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John Crowley, Chairman and CEO, Amicus Therapeutics
To overcome patient access hurdles

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Q&A: Pharm Exec Joins MJH Portfolio

Michael J. Hennessy Jr., president of MJH Associates, Inc., which recently acquired media assets, including Pharmaceutical Executive, from the UBM Life Sciences Group, answers questions about the acquisition and his company’s perspective on the evolving biopharma market and treatment landscape.

The acquired UBM Life Sciences assets have formed a new business unit within MJH called MultiMedia Healthcare, LLC. How do they fit into the overall MJH portfolio?

We recognized that combining the great content platforms and leadership brands that UBM Life Sciences has with our capability of developing multimedia platforms will leave no question that the new company will be at the forefront of providing access to trusted healthcare information now and into the future.

Your clients currently comprise world-leading pharmaceutical, medical device, diagnostic, and biotech companies. From your vantage point, what are the major challenges currently facing biopharma executives?

This is a time of extraordinary innovation, with the industry developing therapies that significantly increase survival or even cure diseases. However, these treatment advances often come with corresponding cost increases. The two chimeric antigen receptor (CAR) T-cell therapies, Kymriah and Yescarta, both have price tags above $400,000, and the gene therapy Luxturna, which treats inherited vision loss, costs more than $400,000 per eye.

The biggest challenge is getting payers to cover these life-saving and industry-altering treatments so that patients can actually access pharmaceutical innovations. A cure can’t do any good if the people who need it most cannot access it.

Alternative payment models and novel financing mechanisms will play a role here, but these are largely in nascent stages of development. For instance, with the CAR T-cell therapies, reimbursement remains a challenge. We speak regularly with industry experts who tell us how the low reimbursement rate from CMS means medical centers are losing over $100,000 per patient when they treat with CAR T therapies. Obviously, that’s not sustainable, and without a solution it will mean not every patient whose life could be saved will have access to CAR T therapy.

We’re not just in the business of reporting on the problem; we bring together clinical, payer, and policy leaders to find solutions.

Many of the MJH brands cover specific therapeutic areas and are directed toward physicians. How is the physician landscape changing? What specific changes affect pharmaceutical executives?

The overall treatment landscape has been evolving very rapidly in the past few years. Physicians want access to trusted and timely resources to help inform care and optimize outcomes. At MJH, we have a number of ways that physicians can get access to research and information. We do this through medical publishing, live events, market research, video and digital production, agency services, and medical education.

The pharmaceutical executive also has concerns around pharmacy, specialty pharmacies, and managed care, primarily as it relates to patient access to drugs. As you also cover these industries, what do current trends in the pharmacy benefit and managed care indicate for pharma?

Every stakeholder in healthcare has a focus on balancing use of effective treatments and therapies with the actual cost and the value of the outcomes produced. A trend we continue to see is that these stakeholders are still looking for ways to consistently identify and quantify value; the challenge is that value is more than just dollars, and in some cases are subjective and relative to a disease or even specific patients. When value is achieved and understood, access will follow.

Our approach and advice to manufacturers is to support stakeholders through education. Our platforms are designed to be a source of information to help stakeholders understand the value of care, as well as be a platform and mechanism to share best practices with others.

The information and education we provide is also directed to enable patients to be a part of the decision-making process, enabling them to be leaders in their own care and a partner with their healthcare providers. After all, access to care doesn’t ensure that patients utilize that care.

The MJH Healthcare Research & Analytics group recently issued a report titled “Physician and Institution Preparedness for CAR T-Cell Therapy.” What are some of the key takeaways that would interest pharma executives?

One is that oncologists are most concerned about the cost of the therapy for the patient. Pharma can benefit from providing more training and educational resources about CAR T-cell therapy. Additionally, oncologists would like to see the clinical trial data from pharma companies involved in CAR T-cell trials. In addition, work needs to be done to prepare and train hospital staff to be able to deliver CAR T therapy safely and effectively and have the infrastructure available for the data/analytics needed to build safe and effective CAR T programs.
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Amicus’ John Crowley: Living the Mission
Lisa Henderson, Editor-in-Chief

Pharm Exec interviews Amicus Therapeutics CEO John Crowley, the well-chronicled biotech executive famous for his quest to cure two of his children of Pompe disease, a rare genetic disorder. Here, Crowley discusses the growing role of the patient-centered view in company culture—not just in adding “deeper meaning” to drug development, but in building patient advocacy into everyday business practice and strategy.

Patient Advocacy

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By John Elliott

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Focus Reports, Sponsored Supplement

After a prolonged period of relative stagnation, the strategically influential, clinically rich French life sciences market suddenly seems to be rediscovering its mojo—thrusting the pharma and medtech sectors into the spotlight.
For those readers who are not aware of the Alliance for a Stronger FDA (www.strengthenfda.org), it works to ensure annual appropriations that will adequately fund the FDA’s essential missions, and believes that the American people expect this too. There is no other agency that performs this critical work. The strength of the alliance is that it brings together organizations, groups, and individuals with a wide variety of priorities to advocate for a science-based, predictable, efficient, and appropriately funded FDA.

Anonymous

“Government Shutdown to Have Repercussions for Months to Come”
bit.ly/2H4ki8Q
What Does Patient Centricity Look Like?

FOR PEOPLE WITH NO EXPERIENCE IN PHARMA, it seems very odd that an industry that makes drugs and therapies that treat, cure, or relieve the symptoms of diseases in people actually may not have a “patient-first” view. Observers don’t see the doctor as a gatekeeper to a prescription, or a pharmacy benefit management company as a drug controller, or even laws and regulations that ensure undue influence is not a factor when a patient is educated about a drug. However, those factors do create a history and a muddiness in reaching out to patients as a true “consumer.” But what is more apparent in the past 10 or so years, is that a patient-first mentality incorporated into a biopharmaceutical organization can create a beneficial environment for both the patient and the company.

“It’s one thing to say you are patient centric but how do you actually live it?” That question is posed by John Crowley, chairman and CEO of Amicus Therapeutics, in this month’s cover executive profile. Crowley answers with how the patient-centric processes is incorporated into Amicus, which operates in the rare disease space, Crowley’s philosophies are informed through his life and career path, starting a biotech company that researched and developed drugs for Pompe disease, a protein enzyme deficiency that results in muscular degeneration. It’s the disease two of his children were diagnosed with in 1998. Now Crowley is at Amicus, with a recently launched therapy, Galafold, for another rare disorder, Fabry disease.

What is clear from Crowley’s interview is that incorporating the patient view is life changing for both a company’s employees and the patients it serves. In the article on page 15, based on survey results from biopharma professionals responsible for “patient” functions, it finds that employees have greater job satisfaction and appreciation for their work when they know their work positively affects others. Patient services providers, who operate “call centers” or hub services for patients, are bringing more patients and their stories into their daily interactions and as a result feel better about their jobs and more connected to their patients (view at bit.ly/2NytCTm).

There are barriers to patient-centric success. One is C-suite buy-in and support. Many patient professionals feel that their role is difficult to quantify because of a lack of accountability or clear metrics from executives and senior management. As this function has grown, and as the survey article shows, many feel that training employees in biopharma on patient centricity is lacking. In fact, 69% are struggling with what and how to teach patient centricity to employees.

These barriers are being tackled by The Aurora Project benchmarking project mentioned, as well as a DIA working group investigating the patient and their role in pharma. Amicus Chief Patient Advocate Jayne Gershkowitz helped cofound a nonprofit organization, Professional Patient Advocates in Life Sciences (PPALS, ppals.org), which, in conjunction with Sanford Research Institute, created the Patient Advocacy Certificate Training (PACT) course of study “for health and life science professionals and leaders of patient advocacy organizations to enhance their professional development.” Gershkowitz notes in our article on page 20, “It’s about validating the arrival of patient advocacy in life sciences.”

Incorporating the patient view is life-changing for both a company’s employees and the patients it serves.
Multiple Pharma Issues Top Congressional Agenda

Wide-ranging probes to target marketing, pricing, and access

Both Republican and Democratic committee chairs will be busy in the coming months with a broad range of investigations and hearings on topics that involve pharmaceutical marketing, pricing, access, and shortages.

Drug benefits will figure in the continuing debate about health reform and insurance coverage and cost, including the “Medicare for all” campaign. International trade policy deliberations will raise questions about extended patents for biologics. Medical device makers have renewed their campaign to permanently repeal the 2.3% device excise tax created by the Affordable Care Act (ACA). And FDA is pressing for legislation to reform how it regulates over-the-counter drugs, including new user fees to expand agency resources in this area. While many Democratic proposals approved by the House will fall by the wayside in the Republican-controlled Senate, both parties are eager to address public concerns about access to affordable health care and prescription medicines.

The opioid epidemic will be a continuing focus for the legislators. House Democrats are expected to continue Republican investigations on opioid production and distribution, including the rise in fatal overdoses from fentanyl and other synthetic opioids. At the same time, the concerns of patients in need of pain therapies will be aired, as seen at a recent hearing before the Senate Health, Education, Labor and Pensions (HELP) committee, which invited patients suffering from chronic pain to testify on how initiatives to curb opioid supply may harm many individuals.

