM proposal touches on many issues where opinions are sharply divided. No drugmakers have yet come out with explicit comments on the proposed model, but there are inevitably many questions and definitions that would need further refinement for it to gain political momentum.

Meanwhile, amid the confusions and uncertainties of the rapidly-growing healthcare emergency, the European private-sector pharmaceutical industry is pointing to what it has done and what it is trying to do to respond to the need for care. The main industry body, the European Federation of Pharmaceutical Industries and Association (EFPIA), is repeatedly emphasizing its commitment to seeking solutions, and is flagging up its members’ engagement in collaborative research programs to fast-track therapeutics and diagnostics, their donations of investigational compounds with potential as emergency treatment and for clinical trials, and their work in researching vaccine candidates.

But the messages carry a far-from-subliminal reminder that there is a bottom line to this activity. “The type of research effort needed to address a global health threat cannot be created in a vacuum or turned on and off when Europe needs it. It takes decades to build the right research ecosystem that can respond quickly to such a public health emergency,” said EFPIA Director General Nathalie Moll, as isolationist shutdowns were biting into normal life across the continent.

The industry’s public pronouncements tend to shy away from direct mention of needing adequate prices, preferring more nuanced allusions to ensuring patient access to innovation, maintaining a dynamic environment, or other similar circumlocutions.

One of the crucial conditions, Moll said, was “an intellectual property framework that inspires long-term investment into our R&D infrastructure.” Her trade expert Koen Berden reinforced the message: “Research in general, and research into innovative medicines and treatments in particular, can be characterized by being long-term, high-risk, and expensive.”

Moll reiterated that standard argument that one successful medicine has to earn back not only the R&D costs of the development of the drug itself, but also the costs made for all other failed R&D attempts and compensate capital providers for the risk they have taken with their investments.

Backing outcomes

The message is even clearer in a paper circulating within EFPIA that calls for a shift toward outcome-based pricing for medicines. This shares with AIM the conviction that “current pricing approaches are simply not fit for purpose for some of the innovations now coming to market”—but it diverges widely in its conclusions.

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It candidly states that “a sustainable system should balance budgetary needs (i.e., budget limits and predictability) while maintaining the necessary incentives for continued innovation. The failure to reach such a sustainable approach to medicines pricing is already leading to a situation where many patients do not receive the treatments they need and deserve.”

Core issue

With the current prominent display of society’s dependence on medical innovation to provide solutions to new diseases, the tide of public opinion may be flowing at present in favor of funding innovators to come up with vaccines and treatments that have real value, with less attention to price. But in the post-corona relaxation—whenever it comes—the discussion will again be reignited about where value and price converge. And the old dictum will again be cited: “A cynic is someone who knows the price of everything, and the value of nothing.”
Setting Goals, Meeting Needs

For Sandra Horning, the 2020 Healthcare Businesswomen’s Association Woman of the Year, harnessing the patient experience has been the driving force in a career-spanning—and service-focused—mission to fight disease and accelerate the delivery of new medicines

By Elaine Quilici

It only takes a second to know that Dr. Sandra Horning is a Midwesterner at heart. Her innocent accent recalls the fact that she grew up in Creston, Iowa. But Horning’s childhood was far from quiet.

Horning unwittingly learned about medicine at an early age. After experiencing several serious family illnesses, she underwent a couple of unrelated surgeries herself at the age of six. While the situation was scary, Horning hung tough and was able to find a silver lining. Her admiration for her pediatrician, combined with the mysterious nature of medical science fostered a lifelong interest in the field.

“Medicine seemed to me to be a really exquisite blend of science and helping people,” says Horning, named this year’s Woman of the Year by the Healthcare Businesswomen’s Association (HBA). “That was very attractive to me.”

She also noticed all the doctors were men and all the nurses were women at the time. It was then that she announced she wanted to be a doctor. Instead of feeling threatened, Horning embraced the challenge.

Just as she was planning to enter medical school at the University of Iowa, Horning was dealt another health blow: This time she received news that her father had terminal cancer. The experience again pushed her forward and fortified her resolve to become a doctor.

Horning next spent three years in New York completing her internship and residency in internal medicine. From there, she headed to Stanford University for her oncology fellowship, where she remained as a researcher, professor, and practitioner for nearly 30 years.

“In the school of medicine, you basically do three things,” says Horning. “One is you educate. For me, it was about educating trainees who were already physicians specializing in oncology and in my area of expertise, lymphoma. You do research,
and the research I did was clinical research, which means involving patients. And I practiced oncology in a very active clinic, taking care of patients in the hospital as well.”

**Timing is everything**

Though she spent most of her life in medicine, as a physician, academic, and educator, Horning’s path to pharma wasn’t a traditional one. In fact, she only moved into the industry in the last 10 years.

“It was a rather late transition compared to many,” she says. “But for me, it was really about the timing being right.”

Horning’s career experience had prepared her for the move, though, and she was ready to take on a leadership role. She had held several significant leadership positions at Stanford, nationally and internationally, and served as president of the American Society of Clinical Oncology (ASCO) from 2005-2006.

The combination of her drive to lead and the opportunity-filled field of oncology made Horning feel that the timing was right.

“I lived through the valley of death in terms of the lack of progress in oncology treatments to emerge in what I would call the golden age of oncology, where the science had developed and ripened to a place where we could really help patients through the development of highly active drugs,” she says. “That was very exciting for me.”

As a senior investigator in two Phase III trials for the drug Rituxan, which was developed by Genentech and Roche, Horning appreciated the major, positive impact the drug had on her patients and her practice. She was eager to continue and grow that type of work.

“It was really that experience that compelled me to want to do it again, make more drugs like Rituxan available to patients,” she says.

**Breaking barriers**

While Horning’s personal past wasn’t easy, her professional journey was likewise challenging. She and her female colleagues were often met with resistance as they rose through the ranks. She describes women in medicine at that time as “pioneers.”

“I feel that I was constantly underestimated in terms of whether I would achieve my dream of being a physician—whether I would or could excel in medical school, residency, or fellowship,” she says. “It was a continuous theme when I got into the academic ranks: whether I would succeed in getting tenure; whether I could secure grants; whether I could be an academic leader; and later, whether I could successfully transition to industry, or be a strong people leader as well as a technical expert.

“The other hand, I would say I was also supported, because a few significant champions opened doors for me and made my eventual success possible. And I’m very grateful for that.”

Horning’s challenges extended beyond just being a woman. She faced questions of credibility, having moved directly from academia into a lead-

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» Dr. Sandra Horning is currently an advisor to EQRx, a biotech startup she co-founded with other enterprise leaders in January. Launching with $200 million in Series A financing, EQRx is focused on making equally as good or better versions of drugs, but making them available to the public at lower prices. Horning is also presently a board member at Gilead and Moderna.

» Horning spent 10 years at Genentech and Roche, first joining in late 2009 as senior vice president, global head of clinical science/oncology and hematology in the product development organization. She was appointed chief medical officer and head of global product development in January 2014. During her tenure, Horning's team secured approval for 15 new molecular entities and numerous line extensions in oncology, hematology, neuroscience, ophthalmology, immunology, and infectious disease.

» Prior to joining Genentech and Roche, Horning was a professor, practicing oncologist, and investigator, and held multiple leadership positions, including vice-chair of the department of medicine at Stanford University, where she is an emerita professor of medicine (oncology and blood and bone marrow transplantation).

» Horning was named a Best Doctor in America consecutively from 1992-2008 and served as chairman of the Eastern Cooperative Oncology Group lymphoma committee and is a former president of the American Society of Clinical Oncology.

» The Iowa native received bachelor of arts and doctor of medicine degrees from the University of Iowa and completed post-doctoral training in internal medicine at the University of Rochester and in medical oncology at Stanford University.
ership position in pharma, becoming the global head of oncology for Genentech and Roche in late 2009, following a recommendation from a former colleague.

“I obviously had a steep learning curve and had to navigate that at the same time that I was establishing my leadership,” she says.

It didn’t help that Horning joined Genentech shortly after the acquisition by Roche. Integrating two cultures with very proud histories was also something to overcome, she says.

During her tenure, Horning’s teams secured approval for 15 new medicines and many more new indications for approved medicines. Two additional therapies are expected to receive FDA approval this year. When it comes to being successful in drug development, Horning says the most important ingredient is having a novel drug that addresses an unmet medical need. “Once you have a great product, it’s really being able to tell the story of the medical value so that it’s widely recognized and clear,” she says.

After being head of oncology for four years, Horning was faced with another formidable learning curve when she moved into the chief medical officer role. Leading the 5,000-person global product development organization required her to reach beyond her expertise in oncology and hematology to oversee all therapeutic areas and all aspects of development.

“What kept me going was my passion for the field of medicine, for patients, for wanting to make a contribution to society, and the purpose I derived from long-ago personal health issues—knowing how these affected my family and me, and wanting to make it better for others,” she says.

Launch and learn

Horning’s strong science background, excellent work ethic, and thirst for knowledge also helped her overcome these obstacles.

“Science is the core of Genentech and Roche’s business, so Sandra’s impressive clinical science background was a natural fit,” says former colleague Gisela A. Paulsen, MPharm, senior vice president, global head, product development clinical operations at Genentech and Roche. “What made her an exceptional leader was how she paired her intellectual capacity with her innate ability to educate. Because of her experience in academia, Sandra came to Genentech and Roche with the technical knowledge and the ability to teach those around her.”