The recent measles outbreak also has drawn attention. The House Energy and Commerce subcommittee on Oversight and Investigations held a hearing on how the anti-vaccination trend was leading to the reemergence of this and other preventative diseases. Similarly, the Senate HELP panel heard testimony recently on how vaccines save lives. FDA policies and programs will be examined, as seen in a query from leaders of the House E&C panel on the agency’s ability to adequately inspect and detect safety issues at foreign drug manufacturers. The concern arises from the discovery of a contaminated ingredient in common blood pressure medicines, which has led to extensive recalls.

Spotlight on drug pricing

The really hot topic for legislators on both sides of the aisle, though, is cost and access to prescription drugs. House and Senate committees wasted no time scheduling hearings on drug pricing, including Trump administration proposals to change how Medicare pays for drugs, establish some kind of reference pricing system, and alter industry-payer negotiation of rebates and discounts. The House Ways & Means Committee, the Senate Finance Committee, and the House Oversight and Reform Committee have all held sessions to hear from experts and patients on proposals to revise drug rebates, permit drug importation from Canada (and elsewhere), modify curbs on Medicare Part D price negotiations, reduce barriers to competition from generic drugs, and set international reference pricing standards. A continuing focus will be stiff hikes in the cost of insulin and other diabetes treatments.

Senate Finance Committee chair Chuck Grassley (R-Iowa) and ranking Democrat Ron Wyden (D-Ore) are setting an aggressive pace. In February, the panel invited leading pharma CEOs to testify on drug marketing and pricing issues, particularly on rationales for highly publicized January price hikes by Pfizer, Merck & Co., Bristol-Myers Squibb, Sanofi, and AstraZeneca. Grassley has long challenged “pay-for-delay” deals between innovators and generics, and plans a major push to further curb such arrangements. And Wyden has introduced a handful of bills that require pharma companies to justify publicly list price increases beyond established benchmarks, to provide greater transparency in rebates and discounts negotiated with pharmacy benefit managers (PBMs), and to reduce the costs that hit Medicare beneficiaries under catastrophic coverage.

But everyone is looking for real solutions and not more finger-pointing. At a Ways & Means Committee hearing in February, chairman Richard Neal (D-Mass) complained that “drug companies point to the PBMs, who point to the insurance companies, who point to the hospitals,” noting that patients are the victims as a result.
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Parallel Trade a Victim of Brexit Collateral Damage

Despite a “temporary fix” at the ready, pharma supply repercussions loom if exit deal isn’t reached this month

A lesser-noticed side effect of Brexit is its impact on parallel trade in medicines. Lesser-noticed, and perhaps lesser-lamented in the drug industry, since most pharmaceutical manufacturers have long opposed the very concept of agile middlemen extracting value from the price differences across Europe’s patchwork quilt of distinct systems, divergent prices, but united in a single market.

Of course, parallel trade will continue as before across the borders of the 27 remaining member states after Brexit. But parallel business across the Channel is going to face more than 19 miles of salt water as a barrier—because the principle of free circulation of goods in a single market falls an obvious victim as soon as a country drops out of the European Union (EU). And that is going to have repercussions on both sides.

‘No-deal’ scenario

On the (highly likely) assumption that the UK leaves the EU on March 29, the disruption is likely to be all the greater, since the departing country has for decades been a happy hunting ground for parallel traders, who have been energetically supplying and sourcing products there.

A joint statement in February from the European Commission and the European Medicines Agency (EMA) says unequivocally that in the event of a “no-deal” Brexit, which was still a possibility as of press time, “parallel trade of medicines sourced in the UK is, in practice, no longer possible as of the withdrawal date.”

Parallel trade of medicinal products in the internal market is possible in particular because of two factors, the statement explains. One is that trademark rights are “exhausted”—that is, they no longer provide grounds for opposing supply, once the rights holder places the product anywhere in the internal market. The other is that the summary of product characteristics and the labeling of medicinal products have to be identical (apart from issues of language used).

But after UK withdrawal, neither of these conditions will obtain; the rules for exhaustion of trademark rights will no longer apply to products placed on the UK market, and the terms of the marketing authorization will differ.

The consequence is an immediate stop to around €600 million worth of medicines that are currently exported from the UK to the EU every year.

But they can say this only because the UK focus is limited to medicines flowing in the other direction, that are imported into the UK from the EU—a trade estimated to be worth about £1 billion a year. The UK says it can choose whether or not to allow parallel imports; citing the World Trade Organization’s 1994 Agreement on Trade-Related Aspects of Intellectual Property Rights (better known by its acronym of TRIPS), it claims it is entitled to choose what rules govern exhaustion of trademark rights.

The UK has decided on what it calls a “temporary fix.” “If there’s no deal,’ the UK will unilaterally align to the EU exhaustion regime from exit day, to provide continuity in the immediate term for businesses and consumers and ensure that parallel imports of goods, such as pharmaceuticals, can continue from the EU,” it says.

Meanwhile, “We’re currently considering all options for how the exhaustion regime should operate after this temporary fix,” and “any substantial changes to the exhaustion regime will occur only after a full research program and consultation.”

The UK will also juggle the authorizations so that the other condition is satisfied, and it will unilaterally provide a soft regime at least until 2020.

Export issues

That appears, for the moment, to satisfy the UK government—which, of course, benefits from parallel importing from the EU, to the extent that it can reduce the costs of UK drug supply. As the UK acknowledges in its January guidance, “Parallel import
of medicinal products is an important route of supply for medicinal products in the United Kingdom and provides cost savings to the NHS across the UK, as well as alleviating supply issues.”

What it does not resolve is the impasse for trade in parallel exports out of the UK and into the EU. Nor does the EU give much sign of wanting to help out there either. It will allow duly authorized UK-sourced medicines brought into the EU27 before withdrawal to circulate on the EU27 market.

But the guidance makes crystal clear that EU law “does not cover export or import of the product from third countries” and the UK will be a third country, as from its withdrawal, with or without a deal. “Moreover, as of the withdrawal date, central marketing authorizations (that is, issued after an EMA procedure) cease to be valid in the UK.”

So as of the withdrawal date, the EU rules “no longer apply to medicinal products sourced in the UK for the purpose of parallel distribution in EU27.”

Industry worries
The UK drug industry’s main lobby group, the Association of the British Pharmaceutical Industry (ABPI), has repeatedly pointed out how seriously European drug supply is threatened by Brexit—particularly since around 40 million patient packs go from the UK to the EU every month, and some 2,300 authorizations, representing around 350 products, are held by UK-based companies. In addition, there are valuable medicines that are manufactured only in the UK.

ABPI’s main concern is the interests of its membership among drug manufacturers, not parallel traders. But what it argues about drug supply is valid for all trade, whether it is direct or parallel.

“The regulatory arrangements for medicines and medical devices are complex and changes if no solution for medicines and medical devices is found during the Brexit negotiations,” it went on.

The bottom line is that—irrespective of anyone’s views on the desirability of parallel trade—Brexit could be about to deliver a blow to what has been a flourishing supply of parallel imports out of the UK and into the EU.

The bottom line is that Brexit could be about to deliver a blow to what has been a flourishing supply of parallel imports out of the UK and into the EU. Parallel traders’ sufferings may not provoke much sympathy. Nor perhaps will the cut in adventitious profits that some wholesalers or pharmacists in the EU will experience. But reduced supplies out of the UK could well mean that patients in Europe—and to some extent the organizations that pay less for parallel-traded medicines—find they are innocent victims of Brexit.
Living the Mission

Amicus Therapeutics CEO John Crowley, whose biotech journey and quest to cure two of his kids of a rare genetic disease is well chronicled, discusses the growing role of the patient-centered view in company culture—not just in adding “deeper meaning” to drug development, but in building patient advocacy into everyday business practice and strategy.

By Lisa Henderson

In 1998, John Crowley, chairman and CEO of Amicus Therapeutics, was one year out of business school, in a management consulting position on the West Coast, with a pile of student loans and three young children, when his two youngest children were diagnosed with a rare genetic disorder known as Pompe disease. The condition occurs when there is not enough or any of the enzyme alpha-glucosidase, which breaks down complex sugars in the body. Without the enzyme, sugars build up in the muscles, causing them to break down.

Crowley’s life in biotech is well documented in articles; television appearances; books, including The Cure by Pulitzer Prize-winning author Geeta Anand and Crowley’s memoir Chasing Miracles; and the movie Extraordinary Measures. Starring Brendan Fraser, Harrison Ford, and Keri Russell, the film depicted Crowley and his wife Aileen’s path to find a cure for Pompe disease. With Crowley’s life and lifework very much publicly available, the following interview doesn’t delve as much into the personal aspects—but, rather, how the executive’s personal life and professional path are instructive to the philosophies he incorporates into his business practices, especially around patients and patient centricity.

PE: Your first job in pharma was with Bristol-Myers Squibb. How did that experience affect your future path as a biotech entrepreneur?

CROWLEY: I was a year out of business school and working at a consulting firm and just trying to keep my head above water on the student loans. I went to BMS because we were living on the West Coast at the time, and we wanted to be back East to be close to family, expert doctors, and a job with a company that had good benefits. BMS happened to be the right opportunity. I never went into this thinking, “I’ll do this for a year or two, learn everything I can...
and go start my own company.” That just evolved two years later, when we became frustrated with the pace of development, and time was as much the enemy as nature—and the mistakes of nature.

My first job [in pharma] was a director of area marketing position for the neuroscience and infectious disease franchise for BMS. Then I was director of business strategy for the US pharma business. Those were my two primary roles there and they had absolutely nothing to do with how to run a biotech company.

PE: Coming from your management consulting position, what were your initial thoughts around the pharmaceutical industry?