“[Given my] experience with my family and all my experience with my patients, as well as my knowledge of oncology, I kind of expected to have cancer at some point in time,” says Horning. “What surprised me is that it occurred so early in life, when I still had children at home.”

During her tenure, Horning’s teams secured approval for 15 new medicines and many more new indications for approved medicines. Two additional therapies are expected to receive FDA approval this year. When it comes to being successful in drug development, Horning says the most important ingredient is having a novel drug that addresses an unmet medical need. “Once you have a great product, it’s really being able to tell the story of the medical value so that it’s widely recognized and clear,” she says.

Other strategies Horning suggests include:

» Considering access and affordability very early in development and building that story concurrent with the drug’s development.

» Identifying appropriate physician champions, educating them, and partnering with them.

» Having a strong commercial team at the time of the launch.

“Sandra is one of the brightest persons I know,” says Paulo Fontoura, another former colleague, today senior vice president and global head of development for neuroscience medicines at Genentech and Roche. “The depth of her knowledge about anything is just amazing. She has a true curiosity about things and doesn’t really rest until she has the answer—and she doesn’t settle for good enough.”

Fontoura describes Horning as being “comprehensive” when it came to researching an unfamiliar topic. She wouldn’t just educate herself by reading articles, for example. She also saw the value in interacting with and learning from others.

“Sandra always made sure to listen to a lot of people,” says Fontoura. “She’d listen to all the experts and ask really deep questions to try and find out as much as possible. She was somebody who was open to feedback and other views, but she would certainly make up her own mind in the end.”

Patient perspective

In 1996, Horning had to face the realities of cancer from a new angle when she was diagnosed with breast cancer.

“She has a true curiosity about things and doesn’t really rest until she has the answer—and she doesn’t settle for good enough.”
made a difference. “Going through cancer treatment while I continued to practice created a very special bond with them,” she says.

As an oncologist, Horning had witnessed many of her patients undergo a personal epiphany during their treatment, often expressing ways they planned to do things differently in their lives. Horning didn’t feel like she had to change her path, however. For her, it was all about “doubling down.”

“It did help me to better understand the fear of death, the desire to live,” she says. “One of the first projects that I worked on in my academic career involved the curative treatment of young patients, where I focused on their fertility after treatment and their ability to maintain healthy lives as survivors. Survivorship has been something that has interested me throughout my career. When I was president of ASCO, we set up some of the survivorship programs, and it resonated that I was a survivor myself.”

The journey helped Horning realize how much life there is to live after survival. “That’s really increased my passion for not only wanting to develop curative treatments for patients, but also to focus on quality of life and survivorship,” she says.

Motivated medicine
Horning’s personal background and experience in clinical practice helped her keep patients at the forefront of every decision and prompted her to consider what the right thing for patients was during the drug development process.

“We had this motto, ‘Patients are waiting,’” she says. “Especially when patients have a really serious or terminal disease, every hour, every day, every week that goes by is important. So we focused a lot on acceleration, how fast we could get medicines developed and launched and in the hands of physicians. We had examples where patients were literally waiting, and we were able to deliver our new medicines within hours of approval.”

Horning’s responsibility to patients was even evident during moments of failure.

“Once, when we had a trial that did not work, her first reaction was to say, ‘I’m so sorry, because patients were really counting on this,’” says Fontoura. “What mattered to her was not the scientific success or progression and milestones, it was patients who were waiting for something, and we didn’t manage to give it to them. That really drove home what her value chain was: First of all, the patients.”

In an effort to streamline the development process, Paulsen recalls one of Horning’s boldest moves: a complete overhaul of the teams that manage molecules through Genentech and Roche’s development lifecycle.

“She pushed the organization to work in new ways, adopt iterative work processes, and embrace learning over perfection,” says Paulsen. “Her example catalyzed a 5,000-person organization to initiate a series of initiatives aimed at accelerating every aspect of the development process, from protocol writing to clinical trial execution to regulatory filings. The cumulative impact of these efforts has shortened the previous time for development by several months to date, and we are not done yet.”

As part of Horning’s initiative to bring drugs to market more quickly, she and her team supported the creation of new regulatory pathways, including FDA’s breakthrough designation. Genentech and Roche led the industry with 32 new breakthrough designations across a variety of therapeutic areas in just seven years. They also pioneered a new regulatory endpoint, pathologic complete response, which allows for early approvals in early stage breast cancer.

“What kept me going was my passion for the field of medicine, for patients, and the purpose I derived from long-ago personal health issues, and wanting to make it better for others.”

— SANDRA HORNING
there was a growing buzz around oncology. Research on promising new mechanisms was prolific. Regulators also knew patients facing lethal cancers were waiting for more options and didn’t have time to waste. This, perhaps, created an openness to think about new pathways, says Fontoura.

“I think regulators, pharma, and the community generally saw this as an opportunity,” he says. “This has since opened up opportunities in other therapeutic areas, and that is really exciting. It’s one of those things when we look back, that people will say, ‘This was a turning point’ in the regulatory process, when people recognized that the one-size-fits-all ends up not fitting anyone and that you really have to take a different approach.”

Former colleague Quita Beeler Highsmith, vice president and chief diversity officer at Genentech, sees Horning as a “visionary, mentor, and trailblazer.”

“I have witnessed firsthand how Sandra can be a game changer for this industry,” says Highsmith. “She has influenced patient outcomes by leading a record number of industry breakthrough therapy designations, has served as the American Society of Clinical Oncology president, and her leadership allowed for Genentech to be a driving force to bring more diverse patients into clinical research.”

Leading by example

Horning’s sense of service transcends her ability to connect with patients; it has also allowed her to establish herself as a respected leader in the industry. “Being an effective leader in today’s environment means having a service mindset,” says Horning. “An effective leader needs to have a vision; be an architect of the strategy and create the environment where molecules and people thrive; build a team of A players; and serve as a coach to focus on the success of others, the organization, and the molecules.”

In addition to Horning’s scientific excellence and care for patients, Fontoura believes her ability to change has been integral to her success—not just having the courage to change herself but the power to catalyze change in others.

“She was willing to let go and push others to the forefront,” he says. “When you have such high standards and work ethic, your first instinct is to do it yourself. But she really was able to transform herself and inspire others to do the same. I think she found that secret of great leadership, which is that sometimes when you step back, you create space for others to multiply your impact.”

Horning helped coach and develop many leaders at Genentech and Roche. “In many ways, that’s probably part of her legacy that’s going to outlive her, perhaps even more than the scientific one,” says Fontoura.

Among those colleagues were a number of women who identified with some of the same professional challenges Horning faced throughout her professional journey and welcomed her example. Horning was now in a position to pay it back and inspire other women in the industry.

“In the field of science, it is very important to recognize women thought leaders that are making extraordinary breakthroughs to drive science and
medicine for the next generation of physicians and patients,” says Beeler Highsmith. “What I admire most about Sandra is her authenticity, her drive to make a meaningful difference for patients, and her role-modeling of what it means to be a bold leader. Plus, she is a style icon.”

**New beginnings**

Toward the end of 2019, Horning felt satisfied with what she had accomplished at Genentech and Roche and decided it was time to begin a new chapter of her life. In January, she joined forces with industry visionaries as a co-founder of EQRx, a company committed to making innovative medicines available to the public at lower prices, where she continues to be an advisor. In February, she joined Gilead Sciences and Moderna as a board member.

“I felt the time was right for me to move into a third act, another area of life where I felt like I could continue to contribute and give back,” she says. “What I want to do is be of service to the management and executive teams of new and established biotechnology companies. I’d like to bring my expertise and leadership experience to assist companies in making great medicines for patients.”

Though Horning has traveled a long way to get to this point in her career, it’s her sincere and unwavering passion for medicine, which blossomed early in life, that still keeps her going today.

“One of the most devastating things for me in life was losing my father at a young age,” she says. “Witnessing that loss with its permanent impact on me and my family, and then going on my own journey of illness, followed by the exhilaration of restored health and the ability to live a full life after a serious illness—those are poignant and precious experiences that continue to motivate me today.”

“An effective leader needs to have a vision; be an architect of the strategy and create the environment where molecules and people thrive.”

ELAINE QUILICI is Pharm Exec’s Senior Editor. She can be reached at equilici@mjhlifesciences.com
COVID-19 Highlights Need for More Agility, New Paradigms in Drug Development

The rapid spread of COVID-19 across the globe highlights the absolute need for preparedness in the face of pandemics in order to reduce and eliminate the emergence of new cases and quickly create treatment options for emerging illnesses and diseases. As it stands now, the sluggish response to the current crisis combined with the slow, rigid development process for therapies have been extremely inefficient and ineffective at handling the current crisis, thus signaling the need for faster, more agile paradigms.

In mid-March, Pharmaceutical Executive spoke with three industry leaders who have broad experience in technology, research and clinical approaches to epidemic disease:

- Srinivas (Sri) Shankar, Senior Vice President and Global Head of Life Sciences at Cognizant
- Ulo Palm, MD, PhD, Senior Vice President of Digital Sciences at Alergan
- Thomas A. Bock, MD, MBA, Founder and former CEO, HeritX; Chair of the Columbia Business School Healthcare Advisory Board; and former Senior Vice President, Global Head of Medical Affairs at Alexion Pharmaceuticals

They believe the medical community, life sciences industry, health institutions, governments and regulatory bodies must act with a sense of urgency to examine any and all recommendations that bear a promise to delay or stop the spread of global pandemics such as COVID-19.

Urgency to Act

In just three months, COVID-19 mushroomed from a local outbreak of atypical pneumonia in Wuhan, China, to a global pandemic spread of SARS-CoV-2, a novel zoonotic coronavirus. Retrospective analysis suggests the disease was circulating in China as early as November 17, 2019, although the first case was not announced until December 8. The first case outside China was identified in Thailand on January 13, 2020 and the first US case was identified on January 21. As of March 19, 2020, more than 200,000 cases of COVID-19 had been confirmed worldwide and more than 9,950 deaths occurred as the pace of infection continued to climb.

“We have to solve these kinds of rapidly escalating problems, which potentially can kill millions of people if they are not managed quickly,” Dr. Bock said. “We must act with two critical concerns in mind: what is best for the patient and how to develop treatments and vaccines as fast as possible. Technology today gives us the tools that allow us to move ahead much more aggressively.”

But current technologies and tools are not being applied broadly. In early March, South Korea, a secondary epicenter of COVID-19, was testing 3,692 people per million population. Meanwhile, the United States was testing a mere 23 people per million.

“We do not have the sense of urgency needed,” Dr. Palm said. “We need to be able to act fast to save our patients. We have to think and to act at the speed of viral replication. If we don’t keep up with the virus, we will pay a very high price.”

Combining RCTs with Accelerated RWE Studies and Real-Time Clinical Analysis

Reliance on established procedures is hampering the rapid development and deployment of novel therapies for COVID-19. Traditional drug development relies on randomized controlled trials (RCTs), a framework developed in the 1940s. RCTs have a proven track record, Srini Shankar said, but are time-consuming to execute. It can take months to create a study protocol and receive approval, several more months to set up studies, and still more months for recruiting and randomizing patients, collecting data and analyzing it. All told, RCTs often take several years to complete.

“They stand now, the sluggish response to the current crisis combined with the slow, rigid development process for therapies have been extremely inefficient and ineffective at handling the current crisis, thus signaling the need for faster, more agile paradigms.”
Srini Shankar suggested that in addition to needing to operate in an “emergency mode” for clinical trials, the gold-standard placebo-controlled model is inappropriate for finding treatments to combat current pandemics. “Is it even ethical to use a placebo given the mortality risk? We have an absolute need to think outside the box with non-traditional, unconventional trial designs. RCTs must be complemented with a different approach to bringing therapies to market based on the urgency of the situation,” he stated.

One approach is to adapt existing technologies to drug development using real-time, real-world evidence (RWE) and adaptive biostatistical models to evaluate experimental agents in real patients. “Using real-world data to guide key operational decisions is an established reality in the commercial world,” Srini Shankar noted. “RWE can just as readily be applied to individual patients and populations to track drug response, disease progression, safety and other clinical factors in real time.”

He feels accelerated studies should happen under the umbrella of a simplified pragmatic RWE study, in which the data is collected—preferably on an existing drug in the development pipeline with some demonstrated efficacy and tolerable safety profile based on preclinical studies. The clinical data should then be continuously monitored and analyzed, almost in real time, leveraging existing EHR/EMR networks. Data would be analyzed with statistical approaches that are different from what is employed in RCTs, allowing us to react quickly on a large scale, balancing the vital needs of individual patients with the need to collect scientific data about efficacy and safety.

A precedent for this approach already occurred in Germany. The 2011 outbreak of Shiga toxin-producing *E. coli* hemolytic uremic syndrome quickly overwhelmed hospitals, clinics, dialysis units and other healthcare resources. There were no approved therapies, but an Alexion agent, eculizumab, had a favorable therapeutic profile and acceptable safety.

A coalition of industry, government, researchers, regulators and clinicians created an emergency treatment protocol that began to enroll patients within 24 hours and provide drugs within 48 hours using then-current data collection and analysis techniques, according to Dr. Bock, who led the Alexion team. At the same time, a formal regulatory approved study was prepared. After its initiation in six weeks, the study was back-loaded with accrued patient data and updated daily. Working outside the accepted industry norms and using an experimental agent under controlled, but not randomized, conditions allowed clinicians to curtail and end the crisis in two months.

“Government and regulatory bodies must be included in this kind of program, and the top leadership of the companies be involved because there is a different risk posture than a typical drug development program,” Dr. Bock said.

There have been fragmented attempts to use existing antiviral agents against COVID-19 in places like China, but no coherent global, or even national, experimental use programs. It is possible that existing agents such as Remdesivir (Gilead Sciences), a broad-spectrum antiviral developed to treat Ebola, could be trialed in real time. The National Institutes of Health initiated a randomized clinical trial of Remdesivir in February, but the conventional RCT is not taking advantage of current technology.

**Leveraging Technology for Accelerating Therapies to Patients**

“If it is ethical to move ahead in a clinical trial, why isn’t it ethical in an emergency to use that same agent in patients and be very transparent about it?” Dr. Bock asked. “Technology today gives us the tools that allow us to move ahead and track outcomes in real time.”

One specific technological advancement that might be useful while exploring treatments for vaccines are Patient Reported Outcomes (PROs). In the case of COVID-19, it is believed that the majority of afflicted individuals exhibit mild symptoms and be under self-quarantine. While they may be unable to visit clinics and record clinical data, they can easily use mobile apps for reporting clinical outcomes such as the severity of cough and temperature measurements. Remote monitoring can be orchestrated to support them through this real-time data collection, providing vital data streams for clinical analysis, avoiding the draw site activation and traditional patient recruitment. Mobile apps can be deployed and updated within days, thereby even rendering the RWE study “adaptive,” in addition to real time.

Srini Shankar also believes that a virtual “visual command center” should be created, possibly at the CDC, where data streams from EMR systems and electronic Clinical Outcome Assessment (eCOA) platforms come together from across the globe to provide a real-time assessment of how the infected patient population is reacting to emerging investigational treatments. “Umbrella designs, where new treatments are seamlessly added while ineffective ones are removed, allows you to determine safety and efficacy in real time,” Srini Shankar adds.

**Final Thoughts**

As an industry, Srini Shankar, Dr. Palm, and Dr. Bock say we are nowhere near exhausting what’s possible and the boundaries of human imagination in dealing with a crisis of this magnitude and velocity. The answers are within our reach and we have an obligation to act now.
Doubling Down on Diversity & Inclusion

Exploring just how far new industry initiatives and strategies are pushing the needle on D&I in pharma

By Julian Upton

In the last decade or so, the issue of diversity and inclusion (D&I) has advanced steadily within the life sciences discourse, its growing prominence reflected in the job titles that have emerged at senior levels among the front rank of pharma companies. The industry now boasts heads of diversity & inclusion, chief diversity officers, and corporate leads in D&I, culture, and engagement. To convey the message that D&I is no longer simply regarded as a “nice to have,” or approached as an add-on to existing anti-discriminatory compliance, the industry has boosted its efforts from hiring policies to media campaigns. But the argument persists that pharma is well behind the curve when it comes to realizing its D&I goals, particularly at the senior and board levels, where women remain under-represented (and where people from ethnic and racial minorities remain very under-represented). As this journey toward achieving real balance and representation goes on, Pharm Exec spoke to leading D&I exponents from two big pharmas (Novartis and AstraZeneca) and consultants with long experience of recruiting emerging leadership talent to see how far the latest industry initiatives are moving the needle on D&I.

“Our inclusion and diversity strategy has been essential in ensuring we keep our collective efforts focused,” Rebekah Martin, SVP, Reward and Inclusion at AstraZeneca, told Pharm Exec. She reports “significant strides” in the company’s D&I activities, with D&I now established as one of the three pillars of its People strategy, which “has helped us to embed inclusive behavior and leadership throughout the company.” She goes on to outline the recent recognition AZ has received for its D&I efforts. “We were one of 325 companies selected for this year’s Bloomberg Gender-Equality Index, which distinguishes companies committed to transparency in gender reporting and advancing women’s equality,” says Martin. “We earned a 100 on the Human Rights Campaign’s index to designate AstraZeneca one of the 2020 Best Places to Work for LGBTQ Equality. [And] last year’s Hampton-Alexander Review named AZ plc as one of the top 10 best performers in the FTSE 100 for representation of women on the combined executive committee and their direct reports.”

Over at Novartis, Vice President and US Country Head, Diversity and Inclusion, Marion Brooks says he has seen a lot of progress during his 20-plus years with the company. “The level of focus has definitely grown around D&I,” he told Pharm Exec. It is now “a fundamental part of Novartis’ cultural aspirations, as well as its strategic priorities.” The company has established three global strategic imperatives: “diverse people, inclusive environment, and shaping our society,” says Brooks, under which there are six initiatives: gender balance in management; pay equity and transparency; attracting and retaining diverse talent; creating inclusive learning offerings for everyone; LGBTI equity; and strengthening employee resources groups (ERGs).

The latter, particularly, play a major role in the pursuit of improved D&I. Novartis has over 60 ERGs globally which, according to Brooks, “really
help us to support the values and behaviors, as well as the business codes, of our organization.” Before taking his current position, Brooks spent 20 years on the commercial side of Novartis, but during that time he was heavily involved with ERGs, leading one for a number of years. “ERGs help to foster a level of belonging between the organization and its associates and drive our inspired, curious, and unbossed culture,” he explains.