CROWLEY: It really was a whole new world. I remember going to a meeting at BMS my second day on the job and hearing all these acronyms and the different way people spoke. On the one hand it was exciting, but it was also overwhelming because I was not trained in any science background. I thought I would just be the “business guy” doing marketing plans. I realized pretty quickly that I needed to understand the biology and the science. And in this case initially, it was neuroscience medicine.

PE: It is a more common practice now for pharma professionals to go to biotech, but you made that switch 20 years ago when it wasn’t as common. What do you remember most about being in a startup environment and how that was different from traditional pharma?

CROWLEY: I had a very sharp break from pharma to biotech. I went from one of the most established, well-respected and well-run pharmaceutical companies in the world, with an incredibly deep bench, to literally starting what was then a four-person biotechnology company (Novazyme Pharmaceuticals) based in Oklahoma City that didn’t even have science data in animals; we didn’t have preclinical data. It was as startup as you can get. We didn’t have offices, we didn’t have a bank account.

I didn’t go right out to the Novazyme lab space in Oklahoma, I went to the BIO Convention. This was when the whole genomics phase of biotech was in full swing, the height of the market, and, arguably, the first bubble in biotech. I remember going to that convention, there must have been 15,000-plus people, and it was vastly different from anything I had seen in my pharma experience, which was much more structured, much more controlled.

But in biotech, there was a palpable sense of excitement around the science and the medicine. We really felt then we were just beginning the golden age of medicine with the human genome being decoded, with people increasingly understanding genomics and proteins.

I remember going to the keynote address that was given by Christopher Reeve. He came out on the stage and he was ventilator-dependent and quadriplegic, and the first words he said in that speech were, “Biotechnology. It’s a great big word that for people like me just means hope. It’s hope that one day there will be a treatment for paralysis and I can walk down the beach and hold my wife’s hand again or throw a ball to my kids. But it’s also hope for people who don’t know that they need hope today. It’s the kid that’s going to break his neck in a game next week. And his whole life in an instant and his family’s and his community’s will change.”

For me, this was an incredibly exciting time to come into biotechnology.

PE: Can you elaborate on your experiences with researchers and the science over the years? We see that researchers don’t always meet with the patients who have the disease they are studying, and they also meet with a lot of failures.

CROWLEY: I felt it was incredibly important that the people that worked at Novazyme, and also at Genzyme (which acquired Novazyme in 2001) and now at Amicus, understand what it’s like to live with a disease, and understand a family’s journey. I went to our science founder in our company those first couple of weeks and I said I want to bring in some families to talk to the employees; we were hiring a lot of young scientists at Oklahoma City at the time, and he agreed. I wanted to bring in a Pompe family,

» John Crowley graduated with a BS in Foreign Service from Georgetown University, and earned a JD from the University of Notre Dame Law School and an MBA from Harvard.

» The New Jersey native served as a commissioned officer in the US Navy Reserve (2005-2016), assigned to the United States Special Operations Command, and is a veteran of the global war on terrorism, with service in Afghanistan.

» In August 2018, the FDA approved Amicus’ Galafold, the first oral therapy to treat Fabry disease. The drug is already approved in seven other markets, including Europe, Japan, Australia, and Canada.
I told the employees, I’m glad we did this program; I feel terrible that it failed, but we would rather be the first to fail than the last to succeed

— JOHN CROWLEY

where we were focused, and we found one young lady, but she was too sick to come in at the time. So we found a family with two boys who had mucopolysaccharide II (MPS II) disease and had them come; it was eye-opening. Dr. Hung Do was the first scientist I hired at Novazyme, and is now chief science officer at Amicus; when I hired [Hung] he was right out of his post-doc, with a PhD in glycobiology, had studied at the world’s best universities, but had never met a patient living with any of these diseases until that day. It really changed his perspective.

It’s a tough balance. We’ve now done this well over a hundred times at Amicus, and have families come in and describe their journey. We do these as monthly lunch-and-learns here at Amicus, and we did them weekly at Novazyme and at Genzyme. They are emotional, educational, and inspiring. And they are a two-way street. Principally, it is for our company and our employees to provide them deeper meaning to the work they are doing and also put that work into context. And for them to derive some level of inspiration to work maybe a little more creatively or a little harder on those tough days when you face setbacks.

But for the families coming in, we provide some tangible experience on what it takes to develop medicines. We give them tours of our labs, and we sit down with them in roundtables. Some of them are incredibly knowledgeable and are already running their own foundations or organizations, and others are new to these discussions. We gear the discussions toward however helpful it is for the families. These have been among the most powerful experiences in business, and in life.

CROWLEY: Working with patient organizations and working closely with patient families, it’s the right thing to do and I think it provides deeper meaning to our work. But it’s also going to make Amicus a better company. I have always believed that to have that patient-centered view, you need to build it into every aspect of your business and your decision-making. If you do that, it will become a strategic advantage and you will make better medicines and get them to more people more quickly, which is fundamentally how we build businesses in biotechnology and in pharmaceuticals.

It’s one thing to say you are patient centric, but how do you actually live it? We, early on, built a team of patient advocates. Jayne Gershkowitz (chief patient advocate, Amicus) was one of my first hires into the company because I knew she had the experience and perspective and passion to make us a better organization and to build that patient advocacy function (see article on page 20). I told Jayne from day one that she, above all others, needed to be the conscience of the company and always to remind me and remind us—even though we may think we are living it every day—why we are doing what we are doing and ensure every decision we make is in the best interest of people living with these diseases.

For us, being patient centric means having a chief patient advocate, a C-suite level executive who reports directly to me and who sits on the senior leadership team and is involved in every aspect of the business. It is having patient advisory boards, and we have done this since day one; we have a scientific advisory board and medical advisory board for every disease we work in. But for every disease, we also have the patient advisory board—patients living with the disease or a parent of children living with the disease—and we meet them each a couple of times a year. They are under confidentiality agreements, we don’t pay them, but we share with them all of our confidential plans, programs, and data.

For example, when we went into preparing to enter the clinic with our new experimental medicine for Pompe disease, we first vetted all of the protocols for that study with the patient advisory board, then we took it to the medical advisory board, and then took it back to the patient advisory board.

It’s easy to say you’re patient centric when things are going well; it’s most challenging when things aren’t going well. We had a program we were developing for the worst genetic disease I’ve ever seen, a
rare skin disease called epidermolysis bullosa (EB), where because of a genetic defect, the outer layer of the skin can’t bind to the lower level, and it affects mainly children. It’s just horrible. It’s like living with third-degree burns your whole life.

The early clinical results were incredibly promising for the medicine we were developing, but in the fall of 2017, we unveiled the Phase III pivotal results, and it failed. It was not significantly different from placebo. By that point, Amicus had evolved. We had a great pipeline of medicines, a great team, and a lot of capital, so the company would survive the financial impact. But we came back to the company and had an all-employee meeting, which was probably the most emotional meeting we’ve had. Not because anybody was worried about Amicus; Amicus was going to be fine. But they worried about those kids living with the disease. I told the employees, I’m glad we did this program; I feel terrible that it failed, but we would rather be the first to fail than the last to succeed.

We regrouped and Jayne led the effort around how we can still help the EB patient community. One of the first things we did was reach out to a group of small entrepreneurial companies to share with them the data, all the clinical records and regulatory exchanges from the research process. We then met with the FDA to share everything we learned. The FDA actually held a patient-focused drug development forum six months later and changed the entire paradigm of development for drugs in that disease. A lot of actions we took after the failure were to make sure we never forgot the needs of that patient community. That program was part of the fabric of Amicus and always will be.

**Pe:** What types of professionals do you like to hire and surround yourself with?

**Crowley:** That’s simple. At Amicus, we hire passionate entrepreneurs. Those two words capture the people we seek to attract and develop as part of our team, part of our family. The passion part—they have to embrace this patient-centered focus. They have to embrace the emotional aspects of what we do. And, again, not everybody wants to do that, but that to me is an essential element. They have to be mission-focused.

The second part is they have to be entrepreneurs. We are now a 600-person company, we aren’t that small anymore. But I still want entrepreneurs to work here. When, God-willing, we are a 1,000 or 2,000-person company, I still want entrepreneurs—people who thrive in an uncertain environment, who roll up their sleeves and take responsibility for their direct work, who think differently.

This is a tough industry. No matter what we do—we can hire Nobel laureates, build the most magnificent research and manufacturing centers, invest hundreds of millions of dollars into science, and still almost everything we try, technically, doesn’t work. But when it does work, I still think it’s the best job you can have.

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**The Path to Patient-Focused Accountability**

Benchmark survey assesses the industry’s progress so far in moving from words to action in its approach to patient centricity

**By John Elliott**

*They keep telling us we need to be patient centric, but I already am!*

Is this a familiar refrain from your biopharmaceutical colleagues when you talk about patient centricity?

For over a decade, pharma has been talking about the central role of patients as their *raison d’être*. Almost without exception, biopharma websites have some version of the line, “Improving patients’ lives are why we go to work each morning.” And so it should be. But how are we actually doing at delivering on those well-intended words to put patients at the center of everything we do?

As illustrated in survey results ahead, significant strides have been made, but there is also much room to improve. To move from words to actions, we need to understand in a more nuanced way how we’re doing. The following are the top six lessons from the 2018 Aurora Project Patient-Centric Benchmark Survey.