AstraZeneca has upwards of 15 ERGs worldwide, led by volunteers. They play an important role, says Martin, “in continuing the dialogue throughout the year, keeping our efforts aligned to our business strategy, and providing capabilities to drive forward our inclusion and diversity strategy.” One example is AZ’s SAFE SPACE ERG, which has been “hugely successful” in supporting colleagues and improving the dialogue around mental health. Another ERG, which originated from AZ employees, is AZPride. Last year, AZPride ran the “This is Me” campaign, which saw employees sharing their stories of coming out and their experiences as a member or ally of the LGBT+ community. “These powerful stories,” says Martin, “were spread internally and externally through our social media platforms throughout Pride month and provided education and an opportunity to connect across the organization.”

Global reach

While these D&I initiatives can be very effective in addressing issues that affect an increasingly diverse workforce, there is something of a first-world sensibility to many of them. Multinational companies like Novartis and AZ are well used to operating sensitively within disparate cultures—this in itself requires diverse and inclusive thinking—but can a western-hatched approach to D&I be meaningfully implemented in every corner of the world?

Brooks believes so, and says it’s important for Novartis to make a global impact in this way. Over the last decade, he explains, D&I has become more of a global function and a globally aligned strategy at Novartis. “It’s a part of everything that we do, and it should show in our day-to-day actions,” he says. “We have over 108,000 associates and we operate in over 100 countries, so we have a huge opportunity to help to effect change across the globe.” One example is taking a stance on global topics such as equal pay. “Those are opportunities for us not only to influence and impact our associates but also to help shape the reality of the individuals in the countries where we operate,” says Brooks.

Martin asserts that a global approach to D&I is achievable with the “right framework in place.” Last year, AZ launched its Global Inclusion and Diversity Council. Chaired by AZ’s CEO, Pascal Soriot, it “has enabled us to set the tone from the top,” says Martin. The Council includes representatives from each of the senior executive team areas and also has a Global Standard for Inclusion and Diversity, “which sets out how we foster an inclusive and diverse workforce where everyone feels valued and respected because of their individual ability and perspective,” adds Brooks.

Certainly, AZ appears active in promoting its global D&I efforts. In 2018, it held a crowdsourcing event to help shape the next phase of its strategy, with employees across the world contributing to the activity and generating more than 56,000 ideas, according to the company. Last year, the company released its Global Diversity Report, which found that 48% of the company’s employees are female, up from 42% in 2017, and that 13% of employees are from a visible minority, up from 10% in 2017. The company also has a Global Standard for Inclusion and Diversity, “which sets out how we foster an inclusive and diverse workforce where everyone feels valued and respected because of their individual ability and perspective,” adds Brooks.

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» According to PwC’s Diversity & Inclusion Benchmark Survey, released last year, D&I is a stated value or priority for 68% of healthcare organizations. Only 51% of respondents disagree that diversity is a barrier to progression at their company.

» The survey found that the primary objectives of D&I among respondents is to attract and retain talent (38%), comply with legal requirements (25%), and achieve business results (17%).

» In a report issued in January by the Biotechnology Innovation Organization (BIO), among 100 biotech companies surveyed, on average, only 30% of executive positions and just 18% of board seats are held by women. Roughly four-fifths of CEOs were men and almost 90% were white, the survey found. In early 2018, BIO posted diversity goals for the industry to hit by 2025: gender parity—50% women—among company leadership, and 30% female board membership.
year, for the first time, AZ held eight “Empowerment Summits” across the US, Asia-Pacific, Brazil, Sweden, Poland, and the UK. “While the approach is global, all of the content is curated by local teams to ensure it is relevant and effective,” explains Martin. “They were so successful that we have plans for expansion to other countries this year.”

Diversity and disruption
Pharma companies’ D&I web pages are filled with inspiring copy, but it can be more sobering to compare the sector’s position on the diversity curve with that of smaller biotechs (and other advanced industries). While the recent BIO report, Measuring Diversity in the Biotech Industry, highlighted areas where the biotechnology sector requires major improvement—for example, 88% of the companies responding to the survey had a white, male CEO and only 16% reported having goals to promote or develop women—it also pointed to an emerging trend whereby startup businesses “are generally better than their larger counterparts at recognizing the value of diversity,” says Chris Coe, executive vice president of Talentmark.

“In the report, pre-revenue, smaller, and private companies all showed positive outcomes for representation,” he told Pharm Exec, pointing to findings that show pre-revenue organizations are more likely than profitable organizations to have executive levels that are at least 25% people of color; small businesses are more than twice as likely to have a female CEO, compared with larger companies; and privately held organizations are more than three times as likely to have a person of color as CEO.

“These results may seem unfairly skewed for big pharma,” adds Coe, but “it can be far easier for fledgling businesses to establish a culture of inclusiveness and attract a more diverse workforce.”

Katy Wallace, principal consultant in the life sciences practice at Berwick Partners, observes that “the entry points in the smaller businesses are better for more diverse candidates, because they tend to be more able and willing to challenge the status quo—this is a key factor in improving and championing diversity.” She explains: “Big pharma is definitely moving in the right direction, but progress is slower than we see in some sectors. Some feel that this redefinition of what leadership looks like hasn’t resonated enough in the larger organizations, but it is more of a challenge in pharma to change quickly as so much of the hiring at grass roots is dependent on education. However, when this barrier of entry is removed, unconscious bias is also removed, people are then weighed and measured more fairly on their skills, which allows for a more diverse candidate pool.”

As big pharma is cash rich, change is typically driven by a need to change, which can be down to loss of revenue or legal implications, says Wallace. So that pressure for diversity hasn’t been as strong or as visible as it is now in big pharma. “It is being addressed for sure, and there are some wonderful initiatives in place,” she says. “However, the problem I see with candidates coming up through the ranks is that they don’t feel the speed of change is sufficient and, therefore, will look to work with businesses they feel are more adaptive and action-orientated when it comes to addressing diversity.”

An area where life sciences as a whole is now starting to struggle and, therefore, is going to have a “real problem” in the future is initial grass-roots attraction, notes Wallace. “We will struggle to get these people through the door.” The industry, she says, should be trying to do more to work with schools, particularly disadvantaged schools, which don’t have the resources or the teaching power, to help push students through the STEM subjects.

At the university level, the industry should be helping to make sure candidates don’t drop out of their field of study. Wallace welcomes all initiatives going on within life sciences businesses to encourage a more diverse and inclusive culture, not least the creation of a chief inclusion officer, “which denotes a real desire of the boards to make meaningful change in this area.”

But, she adds, “the industry also needs to be looking at what it can be doing outside of businesses, in the community at entry level.”

Tipping the gender-balance
While, in Wallace’s opinion, pharma seems to have been misguided in considering diversity as a gender issue alone, she notes that pharma initially made outstanding progress in areas such as increasing female representation on boards. But in recent years this progress has slowed down somewhat. “There may have been a bit of complacency, with some of the bigger busi-
nesses saying, ‘Okay, we’ve got one woman on the board now, that’s great.’ The problem is, what happens when that woman leaves, and the board goes back to zero female representation, which unfortunately has happened? No one was looking at that.”

Wallace notes that if an organization has more female leadership, it’s proven that the sense of inclusiveness filters down through the company, and that’s not just an increase in female leaders, but leaders from racial and ethnic minorities too. It’s also been proven, she says, that more diversity heeds better operational results and share performance that is being recognized by some of the industry’s leading businesses. But it doesn’t necessarily rely on a “top-down” approach.

“How do you get women and diverse candidates into those positions in the first place? Eighty percent of directors are male,” notes Wallace. “Progress needs to come from the bottom-up, because your pull of leadership comes from underneath; it comes from succession and how can a business promote diversity at board level; if it lacks the numbers lower down, it becomes an impossible fight for the boards.”

Wallace does say, however, that “the gender-balance conversation is starting again.” She is hopeful that the sector will change over the coming months and years. “We do have more female/diverse leaders, so we’ve got more female/diverse influencers across the industry, which keeps the conversation alive.”

AZ’s Martin reports that the organization improved female representation at management levels from 39% in 2012 to 45.4% in 2019. And at Novartis, “right now, 44% of our managers are female, and overall, 50% of our workforce is female,” says Brooks. “As the statistics improve,” says Wallace, “we’ll be able to see the positive effects a truly diverse and inclusive culture has on bottom-line profit, which hopefully, in turn, will drive more diversity.”

“As the statistics improve, we’ll be able to see the positive effects a truly diverse and inclusive culture has on bottom-line profit, which hopefully, in turn, will drive more diversity.”

**Business values**

Martin agrees that her organization’s D&I work “is far from done.” She stresses, though, that AZ is always striving to achieve more. The company’s recent external activities, for example, include joining the United Nations Free and Equal Standards for Business—aimed at tackling discrimination against LGBTI people—and committing to the United Nations Women’s Empowerment Principles dedicated to corporate actions to endorse equality.

As for Novartis, of its six D&I initiatives, pay equity and transparency is to be executed and rolled out in the US in June, and gender balance in management is “a goal for us by 2023,” says Brooks. He adds that the company has implemented new hiring guidelines that address female as well as ethnic and racial diversity in the US, and has hired a D&I scouting and recruiting team for the first time “to help us to engage with diverse communities and to build a pipeline of diverse talent.”

Like other organizations, AZ and Novartis have also reached an understanding of D&I’s “strategic and economic” importance to their businesses. Key to D&I’s wider acceptance and implementation is its growing impact on the bottom line. “D&I has become a competitive advantage,” says Brooks. “The data show that diverse organizations and teams deliver three things that more homogenous teams do not: higher revenue, more innovation, and they are more responsive to customer needs. Whether you’re a little league team or a major corporation, you want to deliver on those three benefits.”