**1. Importance and confidence on the rise**

We asked how important it was that biopharma, biotech, and medical device companies delivered on their patient-focused missions as a way to understand how ready respondents were to deliver on the
promise of patient centricity. Ninety-one percent of respondents ranked importance an 8 or more out of 10—a 6% absolute increase from the 2017 survey (see Figure 1). We also asked how confident they were that they could deliver on those mission/visions. Although only three in 10 (30%) of the respondents ranked their confidence as 8/10 or more, that was up significantly from the previous year, where only 21% were as confident.

The key insight from the 2018 survey is—once again—the significant gap between how important patient centricity is for respondents versus how confident they are that they can deliver on their patient-centric visions. As will be discussed later in this article, our research suggests this is related to a lack of knowledge of how to be patient centered, and uncertainty over whether we have the will to be it.

Why this is important? Assessing importance/confidence can help determine readiness to change. In order to make meaningful change related to a given behavior, people need to feel like the behavior is worth changing (importance) and that they believe they can change it (confidence). As Gareth Phillips, managing director, UK, and head of EMEA at Ipsos Healthcare, notes: “It’s clear that biopharma wants and needs to be patient centric. While there was some improvement over the 2017 survey, respondents in the 2018 survey remain much less confident on how best to do that.”

2. How biopharma sees itself, how patients see biopharma

In order to understand how well the biopharma industry is serving patients, the Aurora Project Survey planning committee felt it was important to understand clearly what patients want. Guy Yeoman et al. addressed this well in their 2017 BMJ article entitled “Defining patient centricity with patients and caregivers: a collaborative endeavor.” Through a set of interviews and workshops with patients, the authors identified 10 principles that patients and caregivers expected of biopharma companies. These were then validated by over 1,000 patients.

In the 2018 Aurora Project Survey, we asked biopharma associates and patients the degree to which pharma is patient centered using these 10 principles (see Table 1 on facing page). Across those 10 metrics, patients consistently ranked biopharma companies lower than biopharma associates ranked themselves. On average, 57.5% of biopharma associates ranked their own companies a 4 or above on a 5-point scale where 5 was “strongly agree that they achieved each of the 10 standards.” By comparison, only 42% of patients ranked biopharma companies a 4 or above on the same metric.

Why this is important? Cognitive biases like the ones observed above are commonplace. People often rank themselves more positively than an objective observer would. As Dr. Lode Dewulf, chief patient officer, Servier group, and 2018 Aurora Survey co-lead, notes, “Classical cognitive biases are likely to drive the perception gap in the survey. Biopharma professionals may rate themselves more on intent; patients generally rate those companies on their actions—on what the patients actually see and experience.”

Despite the pervasiveness of these cognitive biases, highlighting them in this detailed way is instructive. Once we are aware of how we’re actually doing at serving patients’ needs through their eyes, we’ll understand more specifically what we need to do to close the gap.

Closing the gap requires an ideological leap for biopharma. Dewulf makes this observation: “There is a difference between working for patients and working with patients. Intent is reflected in
for, while with reflects action and method. Historically, pharma has always been working for patients with the best intent, similar to the HCP (healthcare professional). At the same time, patients were—and often still are—excluded from the table; decisions were made for them, without them. Patients want to have a say, they want with.”

There are two metrics in Table 1 where the gap is most pronounced:

» “Communicates with care and compassion, transparent and unbiased information on diseases, treatment options, and available resources.” (29% difference between biopharma and patients)

» “Listens and responds to patient feedback with respect and humility.” (26% difference between biopharma and patients)

Both of these metrics are characterized by qualities that are hard for companies to possess. These are human qualities—the skills and attitudes that must be delivered by company employees. Those people are distributed throughout many departments across the value chain from research to medical and commercial.

A frequent focus in biopharma is on value-added patient programs—which makes sense, but those alone won’t help humanize companies. Perhaps we should be asking ourselves, “What are we doing in biopharma to prepare our people to have the attitudes, empathy, and skills to meet these patient needs?”

3. Patient centricity drives engagement and pride

Seventy-six percent of biopharma associates are confident that their company is making the world a better place (see Figure 2 on page 18). Eighty-one percent of associates are proud to tell people outside the industry that they work in a pharma, biotech, or medical device company. Sixty-nine percent agree that “my customers would say that I help improve patient care.”

Why this is important? Historically, biopharma has engaged in patient-centric activities primarily as a means of delivering on its commitment to improve patient outcomes. However, the real win for all may be in its power to engage people within organizations. There is significant research that demonstrates that employee engagement has tangible value to companies and to the employees themselves.

Dan Pink, in his best-selling book, Drive, summarizes 50 years of research on what motivates us in the workplace. He identifies purpose as one of three keys to exceptional performance. The other two being autonomy and mastery.
activities. Sixty-seven percent of industry respondents agree that patients’ trust would be positively affected if patients secretly observed a typical day in a pharma company.

**Why this is important?** According to the 2018 Edelman Trust Barometer (ETB), trust in all institutions (governments, corporations, the media, and non-profit organizations) is at an all-time low. For biopharma, another telling finding from the ETB is that 82% of respondents believe that the industry needs more regulation. Moreover, lack of trust has tangible business impact, as it impedes our ability to work collaboratively with patients, healthcare providers, and governments.

Survey findings highlight a growing disconnect between how we feel about our own trustworthiness versus how patients perceive us. In essence, if a company doesn’t think it has a problem at a personal level, it’s less likely to allocate energy or corporate resources to make it better. Layered into this is a lack of training to address deeper cultural aspects of improving trust—beyond compliance initiatives—to ensure laws, rules, and regulations are respected.

Ultimately, closing the gap requires companies to make trust-building a daily habit for employees. Having a patient-centered mindset, where behavior is guided by the question, “Is this good for patients?” is what’s needed. Having patients as our focus connects us with the common mission of healthcare providers and institutions—and makes true partnership possible. It puts us on the same team.

### 5. Patient-centric strategies and business success

Seventy-three percent of patients and 85% of biopharma associates agree that focusing on patient need leads to better business outcomes. Ninety percent of all survey respondents agree that long-term focus is a key to success with patient-centric efforts. This need is sometimes at odds with business reality, with 53% of biopharma associates saying their companies are mostly concerned about results.

**Why this is important?** The idea that companies can’t meet both their financial needs and the needs of society is rapidly fading. The research on purpose-driven people and organizations provides plenty of evidence that being driven by purpose is key to exceptional performance.

“Much like technology a few decades ago, purpose has now become a business imperative,” says Aaron Hurst in *The Purpose Economy*.

Creating a purpose-based culture and earning the trust of patients and partners requires a longer-term focus than our business horizons usually permit. As Bjorn Gustafsson, global head of customer-facing excellence at UCB, notes: “Creating a purpose-based culture, and earning the trust of patients and partners, requires a constant and long-term focus.”

Another demonstration of the value of a patient-centric approach is in clinical trials. “The Innovation Imperative,” a report by the Economist Intelligence Unit, found that “…drugs developed using patient-centric designs were more likely to be launched compared to drugs developed without, with nearly 20 percentage point difference (87%) when compared to the control group (68%).” They also found that patient-focused trials took less time to recruit patients. Similarly, Levitan et al. made the case, in an economic analysis published in *Therapeutic Innovation & Regulatory Science*, that patient engagement in pre-Phase II and III trials could result in, “… gains in NPV (net present value) and ENPV (expected net present value) [that] can be several hundred times the investment for engagements that avoid an amendment and improve the patient experience.”

### 6. Training is the missing ingredient to execution

As shown in Table 2 (see facing page), 53% of biopharma associates responded in the survey: “We are actively looking for what and how to teach patient centricity to our people.” Only 22% said: “We know exactly what and how to teach patient centricity to our people.” Another 16% say: “We don’t know what or how to teach patient centricity to our people.” When you combine the “actively looking” group with the “we don’t know what or how to teach group,” we see that 69% are trying to figure out how they should train people to be patient centric.

**Why this is important?** When the finding that 30% ranked their confidence as 8/10 or more is...
juxtaposed with the finding that 69% of people don’t know what or how to teach patient centricity, one of the key insights from the survey emerges.

The low confidence is, in large part, a function of not knowing how to be patient-focused. This brings us back to the million-dollar question: How do we make patient centricity more than just words? It starts with, what does patient centricity mean to the pharma executive? While some think patient centricity belongs to a particular department in the organization, we believe that it needs to live in the culture of companies.

The foundation of being patient-focused is being purpose-driven. “Building a deep and authentic sense of purpose could be a company’s ultimate competitive advantage,” says Rick Wartzman, author of The End of Loyalty.

It’s time to take the vision off the wall and embed it in the hearts and hands of every single person in an organization. Gamze Yuceland, GM of Takeda Canada, agrees. “Instead of defining how our people should be patient-focused, we are empowering each of them to connect with their own purpose and define for themselves what being patient centric means to them,” she says. Yuceland believes employees will develop more and better ideas of how to be patient centric than the company could ever direct them to. And, further, they will be more motivated to act on those ideas.

In Alive at Work, acclaimed social psychologist Daniel Cable outlines the two key ingredients that help people become purpose-driven:

- Help people see the difference they are making,
- Help them develop their own story about the “why” of their work.

Some life science companies have had success with the first point. Whether it’s sharing patient stories, pictures on the walls, immersive opportunities, volunteer work with patient organizations, or virtual patient simulations, companies are starting to help their employees empathize with the patient. An increase in quantity and consistency of this exposure is definitely needed. As Doug Noland, head of patient experience at Astellas, says, “helping our people empathize with the patient experience has been a key focus for us and we see a difference.”