At AZ, Martin believes there’s no doubt that “having an inclusive and diverse workforce was pivotal to our success in returning to growth and essential for our success today.” Bringing together different ways of thinking, she adds, is “the foundation of the company’s ability to innovate, which is a differentiator for us and has propelled our growth at an individual and company level.”

Martin is keen to emphasize, however, that business growth is not the main goal of D&I. “It’s just as important that we are doing the right thing and nurturing a values-based culture where we can all enjoy coming to work every day.”

**Marion Brooks**

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Payer Perspectives on Gene Therapy Reimbursement

Follow-up survey of clinical and financial health plan leaders uncovers evolving and more-open views on the management of high-cost, curative-intended treatments with one-time administration

By Jane F. Barlow, Matt W. Courtney, and Mark Trusheim

The reimbursement of emerging durable and potentially curative cell and gene therapies challenge US payers due to their high upfront costs. In addition, the lack of long-term clinical durability data amplifies uncertainties that complicate payer coverage and reimbursement determinations. These cell and gene therapy characteristics could negatively impact patient access and, ultimately, future developer innovation.

The Financing and Reimbursement of Cures in the US (FoCUS) project was launched in 2016 by MIT’s NEWDIGS Initiative with the objective of elucidating the challenges and financial impact created by durable/potentially curative therapies and providing implementable models to manage the financial impact on the US healthcare system. To that end, FoCUS has conducted two surveys to reveal present payer perspectives regarding current and future management of high-cost durable therapies with one-time administration.

The first FoCUS payer survey (FOCUS I) was conducted in August to September 2017 as the initial chimeric antigen receptor (CAR) T-cell therapies, Kymriah (tisagenlecleucel/Novartis) and Yescarta (axicabtagene ciloleucel/Kite Pharma) were being approved. It consisted of structured telephone interviews with 15 payers across multiple payer segments. At that time, payers had variable awareness and readiness to manage the new cost of these and other emerging gene therapies such as Luxturna (voretigene neparvovec-rzl/Spark Therapeutics), which was approved in December 2017. One-third of these payers were newly aware and learning about these therapies, with 40% watchfully waiting and 27% engaged in active management. Payers were open to financing mechanisms, with 47% expressing a willingness to engage in innovative financing models, performance-based annuities, and risk-pooling.

Additional payer surveys were published by the Alliance for Regenerative Medicine (ARM) and the
National Association of Managed Care Physicians (NAMCP) of 36 respondents in March to June 20173 and the National Pharmaceutical Council (NPC) of 21 respondents in February to March 2018.4 While specific questions and methodologies differed, in combination, the three surveys reported a general progression in awareness and concern regarding management of durable therapies, with 10% (ARM/NAMCP) to 27% (FOCUS I) and, ultimately, 100% of payers (NPC) reporting having started to consider coverage or operational issues associated with these treatments.

The most recent FOCUS payer survey (FOCUS II), consisting of 15 online questions, was conducted with clinical and financial health plan leaders from 77 US payers between September 2018 and April 2019 (note: Novartis gene therapy Zolgensma was approved in the US in May 2019 and launched two months later). The survey again focused on assessing payer perspectives regarding current and future management of high-cost durable, potentially curative therapies with one-time administration.

Participants in the FOCUS II survey represented 153 payer segments, including commercial fully-insured plans, self-insured employers, Medicare, and Medicaid. Payers ranged in size from less than 5,000 insured lives to upwards of 50 million and in total covered over 280 million lives (see Table 1). The survey did not control for more than one person from the same plan completing the survey. Results from intermediaries such as pharmacy benefit managers were excluded to the extent they could be identified.

Respondents self-identified among roles in pharmacy (53%); medical (31%); human resources and benefits (9%); and finance and actuary (7%). When analyzed by payer segment, commercial fully-insured plans expressed the highest levels of concern, with 93% reporting a high or extremely high concern and 7% reporting a moderate concern (see Figure 1). In comparison, only 50% of self-insured employers reported a high or extremely high concern, with other payer segments in-between, with 76% reporting high or extremely high concern.

Payers selected a number of reasons for their high or extremely high concern:

» The total cost is material for the plan (98%).
» Drug performance risk (effectiveness and durability) (91%).
» Actuarial risk (likelihood of encountering an orphan case) (91%).

Three major findings from FOCUS II

1) While payers are concerned about financial risk and impact of high-cost durable therapies, nearly all are covering them.

All payers have some level of concern related to the financial risk and impact of these treatments. Eighty percent of payers rate their concern as high or extremely high, with 39% of 153 payer segments rating their concern as extremely high, 41% as high, 18% as moderate concern, 2% as slight concern, and 0% as no concern.

Table 1. Survey participation by segments and covered lives (n=77 payers).

<table>
<thead>
<tr>
<th>Payer segments n/ (percent of participants)</th>
<th>Commercial Fully-Insured</th>
<th>Medicaid</th>
<th>Medicare Advantage</th>
<th>Self-Insured Employer</th>
<th>Total/Range</th>
</tr>
</thead>
<tbody>
<tr>
<td>Payer segments n/ (percent of participants)</td>
<td>55 (36%)</td>
<td>42 (27%)</td>
<td>46 (30%)</td>
<td>10 (7%)</td>
<td>n=153</td>
</tr>
<tr>
<td>Range of covered lines (000’s)</td>
<td>10 - 54,000</td>
<td>10 - 13,200</td>
<td>5 - 8,000</td>
<td>3 - 400</td>
<td>3 - 54,000</td>
</tr>
<tr>
<td>Lines Covered</td>
<td>184,335,447</td>
<td>59,379,661</td>
<td>36,508,957</td>
<td>933,854</td>
<td>281,157,919</td>
</tr>
</tbody>
</table>

Note: Survey participants reported engaging in more than one business segment.

Figure 1. Level of concern regarding managing the financial risk and impact of high-cost durable therapies, by payer segment (n=153 payer segments).
» Payment timing relative to benefit realization (offsets may not cover the high cost of treatment) (84%).

Other identified reasons for high concern include:

» Burden of multiple high-cost therapies.

» Concern about what this will represent in three to five years with respect to total cost.

» Potential off-label use.

» Operational management of a larger pipeline.

» Adverse selection.

Nearly all payers surveyed (99%) currently covered one-time, high-cost durable therapies; 46% cover all treatments then approved, while 53% cover some. For those covering high-cost durable therapies, payers are evenly divided regarding utilization management practices. Forty-eight percent cover high-cost durable therapies as specified in the FDA-approved label. Forty-nine percent apply utilization management coverage with more restrictions than the FDA-approved label. Three percent cover with less restrictions than the label.

Commercial fully-insured payers are the most restrictive, with 62% managing coverage with more restrictions than the FDA-approved label. Fifty-four percent of Medicaid plans reported utilization management that was more restrictive than the label, with 44% covering as specified in the label. Medicare Advantage is the least restrictive, with 67% managing consistent with the label and 31% more restrictive.

Case management is used by 82% of payers and centers of excellence (COE) are required for 64% of payers surveyed. Thirty percent restrict use of all high-cost durable treatments to COE, while 34% restrict some of these treatments to COE.

Financing Preferences

<table>
<thead>
<tr>
<th>Financing Preferences</th>
<th>Commercial Fully-Insured (n=55)</th>
<th>Medicaid (n=42)</th>
<th>Medicare Advantage (n=46)</th>
<th>Self-Insured Employer (n=10)</th>
<th>All Payer Segments (n=153)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Population risk-pooling</td>
<td>69%</td>
<td>40%</td>
<td>52%</td>
<td>50%</td>
<td>55%</td>
</tr>
<tr>
<td>Short-term MBC</td>
<td>75%</td>
<td>50%</td>
<td>61%</td>
<td>20%</td>
<td>60%</td>
</tr>
<tr>
<td>Long-term MBC</td>
<td>58%</td>
<td>36%</td>
<td>43%</td>
<td>30%</td>
<td>46%</td>
</tr>
<tr>
<td>Annuity: Spread payments over &gt;2 years</td>
<td>33%</td>
<td>14%</td>
<td>24%</td>
<td>20%</td>
<td>24%</td>
</tr>
<tr>
<td>Performance-based annuity: spread payment over &gt;2 years/tied to performance</td>
<td>47%</td>
<td>21%</td>
<td>35%</td>
<td>30%</td>
<td>35%</td>
</tr>
</tbody>
</table>

Note: Milestone-Based Contract (MBC): Pay for therapy upfront and receive refunds tied to performance over the short term (>2 years) or the long term (>2 years).

Table 2. Financing solutions of interest for future management of high-cost durable therapies.

2) Payers are motivated to manage the financial risk associated with durable one-time treatments differently, making this a high priority over the next two years.

In contrast to high-cost chronic treatments such as Spinraza (nusinersen/Biogen) or Onpattro (patisiran/Alnylam Pharmaceuticals), the majority of payers reported they were moderately, very, or extremely likely to change how they manage the financial risk associated with one-time, high-cost durable treatments (see Figure 2).

Payers were somewhat mixed regarding the importance of high upfront cost per patient and cumulative per member per month (PMPM) to their organization’s serious consideration of alternative approaches to manage financial risk of one-time, high-cost treatments. Thirty-nine percent rated both factors as equal. Thirty-one percent reported that high upfront therapy cost per patient is much or somewhat more important, while 26% reported that high total PMPM impact is much or somewhat more important. Neither factor would trigger serious consideration of an alternative payment model for 5% of those surveyed.
www.PharmExec.com features easy-to-use navigation with content available by targeted category, keyword search, or by issue. Fresh content supplied by Pharmaceutical Executive’s expert staff as well as external sources make PharmExec.com the source for comprehensive information and essential insight.
Implementing new management strategies for these therapies is a near-term objective. Fifty-seven percent of 77 payers surveyed expect to implement a new management strategy in the next one to two years, while 13% already have. Figuring out the best way to finance new high-cost durable therapies is a high or very high priority for 76% of payers surveyed, a medium priority for 21%, and a low priority for 4%.