For many, the second purpose-driven ingredient remains elusive, particularly how one’s individual purpose connects to their organization’s purpose. Donahue runs a workshop called “The Power of Purpose” to help individuals make this connection. “People are transformed when they connect to their purpose: the patient,” says Donahue, “both in how they see themselves and how others see them. They become more committed, more passionate, more innovative, more engaged and engaging.”

Moving to patient-focused accountability benchmarks

The Aurora Project Patient-Centric Benchmarks Survey has been a great tool in helping pharma measure progress in patient centricity. The main limitation of the Aurora Project surveys to date has been selection bias: the possibility that those most interested in patient centricity might be more likely to complete the survey. In order to help the biopharma industry move from words to actions to outcomes, we need more robust and accountable data to know how we’re doing.

We believe that patient-centric accountability (PCA) is the next phase in the evolution of the patient-centricity movement in biopharma. PCA is the idea that biopharma companies deeply benchmark how they are doing at an organizational level with all associates—not just those with an interest in patient centricity. Those benchmarks would allow companies to cascade responsibility for patient-centric outcomes within their organizations that could be included, as an equal measure, with financial performance.

Just the beginning

While many in pharma feel we’ve already “checked the box” on patient centricity, the 2018 survey shows that we’re only at the beginning of the journey of moving beyond words. Patients can’t wait for us to put them authentically at the center of our value-creation models. They need companies to continue innovating—not just with medicines but with patient-focused business processes and people.

If you or your company are interested in being part of the next survey, please reach out to John Elliott at john.elliott@excellerate.ca.
The Corporate Mandate

Perspectives from big pharma and the rare disease space on how patient advocacy is evolving as a corporate function

By Julian Upton

The recent emergence of the chief patient officer or the chief patient advocate (or equivalent role) has seen pharma further acknowledge and coordinate its efforts to embrace the growing empowerment, sophistication, and influence of patient organizations across many disease areas, both common and rare. But while some companies are more advanced than others in embedding and optimizing patient advocacy within their corporate cultures, it remains a burgeoning function still making its way toward standardization and broad acceptance.

To explore how the function has evolved, both at large and smaller companies, and to see where it is heading in a landscape increasingly defined by patient centricity, Pharm Exec talked to three patient-advocacy leaders to trace their journeys through the industry and discuss the qualities and skills needed to succeed in their roles.

Communicating science

Dr. Joel Beetsch, vice president of patient advocacy at Celgene, has been involved with patient advocacy for around 12 years, but his career started at the bench—“relatively far away from the patient.” He trained as a cellular scientist and expected to continue with bench research when he joined the industry over 20 years ago. Until then, working in clinical departments in a hospital environment, he had seen patients “but never really felt close to them.” Moving into medical liaison and clinical trial roles, however, allowed him to edge closer to understanding their needs. Prior to joining Celgene, he was drawn to an advocacy role at Sanofi that bridged the gap between the company’s R&D activities and its corporate messaging, which led to patient-specific work in oncology and other areas. Now, in his role at Celgene, Beetsch explains, he “speaks with patients and advocates on a daily basis.”

Beetsch’s work revolves around establishing long-term partnerships with professional and patient organizations and working to better understand and identify the unmet needs of patients. Structurally, his responsibilities are multifold. His group engages in therapeutic-related outreach efforts to patient and professional organizations and coordinates all of the company’s community outreach efforts—in the Tri-state area of New York, New Jersey, and Connecticut, and at sites in Boston, Seattle, San Francisco, and San Diego—and is responsible for all donations, sponsorships, and granting mechanisms that fall outside of medical education. It also works with patient assistant foundations in the US.

Beetsch has observed the evolution of patient advocacy across the industry and within the advocacy community itself. Regarding the latter, he says, “the willingness and the desire to work with biopharmaceutical companies has increased certainly in the US, and there has been guidance measures and guard rails put in place across the world that have allowed us to do more of that outside the US.” On the industry side, “the focus has been to really understand the importance of engaging the patient and professional community in your everyday business all across the lifecycle, from early development to loss of exclusivity.”

Noting that Celgene was already very patient-focused and engaged with the advocacy community when he joined, Beetsch explains that his role has been “to build on that foundation, to help put a department in place, to demonstrate effectiveness within the patient community, and show those results that both reflect the benefit to the patient organization and to the company.”

Understanding the power of communities

Jessica Riviere, senior director of global patient advocacy at Biogen, started her journey toward the patient advocacy function working for Senator Ted Kennedy in the 1990s, during the Clinton health-care-reform era. “I was working with a lot of organizations and communities and I came to appreci-
ate and value the power of communities to make change and impact public policy and the greater good,” she told Pharm Exec.

Riviere points specifically to the HIV community, which at that time was a “trailblazer and really revolutionized patient advocacy.” She later worked again with the HIV community as director of advocacy and policy for HIV/AIDS and viral hepatitis at Bristol-Myers Squibb. “At lot of what we see in patient advocacy today came from the HIV community,” Riviere explains. “They were always super-focused on what their asks were when partnering with industry and other stakeholders. If you look now at advocacy in oncology and some rare diseases, for example, they’ve taken many pages out of the HIV action plan.”

On her Biogen appointment, Riviere says, “I think it was the first time that the company had hired a professional who had focused on advocacy to lead the team. It was a signal that the company valued patient advocacy and that it saw there was an opportunity to meet the needs we have and build a foundation for the future.”

As with Celgene, Biogen’s patient advocacy function is multi-faceted. Riviere is responsible for the overall strategy for currently marketed therapies and for those under drug discovery, and then throughout the product lifecycle. A key activity is “helping colleagues to understand the difference between an individual patient’s experience and working with a patient community,” she says. “Both are powerful, but it is the patient advocacy communities that shape and influence the environment in which the individual patient will have access to a physician, access to care and treatment, and will understand the types of services that are available to support them through their journey. Our role is working with patient advocacy groups to ensure that the drugs and many robust support programs we develop reflect the needs of the patient community.”

Rare diseases: Working at the grassroots level

Founded in 2002, Amicus Therapeutics (Cranbury, NJ) is a global biotech company focused on medicines for rare metabolic diseases. It has one globally commercialized drug for Fabry disease, an investigational enzyme replacement therapy in late-stage development for the treatment of Pompe disease, and a gene therapy pipeline for lysosomal storage disorders. The “patient-dedicated” company is headed by John Crowley, who himself became a parent advocate when two of his children were diagnosed with Pompe disease in 1998 (see Q&A on page 12). Crowley, then a BMS executive, went on to cofound Novazyme Pharmaceuticals, a biotech startup conducting research on a new experimental treatment for Pompe disease. Novazyme was acquired in 2001 by Genzyme Corporation (now Sanofi Genzyme), where Crowley continued to play a lead role in the development of a drug for Pompe disease and joined Amicus as Chairman and CEO in 2005.

Amicus’ chief patient advocate, Jayne Gershkowitz, joined the company soon after, having known Crowley for several years from the nonprofit work she did as the executive director of the National Tay-Sachs & Allied Diseases (NTSAD) Association, which serves the lysosomal storage disorder community. She describes her primary activity at Amicus as “setting the tone for the company culture of commitment and dedication to patients and their families, not only externally but internally, and ensuring that the patient perspective is incorporated across everything that we do.”

By being close to the patient community, providing programs and services and initiatives that help individuals as well as organizations to be better educated, she explains that Amicus aims to help patients to be more empowered and be their own best advocates. “It is also about being the conscience of the company,” she told Pharm Exec. “Keeping everybody mindful of why we do what we do and who we do it for.”

Patient advocacy in the rare disease space is “very grassroots,” Gershkowitz says. “In many cases, for example, it’s about parental advocates finding somebody who is interested enough to take on their child’s disease in the lab. There’s a very small number of both basic and clinical researchers, and not many companies involved on the industry side. So, partnerships naturally ensue. Without these multi-stakeholder partnerships, sometimes the work needed for rare diseases would not be able to move forward.”

Gershkowitz notes that patient & professional advocacy (P&PA) at Amicus has grown into something that is “a model for many other companies.”
Amicus has a global P&PA team of 11 people, which “is among the largest teams in biotech, if not the largest.” Among the team is a genetics nurse, an attorney with a public affairs background, a pharmacist, and a medical social worker, to name just a few. “They bring all different skills and experience,” Gershkowitz adds. “We work very hard at ensuring that we speak in a consistent way across all of our key audiences. Everything that goes into our early work, from our clinical trials to marketing and commercialization is informed by what we learn from patients, their caregivers, and communities.”

Advancing advocacy

Gershkowitz sees the value that patient advocacy has brought to the rare disease space moving increasingly into the common disease space: But there are still areas around the standardization of the function—and where it sits in an organization—where some of the bigger companies could take inspiration from their smaller, rare-disease-focused counterparts. At Celgene, like many drug-makers, patient advocacy sits within corporate affairs, but here it serves as a kind of shared-service function across all elements of the company, from R&D to late-stage commercialization.

“What I like about the structure we have,” says Beetsch, “is that we are shared amongst everybody and we occupy an elevated platform. The patient advocacy team is in high demand; we are very cross-functional within the team and across corporate affairs, and ultimately across the company.”

In other companies, however, patient advocacy may be more aligned with government affairs and public policy, medical affairs, community relations, or it may sit within the commercial business. And in terms of the standardization of the role, as Riviere points out, “there is still work to be done.” She explains: “We’re better than where we were 10 years ago, but I don’t think there is really any standardization. If you’ve seen one patient advocacy function at a company, you’ve seen one patient advocacy function at a company! It’s not like medical affairs or marketing, where every company has a variation on the same thing. You cannot say that about patient advocacy yet, so I am hoping that we move to a broader understanding of its value and impact.”

Beetsch acknowledges some of the challenges that larger companies may have. For example, he explains, “they can have two different types of patient advocacy organizations. One might be in a center of excellence within their R&D community that is really working to recruit clinical trials. Then there’s a separate function that is more associated with the business. Often, those two functions within the same company don’t communicate regularly with one another. At Celgene, we are fortunate that we are allowed to do all of that within one department—we have to talk to each other because we all work with each other.”

At Amicus, P&PA is positioned as an independent, product-agnostic function, reporting directly to the CEO and working directly with functions all across the company “right through to facilities, legal, and compliance.” Gershkowitz says: “We are structured as one cohesive team. Everyone is central; they may have their main area of focus but the work we do is both disease-specific and overarching. It made sense to become a standalone function because for a company to be truly patient-focused—that’s a term that I prefer to ‘patient centricity’—then patient advocacy deserves to sit at the table as any other strategic function.”

Gershkowitz states that the field of patient advocacy is burgeoning, however. She would like to see more companies embrace advocacy as an integral function, “so that it rises with even more corporate structures.” To that end, she has been active in providing more educational opportunities in advocacy. She helped to cofound a nonprofit organization, Professional Patient Advocates in Life Sciences (PPALS, ppals.org), which, in conjunction with Sanford Research Institute, created the Patient Advocacy Certificate Training (PACT) course of study “for health and life science professionals and leaders of patient advocacy organizations to enhance their professional development.” The program serves to “help people see advocacy as a career choice and to give them some grounding in it. It’s about validating the arrival of patient advocacy in life sciences,” says Gershkowitz. “We’re about to start our fourth year of face-to-
face courses, and we’re looking to develop a master’s program at an independent academic institution. We want to move this discipline forward and bring more people to the field. Historically, patient advocacy as a professional role has not been supported by a dedicated course of study. Now, it is.”

It’s important to help more people find advocacy as a career choice, adds Gershkowitz. “My team comes from many different professional experiences, and that’s a perfect example of how people come to advocacy. It’s circuitous. What we’re seeing now is people seeking out a career in patient advocacy, as opposed to coming to it via any number of different roles in medicine, healthcare, or corporate drug development.”

Riviere notes that advocacy is “no longer about saying, ‘Hey, I like working with patients.’ It’s no longer just the ‘feel-good’ role in a company. It’s a profession that requires a much more professional skill set.”

Key skills

So, what does it take to succeed in the pharma patient advocacy function? Beetsch says it is helpful to have customer management experience, along with some level of clinical or scientific background. “It’s always useful if people understand the science and can communicate in terms that patients and patient organizations can understand.” He also looks for people with experience working in the early pipeline space. “The reason for that is that we are working hard to put that patient voice into all elements of our development process.”

For Riviere, people working in patient advocacy roles need to be strategic, able to work with diverse communities and to listen to them, to build relationships over long periods of time, and to agree to disagree on certain issues and find common ground on others. “I look for people who are passionate, determined, and intuitive, who are forward-thinking and can look over a longer horizon,” she explains. “There is not a huge growth curve in patient advocacy roles; you’re not suddenly going to be the president of a biotech company if you started out in patient advocacy. These are roles that people often stay in for years, much like government affairs and public policy roles.”

“Clearly, empathy and creativity are essential,” says Gershkowitz. A key quality is the ability to strike a balance between flexibility and assertiveness, working with different communities that have distinct needs but also needs that are common to other rare diseases. “You have to understand where the patients and their families are coming from and how to incorporate what you’ve learned from them, because you’re helping to manage expectations around the drug development process.”

The road ahead

Pharma’s patient advocates are increasingly facing up to the challenges relating to disease awareness, access to innovation, and reimbursement that patient groups have been talking about for years. The ability to work side-by-side with patient organizations is essential to improving the industry’s efforts, says Beetsch. “They have the ear of governments, payers, and professional societies, which often the industry does not have. To be able, through forums like the European Patients’ Academy (EUPATI) and the Clinical Trials Transformation Initiative (CITI), to sit at the same tables as the patient community and learn from each other, learn how to do things better, is a great opportunity.”

While industry–patient groups’ relationships will continue to be scrutinized, pharma’s engagement with advocacy groups “is as beneficial to them as it is to us,” says Riviere. “We recognize their independence as organizations. There are plenty of places where we can partner to have a substantive impact, and other places where it is appropriate not to partner.”

Ultimately, says Gershkowitz, pharma’s patient advocacy function “is being increasingly recognized for the intrinsic value that it brings to the drug development process, regardless of where it is in the continuum.” She adds, “I’m looking forward to seeing more of that growth.”

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Patient Advocacy

Research Bonds Strengthen

Disease groups illustrate the growing advocacy-industry relationship

By Michelle Maskaly

In the past, the term “patient centricity” was sometimes brushed off as something pharmaceutical companies would say to sound good. Not these days.

“There has been much talk in the industry about putting the patient in the center, but for the first time this is really becoming a centerpiece for most pharmaceutical companies—the patient really comes first,” says Lynn O’Connor Vos, president and CEO of the Muscular Dystrophy Association (MDA). “It comes in as early as designing clinical trials. Many advocacy groups now come together with industry to say, ‘How early should we engage a patient in the discussion of a clinical trial?’ and ‘What kind of outcomes would be meaningful for you?’ We’re seeing in every phase of product development that there’s a role to engage patients, so I would say the role of patient advocacy is getting much stronger.”

Someone who has had a front row seat to this change is Alison Moore, founder and CEO of the Hereditary Neuropathy Foundation (HNF). She founded the group after learning of her sudden onset of Charcot-Marie-Tooth (CMT) disease, an inherited neurological disorder characterized by progressive loss of muscle tissue. “It’s very positive,” says Moore of the climate between drug manufacturers and patient advocacy groups. “More pharma companies are hiring advocacy directors to work with people like me. That’s a good sign.”

For Moore, the dynamic is quite different than when she first started reaching out to pharma companies years ago. All but one were not very interested in working with her. That one was the French biopharma Pharnext. On a whim, Moore emailed the company’s then-CEO in 2013, before leaving for a family vacation to Europe. To her surprise, he got right back to her and after some itinerary changes, Moore ended her vacation in France, talking with the Pharnext about her disease.

“I spent two days with these guys,” she recalls. “I knew a good amount already, but I learned so much … and they were excited to meet someone with CMT.”

That meeting began a long-standing, mutually beneficial professional relationship.

Two-way street

Patient advocates say the positive relationship that has been developing over the past few years between industry and advocacy groups is helpful to both parties, and, in turn, patients. In fact, this opportunity was a key reason Vos took the position with MDA.

“What’s remarkable about the Muscular Dystrophy Association and the neuromuscular medicine category is there have been so few therapies available for patients,” she says. “That is what attracted me to the MDA—the opportunity to build a greater partnership with pharma and biotech to help make sure diseases are better understood and characterized so that we can bring better therapies to market for our patients. We are helping pharmaceutical companies facilitate clinical research studies and recruit patients by gathering clinical and other key data sets.”

MDA is able to do this through its MOVR (NeuroMuscular ObserVational Research) initiative, a data platform that will combine clinical, patient, and genetic data all in one location for better collaboration among care centers and experts. The goal of MOVR is to help pharma design better trials, to ultimately create more effective products for MDA’s patient community, Vos says.

Making the connection

Vos and Moore point out that, in many cases, their groups serve as a middleman between the patients and the pharma company.

“Patient advocate organizations are the ones that can be the convener and connector to big pharma and patients to make sure the patients’ perspective is always being represented,” says Vos.

Both MDA and HNF have held events and programs that bring patients to the forefront. The 2019 MDA Clinical and Scientific Conference is set for April 13.

“This is the largest neuromuscular medical event that serves as the intersection of pharma, our care centers, our doctors, and our medical and scientific community,” says Vos. “We will be featuring technology companies, as well as pharma/biotech and other patient advocacy organizations. Our role is to be the convener and set the stage for a great dialogue, conversation, and sharing.”

For personal, non-commercial use
Historically, there has been a negative perception around the complexities and costs of developing drugs in Japan, which has seen many innovators excluding it from their strategy and has resulted in Japanese patients being prevented from accessing some of the most effective and safest treatments that have gained approval in other countries.

The Japanese government has been working hard to change this and has implemented several systems to reward innovation, resulting in a significant increase in the number of multi-region clinical trials including Japan—with growth at over 400% in the last 10 years.

This webinar will discuss some of the rewarding systems implemented by the government and regulatory considerations of developing in Japan, the second largest market in the world for branded pharmaceuticals.

Key take-aways:

- Overview and changes in the Japanese regulatory environment
- Regulatory considerations on international development strategy
- Incentives and rewarding schemes for drug developers in Japan

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For technical questions about this webinar, please contact Martha Devia at martha.devia@ubm.com
Pinpointing Performance of Key Access Activities

While access functions have evolved, the same can’t be said for measures used to gauge the contribution of these activities on overall business. To that end, an industry working group has developed a common framework focused on the most relevant access performance metrics (APMs).