New strategies that payers reported have already been implemented included value-based or outcome-based agreements and changes in provider contracting, including carving out payment for these treatments to a third party such as reinsur ance or risk pools and global case rate payments.

Payers expressed interest in multiple financing approaches for the future. Overall, payers were most interested in short-term milestone-based contracts, defined as contracts of less than two years duration where the therapy is paid for upfront and the plan receives refunds tied to performance (see Table 2 on page 26).

Self-insured employers were most interested in population risk-pooling, stop-loss/reinsurance over the broader population. Installment payments, annuities without a performance component, were the least appealing, with only one-third of commercial plans to a low of one-seventh (14%) of Medicaid respondents expressing interest.

3) Addressing contract terms and barriers will matter

Payers see multiple benefits of alternative financing approaches:

- Reducing upfront budget impact of the new therapy by smoothing payments over time
- Aligning the timing of the therapy costs with its benefits
- Only paying for therapy that works by including performance-based requirements for initial or continued payment

Of the three, payers see the most benefit in paying for what works, with 83% identifying this factor as extremely or very beneficial (see Figure 3).

The majority of payers identified a number of elements of multi-year, performance-based agreements as very important or a deal-breaker. These include:

- The inclusion of performance-based requirements for payment (72%).
- Termination of payment obligation with the death of the patient (67%).
- Access to data on specific measures (66%).
- Term: The number of years over which the payouts are stretched (65%).
- Ability to track performance even if the patient has left the plan (62%).

Finally, payers rated a number of barriers to alternative financing approaches (see Figure 4 on facing page). Using the two top levels of importance—extremely important and very important—payers identified operational, strategic, and structural barriers.

The top-rated operational barriers reported were:

- Program administration complexity (83%).
- Identification of appropriate milestone measures (81%).
- Information burden for tracking patients and providing relevant data (79%).

The top-rated strategic barriers were:

- Paying for patients who are no longer insured by the plan (77%).
- Paying for patients who are no longer responding to therapy (76%).

The top-rated structural barriers were:

- Uncertainty in cost accounting for multi-year agreements (71%).
- Pricing and reporting regulations (e.g., Medicaid best price) (59%).
- Insurance regulatory barriers (e.g., minimum reserve requirements) (57%).
- CMS regulatory burden (56%).

Implications

The FOCUS II survey shows payers awareness and concerns have grown since FOCUS I in 2017 regarding the impact of high-cost durable, potentially curative ther-
thepies with one-time administration. Compared with the prior FoCUS survey and the 2017 ARM/NAMCP survey that reported just over 10% of medical directors had started to consider coverage or operational issues associated with these treatments,3 payers now have a sense of urgency. This level of concern has increased over time as more treatments have entered the market and clarity regarding the pipeline and likely approvals over time has grown.5,6

Payer concerns were consistent with the financing challenges FoCUS has previously identified,7 namely actuarial risk, therapeutic performance risk, and payment timing relative to benefit gained. In addition, payers emphasized the impact of the total cost of treatment, both for an individual patient and the burden of multiple high-cost therapies.

As a result, payers have already started to manage these treatments using many of the current management strategies they currently employ for other high-cost treatments. And payers are interested in managing the financial risk and impact associated with these one-time curative therapies differently. Payers are open to multiple different approaches, although most favor short-term milestone-based contracts where therapy is paid for upfront and potential refunds and likely approvals over time has grown.5,6

Figure 4. Payer barriers to alternative financing, by importance (n=77 payers).

Those actions will face a number of barriers. Payers will need to work through administrative issues and strategizing to facilitate alternative financing approaches. Of somewhat less importance to the plan, but high interest to other stakeholders, are issues related to regulation. Flexibility in current price reporting, such as Medicaid best price, and other insurance regulatory and accounting requirements will need to be addressed at a structural, systems level to enable implementation of these innovative models.8

References


Transforming the CIO Role to Embrace Digital Health

Strategies for those chief information officers taking the digital reins at their organizations

As new and advancing digital technologies impact industries, chief information officers (CIOs) are under pressure to stay ahead of existing and emerging competitors. Digital health offers significant potential to enhance healthcare delivery and make medicines more personalized. This past decade, the rise of the digital economy has changed the role of the CIO. Change, however, is slow to come to healthcare as this industry lags most others in bringing about digital transformation.

“Digital Maturity is Paying Off,” a study by Boston Consulting Group (BCG), surveyed more than 1,900 companies in Europe and the US to estimate their digital maturity based on 37 dimensions. Of all the industries analyzed in this survey, healthcare had the largest share of laggards (43%). In healthcare, there is wide disparity, with medical technology companies showing the fewest laggards (10%), while 70% of biopharma companies have not embraced digital transformation.

Understanding how CIOs can overcome the challenges of building a digital health internet of things (IoT) infrastructure is essential for harnessing these technology advances in cost-effective and scalable manners.

The CIO evolution
Some CIOs still spend most of their time on legacy IT issues, leaving limited bandwidth for digital strategies. As the speed of business accelerates, CIOs are being asked to do more, faster. CIOs at top-performing companies are less focused on IT outcomes and costs, and more focused on business outcomes, revenues, and platforms.

Moving from cost-controlling to revenue-building is the biggest change for CIOs, allowing them to help drive product strategy. A BCG survey observed that companies transforming the CIO role increased market share by 7% while laggards saw their market share drop 11% from 2012 to 2017. CIOs are also working more closely with CEOs, offering authority to lead initiatives that change business.

As CIOs take the digital reins at their organizations, they must build the proper, scalable foundation to cost-effectively enable their digital health products. This requires CIOs to know when to build or buy supporting frameworks and how to best leverage internal resources to differentiate their digital products from the competition.

Top IT challenges
Healthcare’s transition from proprietary solutions to interoperable systems adds challenges to the process of getting partners to support integration with siloed platforms. The next-generation biopharma and medtech CIO will help organizations move from siloed platforms and one-off integrations, to leveraging scalable platforms that integrate with a variety of systems.

Companies that build their own custom digital health platforms in regulated industries frequently underestimate the ongoing costs, time, and maintenance these systems carry, including:

1. Growing security and privacy concerns. Developing digital products increases risks posed by security and privacy breaches. CIOs experienced in protecting IT infrastructures and data from external threats, are now also responsible for large sets of patients’ medical information.

2. Managing the complex regulatory burden. The FDA and similar regulatory agencies globally regulate biopharma digital health platforms and certain medical device software differently. In the US, for example, FDA has refined its position on regulating medical software at least eight times over the past two years.

3. Turning massive amounts of data into insights. Digital health platforms will exponentially increase the amount of data generated. The faster a CIO can draw insights from their platform’s data sets, the quicker they will transition from a cost center to a revenue enabler.

4. Scalability across regions, brands, and health IT systems. When aggregating data from tens of millions of users in real-time, and across multiple therapies and systems, scalability is a significant concern.

5. Cost of building and maintaining a digital health IoT platform. The total investment required to build a custom regulated digital

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health IoT platform, as opposed to using a pre-built platform, can hit $50 million, and the annual maintenance fees can range from $10 million to $20 million per single brand or product. Further, it can take around two years to build a new platform, delaying products and first-to-market advantage.

**Tips for tackling digital health**

Executives who get digital transformation right will be disciplined, agile, and pragmatic in their approach. These business leaders will act quickly to bring in early wins, while also carefully developing an innovation roadmap to build out the technical capabilities and resources needed to support digital efforts long-term.

Three recommendations have emerged for CIOs leading digital health at their companies:

1. **Engage the CEO.** Many IT departments lack the agility and specialized expertise to meet evolving needs, leading business units to hire chief digital officers (CDOs) to head their digital efforts.

   The most effective CDOs will be temporary leaders who complete their digital projects in three to five years before transitioning their responsibilities internally. At companies where the CIO leads digital initiatives, they should report directly to the CEO or be on the CEO’s executive leadership team. As an executive team member, the CIO can ensure leadership fully leverages IT’s expertise in digital technology and architecting platforms for success.

2. **Partner to build the digital health IoT platform.** Digitization has enabled “stack architecture,” which significantly reduces integration costs. Non-differentiating technologies like infrastructure, platform, and standard software packages are now readily available. Managed services can deliver quick wins in one key area, such as accelerating clinical trials or improving patient engagement. Rather than taking years, these initial projects demonstrate their initial value within weeks or months and could potentially help pay for longer-term digital transformation initiatives.

   Recognizing the evolving role of the CIO will help businesses harness digital health. Empowering CIOs enables these leaders to more effectively navigate biopharma organizations through this transition.

3. **Quick wins fuel growth.** Digital products will unlock new insights and revenue streams that are hard to predict from the outset. These initial projects should be focused on discreet, rapid digitization efforts that can deliver quick wins in one key area, such as accelerating clinical trials or improving patient engagement. Rather than taking years, these initial projects demonstrate their initial value within weeks or months and could potentially help pay for longer-term digital transformation initiatives.

   Recognizing the evolving role of the CIO will help businesses harness digital health. Empowering CIOs enables these leaders to more effectively navigate biopharma organizations through this transition.

**References**

Although dwarfed in size by some of the biggest economies in Europe with which it shares its borders, the Czech Republic is one of the Central and Eastern European (CEE) region’s best-performing economies. Indeed, on a per capita level, the Czechs are well ahead of their CEE neighbors, with GDP (PPP) per capita standing at USD 39,337 in 2019. Moreover, the Czechs can proudly boast one of the best healthcare systems in the CEE; despite healthcare expenditure as a percentage of GDP standing at 7.2 percent compared to an EU average of 9.8 percent. A universal healthcare system funded by seven insurance companies covers the healthcare needs of the vast majority of the country’s ten million citizens.

In the words of Janssen’s managing director, Martin Minarovič, the Czech healthcare system is “slowly progressing and converging with Western standards.” Minister of Health Adam Vojtěch agrees, stating that, “we are comparable to all EU members and can serve as an example to countries in the East.” Minister Vojtěch adds, “There are no barriers for patients in need of treatment.”

Against this backdrop stands a USD 3.38 billion domestic pharmaceutical market buoyed by regulatory reforms aimed at increasing patient access to innovative new therapies. Although still tightly regulated, industry stakeholders are cautiously optimistic that the Czech Republic stands to further close the gap with Western Europe.

The country’s diminutive size also belies its historical weight in scientific innovation. “The Czech Republic is the birthplace of medical inventions that have improved the lives of millions” exclaims Patrik Reichl, CEO of CzechInvest, the country’s investment agency. Two of the most notable discoveries include the first soft gel contact lenses, first produced in 1961 by Otto Wichterle, and the breakthrough antiretroviral drugs developed by pioneering Czech scientist Professor Antonín Holý which have revolutionized the treatment of HIV and hepatitis B. Holý’s invention is still considered the backbone of HIV therapy, and Gilead Sciences’ managing director Pavel Brezina believes “his achievements have not received the appreciation they deserve.”
AN OUNCE OF PREVENTION

Over recent years, the Czech healthcare system has undergone major advances in the treatment of certain therapeutic areas including oncology, cardiology, and diabetes, in line with Western European standards with an increased focus on preventative treatment.

The updated Czech Health Strategy 2030 aims to introduce more preventative measures, whilst focusing on difficulties in primary care and tackling the country’s lack of education and misinformation. Introduced at the start of 2020 by Minister Vojtěch states that, “the reform of primary healthcare will tackle prevention for non-communicable diseases, as the role of general practitioners is very important here.” He continues, “Better education will lead to better results in the area of prevention,” acknowledging that the Czech Republic has one of the lowest health literacy scores in Europe and concluding that “despite the obstacles that the system presents in certain areas, we should be proud of what we have.”

For example, in the past 15 years, the mortality rate of breast cancer has dropped from 43 to 29 percent thanks to the adoption of a rigorous breast screening program, a recent European Coordination Committee of the Radiological, Electromedical and Healthcare IT Industry (COCIR) study shows. In diabetes, it is mandatory for Czech citizens over the age of 45 to be evaluated by a GP every two years, which Jan Škrha, president of the Czech Diabetes Society, feels is a “positive step in the context of early diagnosis.”

A NEW ERA FOR ACCESS?

Pharma industry stakeholders are generally quick to suggest that the Czech Republic is one of the most over-regulated markets in Europe, with new or innovative therapies facing significant delays before being able to enter the market. “Compared to Slovenia, Slovakia or Hungary, the regulations involved in permitting access to innovative therapies in the Czech Republic are very complex” comments Janssen’s Minarovič.

For companies to overcome this barrier, they must first engage in a dialogue with the State Institute for Drug Control (SUKL). Emmanuelle Boishardy, general manager of GSK, agrees that “the market access process takes a long time, and it is difficult to predict the outcomes of assessments as SUKL lacks transparency.” However, Boishardy adds that “we can already see a lot of effort being made by SUKL.”

Ipsen’s VP for Central Eastern Europe Patrik Zachar also feels that there have been “significant improvements particularly over the last couple of years” in terms of the discussions that companies can engage in with SUKL, adding, “this has led to an improved and more predictable overall process with fewer bottlenecks.” Minarovič notes that “while the timelines are still outside the defined framework, the situation is much better than it was before and continues to improve further.”

Following approval from SUKL, companies must then negotiate with the seven insurance companies on an individual basis, as reimbursement is fully funded by them. The General Health Insurance Company (VZP) is the biggest, covering around 60 percent of citizens, while the other six share the remaining 40 percent.

Takeda’s Kieran Leahy, general manager for Czech Republic and Slovakia, explains, “the current health technology assessment (HTA) process was not built for the portfolios pharma companies have now. It was built to assess treatments for thousands of patients that cost tens of euros, such as cardiovascular or diabetes medicines. We now have treatments for tens of patients that cost thousands of euros.” Minarovič concurs, adding that “the main bottleneck is now the negotiation process with payers and budget caps.”

In response, VZP has committed to invest CZK 14 billion (USD 581 million) in modern treatments in 2020, a 12 percent year-on-year increase and 28 percent increase compared to two years ago. David Šmehlík, VZP’s deputy director emphasises “we want to improve access to modern treatments, thus increase investments every year in this area.” While insurance funds are increasing their budgets allocated to innovative therapies, the process remains difficult and lengthy.

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**TOP 10 PHARMA COMPANIES IN THE CZECH REPUBLIC**

<table>
<thead>
<tr>
<th>COMPANY</th>
<th>MAT OCT 2018 - OCT 2019 (USD MILLIONS)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. NOVARTIS</td>
<td>$251.4</td>
</tr>
<tr>
<td>2. SANOFI</td>
<td>$170.9</td>
</tr>
<tr>
<td>3. ROCHE</td>
<td>$149.8</td>
</tr>
<tr>
<td>4. PFIZER</td>
<td>$147.7</td>
</tr>
<tr>
<td>5. TEVA</td>
<td>$131.7</td>
</tr>
<tr>
<td>6. SERVIER</td>
<td>$128.6</td>
</tr>
<tr>
<td>7. JOHNSON &amp; JOHNSON</td>
<td>$102.5</td>
</tr>
<tr>
<td>8. ZENTIVA</td>
<td>$98.8</td>
</tr>
<tr>
<td>9. BAYER</td>
<td>$98.4</td>
</tr>
<tr>
<td>10. MERCK &amp; CO</td>
<td>$95.1</td>
</tr>
</tbody>
</table>

Source: IQVIA
BUILDING TRUST

This is not the final hurdle to bringing innovation to the patients, as Lilly’s general manager, Miha Kline explains. “The other half of the challenge is to establish trust with the physicians who will prescribe the treatment to eligible patients,” he notes. Ipsen’s Zachar agrees, as they have to work on two fronts: educating healthcare professionals (HCPs), key opinion leaders (KOLs), and specialists, while increasing the awareness of patients and their families about new or different treatment options.

Although there is still a long way to go in educating doctors, AstraZeneca’s Emelie Antoni believes communicating with HCPs on a one-to-one basis is easier in the Czech Republic than in Western Europe. “[Czech HCPs] very much welcome our sales representatives to learn from them about new science and knowledge. HCPs can receive tailored support and service. As a result, we still maintain quite a large field force, unlike in other markets” she states.

REFERENCE PRICING: HOW LOW CAN YOU GO?

The Czech Republic’s reference pricing system, where reimbursement is set based on the lowest price in the EU, adds another layer of complexity to the already lengthy market access struggle. Drugs sold in the Czech Republic are, on average, priced 30 percent lower than the EU average. Sanofi’s country chair Paul- François Cossa sees this as a severe limitation to Czech patients’ access to innovation. “Strong price pressures limit our room for maneuver in launching innovative therapies while creating value for the company, especially considering the complex business model pharma companies have to navigate,” he posits.

GSK’s Boishardy agrees that there is a balance that global firms have to consider. “Volumes in the small Czech market cannot compensate for a price decrease in France,” meaning...
One notable trend in the Czech Republic is the country’s dynamic channel shift in the OTC market, with the increasing consolidation and vertical integration of pharmacies, coupled with an emerging e-commerce and mass market. This has resulted in chains such as Dr. Max and BENU controlling an increasing percentage share of the market. Paul-François Cossa, country chair of Sanofi Czech Republic & Slovakia and general manager consumer healthcare (CHC), describes the Czech consumer healthcare market as “fascinating due to the level of maturity it displays compared to other European markets. The market has made major advances in e-commerce.”

Looking ahead, Sławek Ludwiczuk, country head for Bayer Consumer Health Czech Republic & Slovakia, is on a mission to transform the local affiliate to succeed in a consolidated market, reasoning “My mission was to be at the avant-garde of this transformation because I would rather be in the driver’s seat than simply a passenger” in terms of positioning their OTC brands on the market. He understands that changing the mentality of his team before opening dialogues with the stakeholders in this area is key, adding “as experts in our niche categories, we can bring insights to those partners. It is just a matter of finding intersections where we can jointly make our value propositions more relevant for the consumer.”