Amid the ever-increasing healthcare funding challenge, securing patient access to medicines is becoming more and more complex. The access landscape continues to evolve shaped by three drivers:

- Increasing demand for healthcare, driven by aging societies and lifestyle choices that propagate chronic diseases, as well as improvements in diagnostic capabilities and the accessibility of treatments to broader sections of the population.
- Growing number of treatment options, as scientific innovation continues to provide healthcare systems with vastly improved treatment options and different treatment regimens (e.g., combinations, curative/gene therapy settings).
- Consequently, constraining healthcare are budgets that need to “buy more healthcare”—driven by both volume and cost increases, which lead payers to deploy further measures to optimize healthcare budgets.

Additionally, evolving regulatory pathways, as well as broader technology trends around data availability and analytics, are impacting the access landscape. Overall, achieving access is becoming an increasingly complex and cross-functional endeavor for pharmaceutical companies. And failing to secure patient access for a drug can have dramatic effects for patients and the manufacturers. A relentless focus on securing access to medicines is, therefore, becoming critical to the pharma business model. Access now concerns and impacts almost all levels of a pharma organization.

With access being increasingly core to the business, cross-functional involvement and prioritization becomes even more important. Consequently, the access function requires more sophistication in measuring the influence of access-related decisions. As the adage goes, “you can only manage what you measure,” hence, developing and using adequate access performance metrics (APMs) is key.

Convening power

The Boston Consulting Group (BCG) Market Access Roundtable is a forum that brings together senior market access leaders and serves as a platform for interactive discussion on industry-level topics.

The Roundtable members created a working group with the goal of developing a framework of the most relevant APMs. The framework, summarized in this paper, covers key access-related time...
points along the product lifecycle and thus enables relative comparisons over time.

**Toward common and comprehensive APMs**

While the access functions at all participant companies have begun to measure access performance, pharma companies think differently about what and how to measure. Also, there is no common language to describe different APMs, let alone common definitions. Some of the variance is driven by differences in companies’ portfolios, and, therefore, diverging access priorities and focus. The other driving force of the variation is the relative youth of the access function.

More “traditional” functions, for example, finance, regulatory, marketing, or manufacturing, have sets of long-established performance metrics that can be reviewed in any college textbook. As the access function has evolved in breadth and depth over the past years, there are no similarly well-established performance metrics that companies can follow. Most companies have developed their own metrics based on their needs at the time and what data was readily available. Thus, there are different metric definitions and use-cases across pharma organizations. Further, most of the roundtable participating companies acknowledged that the way they are currently measuring the performance of the access function could be improved.

The roundtable set out to see if a common framework could be created to serve the following two purposes:

1. Provide a more robust basis to measure access performance, and enable better decision-making upfront or in-flight course correcting where necessary.
2. Evaluate and demonstrate the role and contribution of access activities and achievements to the overall business success.

**Framework’s five principles**

Toward the mentioned objectives, the Roundtable defined a framework that focuses on the most relevant APMs, and is guided by five design principles:

» Account for the interdependencies between different APMs.
» Cover key milestones along the product lifecycle.
» Enable relative/time-based comparisons.
» Encompass lagging and leading metrics.
» Cover the full scope of modern access functions.

1. **Account for the interdependencies between different APMs.**

The three key access objectives—price, time to reimbursement, access level—are at the core of every access strategy. Collectively, they describe the strategic

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**Working Group Members**

(And co-authors of this report)

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- **Indranil Bagchi**, SVP, Head Global Value & Access, Novartis Oncology
- **Katja Berg**, Head of Operations and Payer Intelligence, Sanofi
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* The opinions expressed in this paper are solely those of the authors and do not represent the positions of the organizations they are a part of.
## 20 Access Performance Metrics

<table>
<thead>
<tr>
<th>Dimension</th>
<th>Access Performance Metrics</th>
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<tbody>
<tr>
<td>Access levels</td>
<td>% of TPP/label with access</td>
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<tr>
<td>Price levels</td>
<td>List price</td>
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<td></td>
<td>Net price</td>
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<tr>
<td>Time</td>
<td>Timeliness of global deliverables dossier (inc. availability of data)</td>
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<td></td>
<td>Time from regulatory approval to P&amp;R submission</td>
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<td></td>
<td>Time from regulatory approval to P&amp;R (national) approval/Time from P&amp;R national approval to first sales</td>
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<tr>
<td>HTA outcomes</td>
<td>Benefit rating/outcomes</td>
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<td></td>
<td>Achievement of additional benefit ratings (sub group vs total)</td>
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<tr>
<td>Contracting</td>
<td>Types of contract used to achieve/maintain P&amp;R</td>
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<tr>
<td>Policy outcomes</td>
<td>Legislation/policies impacted</td>
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<tr>
<td>Stakeholder engagement</td>
<td>Number of times advice sought from payers/HTA</td>
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<tr>
<td></td>
<td>Number of times input sought from patient groups</td>
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<tr>
<td></td>
<td>Customer satisfaction</td>
</tr>
<tr>
<td>Evidence produced</td>
<td>Inclusion of access-related considerations in early evidence generation</td>
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<tr>
<td></td>
<td>Number of economic models/RWE published</td>
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<tr>
<td></td>
<td>Timeliness of evidence availability</td>
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<tr>
<td>Planning/alignment/</td>
<td>Number of programs discontinued/not filed/not submitted due to access concerns</td>
</tr>
<tr>
<td>decision-making</td>
<td>Degree of alignment of access with brand and medical plans</td>
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<tr>
<td></td>
<td>Number of TPVs/clinical plans adapted/tailored for market access</td>
</tr>
<tr>
<td></td>
<td>Degree of access launch readiness</td>
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### Figure 1: APMs across all dimensions.

Choices a company made during development and launch (and beyond). In an ideal world, one would be able to achieve a value-driven price, quick time to reimbursement, and broad access all at the same time. In reality, drug manufacturers need to make choices and tradeoffs between these three objectives. For example, to achieve a value-driven price point, longer negotiations may be required—thus impeding the objective of time to reimbursement. Similarly, when optimizing the price point, it is likely that payers limit reimbursement to a high-benefit subpopulation only, and rendering broad access impossible.

Hence, looking at APMs on these three strategic objectives in isolation is not helpful. Whether or not a certain time to reimbursement is fast can only be assessed in the context of the other two elements of the trinity of price, time to reimbursement, and access.

As a side note, the set of access parameters is not intended to form the basis for a benchmarking effort. Given the varying contexts of different products, and the divergent strategic decisions companies have taken, it is not meaningful to try to compare across products and companies. For example, comparing the price achievement or the time to reimbursement of two different products (or even companies) is meaningless without knowing the strategic objectives and tradeoffs taken. If the strategic objective was to optimize time to reimbursement, a company may have accepted a lower price point than the value of the product would have warranted. But as ex-post benchmarks cannot cover the strategic decisions taken many years before a price is achieved, a benchmark would hardly compare apples to apples.

### 2. Cover key milestones along product lifecycle.

There are four key access time points along the product lifecycle that APMs need to cover: initiation of pivotal trial program, regulatory approval, (national) pricing and reimbursement approval, and peak sales—about five years post-pricing and reimbursement. Pharma companies make important access-relevant decisions long before launch, and APMs need to cover access-related decisions from the initiation of pivotal trials to give management a tool to track and course-correct before the impact is felt at launch. For example, evidence plans need to have incorporated access considerations already at the initiation of the pivotal trial program to avoid risking access evidence gaps.

### 3. Enable relative/time-based comparisons.

It is important to note that some metrics (e.g., price, health technology assessment [HTA] outcome, access level) require “normalization” in order to be meaningful (i.e., by measuring actual versus planned). Covering APMs over time and including early access-related decision points allows for relative comparisons and comparisons over time. Many APMs are meaningless when looking at their face value only. A point-in-time measurement of, for example, the “number of stakeholders involved” does not express if this number is high or low.

Similarly, a price achievement at launch provides richer insight if one can compare against planned price levels before launch. Any deviation from planned price levels then provides management an opportunity to dig deeper to understand “why,” and derive the required insights and actions. It is important to run such analysis at several points along the lifecycle as more becomes known about the drug.

### 4. Encompass lagging and leading metrics.

We differentiate between leading and lagging metrics. While lagging metrics are important to assess outcomes (and derive learnings), most cannot be used to course-correct in-flight.
Hence, it is critical to include a set of leading metrics that allow functional leaders and senior management to track in-flight progress, and take corrective action before the impact is felt.

5. Cover the full scope of modern access functions. While price is often the most visible, and most well-known, responsibility of the access function, it is by no means the only element that needs to be tracked. Just as the scope of the access function evolves, APMs need to go beyond pricing and reimbursement-focused topics and cover the full breadth of the access function. Thus, the framework covers the traditional access metrics of price (including contracting), access (including HTA outcomes), and time to reimbursement.

On top of that, it also covers APMs for aspects of access that have become more important recently: policy outcomes, stakeholder engagement, evidence generated, and cross-functional planning/alignment/decision-making. By covering all access-related aspects, it allows access leaders, as well as other functions and senior management, to focus on the right levers and derive meaningful insights.

Six additional APM dimensions

Based on these five design principles, the Roundtable defined an access performance metrics framework covering 20 APMs along four time points over the product lifecycle. In addition to the three key access metrics already discussed, the framework covers six other dimensions of APMs.