A cross-industry push for better and broader access to innovative treatments is underway in the Czech Republic. Ripe for reform is the country’s orphan drug legislation as no standard pathway for their assessment, pricing and reimbursement currently exists. Companies must apply for exceptional reimbursement under the ominous ‘Paragraph 16,’ which takes up considerable time and energy for local market access teams, with SUKL offering a simple “accept or reject” result. Roche’s general manager Robin Turner indicates that a change to this process “will be beneficial to increase access to orphan drugs for which it is almost impossible to demonstrate cost-effectiveness,” which is standard criteria for an approval from the regulatory body.
This ambitious reform, called the Act on Public Health Insurance, is set to become law in late 2020. “It is not just about cost-effectiveness” explains Minister Vojtěch, adding that “through this amendment insurance companies, industry associations, patient groups, and experts will evaluate how impactful a given orphan drug is and how it will improve the quality of life of patients.” Takeda’s Leahy adds “Our goals are aligned with those of the Minister of Health, which are for patients to have access to innovative life-changing medicines as quickly as possible in an affordable way.”

Increased dialogue between SUKL and insurance companies has enhanced patient access for highly innovative, but extremely costly CAR-T treatments. In 2019, VZP announced it had negotiated access to two breakthrough CAR-T therapies: Gilead’s Yescarta for large B-cell lymphoma and Novartis’ Kymriah for B-cell acute lymphoblastic leukemia. “We have special proceedings for early access to modern treatments such as cell and gene therapies and orphan drugs” shares VZP’s Šmehlík. To achieve this, VZP and the Czech Haematology Society signed a memorandum to grant funding to these therapies for the next twelve months, defined patients with a strong medical need for treatment and developed recommendations for both products.

Roche’s Turner agrees that for cancer treatment the country has been “quite successful in moving the needle, but we can do a lot better in small niche-indications.” He feels it is near impossible to gather enough clinical trial data, claiming “the whole model is not suited to the advent of personalized medicine based on genomic profiling.” If the Czech Republic wants to close the gap on Western Europe, “the country will have to change its approach to modern treatments.”

Turner quips, “the government is trying to fix the symptoms rather than treat the cause” and is less enthusiastic about other market access regulations for innovative pharmaceuti-
cals in general. Gilead’s Brezina agrees, sharing his opinion that “these so-called soft criteria that orphan drugs will be assessed against should be taken into consideration for regular prescription drugs as well.”

**GENERICS AND BIOSIMILARS: OPPORTUNITY KNOCKS**

Introducing expensive, innovative therapies weighs very highly on healthcare budgets. “Moving forward, health funds and budgets will not be able to continue paying for expensive innovation,” laments STADA’s executive director, Tomáš Mihál. This serves as an opportunity for generic companies to come to the fore. As Milan Černek, general manager of Mylan claims, “money saved through generics opens the door to the introduction of innovative therapies,” communicating that the country “needs to strive for a sustainable healthcare system that benefits patients and the community as a whole.”

Ingrid Šmerdová, a seasoned general manager at Adamed, a Polish company with a footprint in the Czech Republic since 2016, outlines “our mission is to respond to the key challenges of modern medicine” to add value to the healthcare system, explaining “the ultimate goal is to bring new possibilities of treatment to Czech patients and the whole healthcare environment.”

STADA’s Mihál also boasts of the benefits that biosimilars can bring to the country, boldly declaring “they can be the answer if health funds are looking for savings.” Many stakeholders agree that the increase use of biosimilars is inevitable, including the VZP’s Šmehlík, who says that as a result of introducing biosimilars, “patients being treated with modern medicines have increased faster than spending, thus the yearly cost of modern treatment per patient has decreased.” STADA aims to capitalize on this shift by launching eight biosimilars in the country over the coming years, and Šmerdová believes...
“our new pipeline will be a vital driver to differentiate the company locally.”

**A PRIME LOCATION**

Scratching below the surface, the Czech Republic boasts an infrastructure ready for the healthcare advances of tomorrow and a location right at the heart of Europe. Roche’s Robin Turner praises the country’s centers of excellence in oncology, adding “the more patients that can receive treatment in specialized centers, the better their chances are of survival.” These centers can also provide CAR-T therapies, having undergone rigorous certification procedures. “All this is done with the single aim to assure the highest quality standards which are critical for the best possible treatment outcomes for the patients” attests Gilead’s Brezina.

These specialized centers are also perfect sites for clinical trials, as Jakub Dvořáček, executive director of the Association of Innovative Pharmaceutical Industry (AIFP) numerates, “CZK 1.7 billion (USD 72 million) is invested in clinical trials per year, with 21,000 patients treated.” The Czech Republic is often picked for conducting clinical trials within the CEE Region, with the country contributing as many patients as some of the biggest countries in the EU. Novartis currently conducts more than 90 clinical trials in the country, with Janssen establishing a Global Clinical Operations department dedicated to this area. As Martin Puchwein, country president & general manager of market leader Novartis puts it, “this showcases the quality of Czech clinical centers and demonstrates the openness of physicians to work at the forefront of new therapies together with pharmaceutical companies.”

Moreover, multinationals continue to pin the Czech Republic, and Prague in particular, as the perfect destination for their Regional Hubs, supporting global or EMEA business operations. The capital is home to MSD’s IT Centre, Bayer’s Pharmacovigilance Hub, and Novartis’ Global Service Center, providing a wide array of services in areas such as finance, IT, HR and procurement. Puchwein feels that “the city has positioned itself as a technology hub, thanks to a combination of top universities, a great start-up ecosystem and big multinational players.”

**CLOSING THE GAP**

With a robust economy, a regulatory framework increasingly conducive to faster market access for innovative therapies, and a budding innovation and operational ecosystem, the future looks bright for Czech healthcare and life sciences. The main challenge moving forward will be for all key stakeholders to take a holistic view of the added value of innovative therapies, weighing up the cost from both a social and economic perspective. Takeda’s Leahy points out that “constructive dialog must be kept among all to make new medicinal technologies accessible.” Roche’s Turner adds that “Czechs should believe in their proven ability to succeed and invest in a truly healthy future.”

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Jakub Dvořáček, executive director, AIFP; Martin Puchwein, country president & CPO head Czech Republic, Novartis; Pavel Brezina, managing director Czech Republic & Slovakia, Gilead
In today’s hypercompetitive biopharmaceutical marketplace, prescribing behaviors are shifting rapidly, in line with fast-paced, market-moving events. A high-volume prescriber of a drug can quickly become a low-volume prescriber and vice versa. Unfortunately, too many biopharma segmentation and targeting plans are built for the more static industry of the past, basing plans on historical prescription volume. This leads to three big problems for brand marketers:

» Anemic brand launches.

» Inability to counter competitors’ launches.

» Flatlining of brand growth.

The key to success is to create targeting plans that get ahead of tomorrow’s prescriptions. But a backward-looking volumetric approach to targeting can inadvertently lead a brand team to overcommit resources to reaching historically high-volume prescribers, often at the expense of middle-tier or low-tier ones who may be on the cusp of becoming top prescribers in the near future. These emerging growers can represent a significant opportunity for a brand. The traditional approach similarly can’t help a team recognize when a prescriber is on the verge of switching a significant number of prescriptions for the company’s brand to a competing brand (emerging switchers). And it certainly can’t help a brand team identify those who don’t yet write the company’s drug but are ripe to be converted (emerging adopters).

If a brand team were able to identify these emerging growers, switchers, and adopters, it could use a custom mix of sales and marketing tactics to reach them with messages that resonate and convert these emerging brand opportunities. But this proactive effort is impossible when a brand team relies on historical prescription data trends to dictate its future targeting strategy. By overhauling the old framework and deploying sophisticated big data management, advanced analytics, and machine learning (ML) techniques, teams can uncover the hidden trends within large volumes of prescriber- and anonymized patient-level data, identify strategically important prescribers, and proactively increase the breadth and depth of prescriptions.

**Prescribing Patterns: How to Get Ahead of the Trends**

**Make targeting smarter**

ML can help a brand team discover underlying patterns within big data sets that aren’t easy to discern using traditional analytical approaches or simple rules-based algorithms. Armed with these insights, the team can identify the distinguishing characteristics of emerging growers, switchers, and adopters. From there, it can score and rank target HCPs or accounts within each category based on the likelihood of growth, switching, and adoption in the near-term, and create a dynamic target list that drives the call plan. After that, the team can craft and deploy tailored sales and marketing tactics and messages to engage with these targets and convert them.

One of the biggest impediments to successfully executing ML projects is poorly organized data. The brand team must create an organized repository of prescriber- and anonymized patient-level data and incorporate data on its current sales and marketing outreach. It should fold in prescriber attributes, prescription data for competing drugs, as well as relevant information about patients’ treatment journeys, where applicable.

Another step in implementing dynamic targeting is to measure its effectiveness. A small portion of the sales force can continue to use the traditional, volumetric-based targeting plan, while the majority of the sales team uses the new ML-based plan. The brand team can then compare sales results to measure and validate the effectiveness of its new approach.

**Embed into call plans**

Industry trends give biopharma companies little choice but to embrace ML-informed dynamic targeting. Some companies have already fully integrated ML-based insights into their call plans. Others operate in an intermediate stage on this path toward dynamic targeting. For example, a firm may still use a traditional, volume-based call plan, but also leverage simple rules-based algorithms to analyze recent prescribing trends and send time-sensitive alerts to reps regarding targets in their territories. But these algorithms and alerts can miss the underlying prescribing patterns and emerging trends that ML techniques would help the company identify. It can also be onerous for reps to change their call plans on short notice in response to these ad hoc alerts.

Companies in this intermediate stage should seek to push beyond it. A wholehearted embrace of ML-driven dynamic targeting as the basis of call plans will help them navigate a rapidly changing prescriber landscape with necessary agility.
More than half of your patients stop taking their prescribed medications within 3 months.†

Are you educating the health care team member who can influence adherence?

The pharmacist is key in helping 56% of patients adhere to their medication regimens.‡

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