> **HTA outcomes:** This APM is typically “owned” by HEOR (health economics and outcomes research) teams, and relevant for specific countries (e.g., Germany, France).

> **Contracting:** Covering the types of contracts used shows the local capabilities to devise and negotiate innovative contracts, as well as giving insights on whether the full breadth of possibilities was explored to achieve favorable access conditions.

> **Policy outcomes:** As health policy becomes more important, access leaders are required to track and measure the impact of policy work. Given the qualitative nature of the type of work that is being measured, this APM is less of a “hard” measure than, for example, price.

> **Stakeholder engagement:** With the expanding scope of access, and the proliferation of stakeholders involved in access-related decisions, APMs around stakeholder management and engagement (e.g., via early parallel scientific advice) set the right incentives to access functions to engage with their stakeholders more systematically.

> **Evidence generated:** Integrated evidence planning (i.e., the inclusion of access-related considerations when generating early regulatory evidence) is quickly and rightfully becoming the industry norm. To help the organization prevent evidence gaps at launch, tracking the access input into evidence generation is important. Other APMs, such as the number of HTA models and timeliness of evidence availability, also look at the organizational efficiency and effectiveness.
Planning/alignment/decision-making: The last set of APMs cover internal alignment and collaboration. Given the increasingly cross-functional nature of access, the alignment with other functions is an important leading metric to track. The more input the access function gave into TPP and clinical trial plans, and the higher the alignment between medical, commercial, and access plans, the higher the likelihood of achieving launch and access goals.

Figure 1 (see page 28) lays out the 20 APMs across all dimensions, and Figure 2 (see page 29) maps the APMs along the key time points in more detail.

Current APM adoption observations

While Roundtable companies agree on the need for more systematic tracking and measuring of APMs, only few apply all APMs across products in practice today. A survey on the adoption of APMs by Roundtable companies shows how the role of such metrics is evolving, but also demonstrates the large variance in metric definitions and usage to track performance and inform decisions. Based on the online survey of 14 Roundtable companies conducted in late 2017/early 2018, we make three overarching observations:

1. Most companies track three core APMs. These three APMs constitute some of the earliest and most well-established APMs.
   - Negotiated price at list and at net price level as the fundamental metric to manage performance and track predictions (all companies).
   - Timeliness (of global deliverables), which is widely seen as critical for launch success, as it drives resource allocation (~85% of companies). For some companies, it is seen as even more important than commercial deliverables.
   - Access components of launch readiness. Relatively widely used to ensure focus and prioritization, but sometimes inconsistently applied across products (~70% of companies).

2. Tracking broader access factors versus a sole price focus is gaining traction. About half of Roundtable companies have moved to tracking product-specific access levels—a big change from only about five years ago, when access was rarely tracked systematically. The most advanced companies in this regard have now also included certain access achievements in their top-management personal goals. Overall, we note a move from pure focus on price achievement and management and launch preparation, to broader consideration of access levels, outcome metrics (e.g., benefit ratings), payer inputs, and evidence planning, among others.

3. There is a large variance in adoption and usage of APMs. While some companies systematically track 10-plus APMs, others track only a handful (see Figure 3).

Figure 3 shows the self-reported APM adoption of 14 Roundtable companies. The most commonly adopted APMs are shown on top, the least adopted APMs at the end. Each column represents one company.

Using the APM framework

The Roundtable is not suggesting that all companies use all APMs equally. Given each APM requires effort in generating, interpreting, and discussing the data, companies need to be selective in their use of APMs. Which APMs to track and what to use APMs for depends on several company-specific factors, including:

- Company portfolio in terms of type and number of products, disease-area focus, and breadth.
- Sophistication of the access function.
- Organizational setup.
Data availability and/or effort required to generate certain APMs.

Given such company-specific considerations, there is no one-size-fits-all approach in tracking and using APMs. The framework provides a structured selection of the most relevant APMs. Companies, and especially access functions, need to define which of their objectives APMs can support, what they are trying to achieve and incentivize through an APM, and how they plan to use the metric. While APMs cover access themes, each metric does not necessarily need to be “owned” by the access function. Depending on what is measured and how the metric is used, responsibility for an APM may be better placed in a different function, or at regional/affiliate level.

Also, when using these measures, it is important to define “what good looks like.” Failing to do so can lead to unintended consequences. It is human nature to react to incentives—positive and negative—and an APM creates an incentive to optimize the outcomes of a certain APM. For example, an APM on “time to reimbursement” can in the worst case lead regional and local organizations to accept lower price points in order to achieve the target time. It is likely that local teams did not capture all the value in their pricing strategy and negotiations.

Looking ahead
Access functions need to continue to move from being purely technical to addressing much broader, business-critical questions. Today, access no longer focuses just on health economic modeling or price predictions, but instead addresses much broader, business-critical questions. Access leaders and senior management should not strive for 100% of markets to achieve the APM target of time to reimbursement. Eighty percent of the markets achieving the APM (and, consequently, 20% of markets not achieving reimbursement by the target time) is the better outcome; it means that the price level was set at the optimal level. If all markets achieved reimbursement in time, it is likely that local teams did not capture all the value in their pricing strategy and negotiations.

Moving forward, the Roundtable hopes this framework helps provide clarity and greater consistency in how the role and contribution of access is being measured, managed, communicated, and conceived by other stakeholders. Given the cross-functional nature of market access, it is critical for all teams and departments in a pharma company to have a working knowledge of access, and understand how their decisions can impact patient access.
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After a prolonged period of relative stagnation, the strategically-influential, clinically rich USD 65 billion French life sciences market suddenly seems to be rediscovering its mojo. With the UK economy consumed with Brexit and neighboring markets such as Italy afflicted by the vicissitudes of populism, France, in the midst of a welter of ambitious reforms instigated by its energetic president, has been rocketing up to the top of investor priority rankings. Moreover, “this turnaround has been looking pretty comprehensive,” says Frédéric Collet, president of Novartis’ local affiliate, noting that “according to the latest IPSOS figures, investor confidence in our country is now topping 74 percent when only a few years ago it was languishing down in the low 20s.” And with the freshly elected President Emmanuel Macron now pledging to “rebuild France’s self-confidence and global stature as an innovation nation,” it is very much the pharma and medtech sectors that suddenly find themselves in the spotlight.

Indeed, the 8th Strategic Council for the Healthcare Industries (CSIS), a biennial confab between central government and industry stakeholders, held last year was unprecedented both in terms of its content and method. “What we observed this time around stretched well beyond the usual simple declaration of intentions to a true process of implementation of a number of major substantive decisions aimed at promoting attractiveness for future investment while simultaneously increasing France’s competitiveness on the global scale,” affirms Jean-Luc Bélingard, president of the French Federation of the Health Industry (FEFIS), who has been appointed by the authorities to oversee the implementation of the commitments agreed upon.

“This administration, more than ever, views partnerships and innovation at the forefront of our future national economy and strives for the country to fully mobilize the natural talent, creativity and performance of French enterprise,” proclaims Minister of the Economy and Finance Bruno Le Maire, highlighting the new spirit of togetherness and the elevation of pharma and medtech as one of the centerpieces of the new national industrial strategy. “We consider the healthcare and life sciences sectors as none other than a catalyzer of these attributes, both as a dynamo of technological advancement and host to some of the great emerging high value-added industries of the future, from genomics to artificial intelligence to health data,” he declares.
There can be little doubt that there is a lot of catching up to do as the French life sciences industry has been going through a rough patch of late: visibly losing competitiveness on multiple fronts. First and foremost in the minds of many captains of industry has been the comparative lack of profitability. “Sector growth has been frankly stymied by a rather unfavorable policy environment because, although pharmaceuticals constitute a mere 15 percent of annual social security spending, we have been consistently called upon to contribute to over 50 percent of the overall social security savings!” exclaims Collet.

What’s more, a narrative of systemic underperformance seems to have been replicated across the board. Manufacturing productivity, for example, has been on the slide. “France used to be number one in Europe for the production of healthcare products and pharmaceuticals, but within the space of only five years we have fallen to sixth place, even surpassed by Italy. Not so long ago, this would have been pretty unthinkable,” laments Philippe Lamoureux, director of the pharmaceutical industry union, the LEEM.

Indeed, according to figures compiled by G5 Santé, a think tank grouping eight of the most influential indigenous French healthcare companies, the value of France’s pharmaceutical manufacturing output sank to EUR 21 (USD 23.8) billion in 2017 when it had been in excess of EUR 25 (USD 28.3) billion back in 2009. “Our estimations tell us that the country has squandered somewhere in the region of EUR 10 (USD 11.3) billion worth of production, much of which constitutes unrealized exports, when you factor in that German, Swiss and Italian production have all been climbing during the same period,” reveals Yves L’Epine, CEO of medical imaging player, Guerbet, and president of the G5.

So too has France’s dominance in clinical research been eroding. “We are undoubtedly losing our competitive edge as a premier clinical trials destination, because we’re seeing a proliferation of firms instead choosing to perform their research in Belgium and Spain,” observes Denis Comet president of the French Association of Clinical Research Organizations (AFCRO). “We do possess all the requisite technical requirements to conduct clinical trials in France, but it is, in reality, rather difficult to set them up, as that generally requires between 12 and 18 months to secure the necessary agreements with ethics committees,” explains Thierry Hulot, president of biopharma at Merck.

How is it, then, that French pharma has managed to lose its edge? Many firms complain of regulatory framework that inhibits smooth market access. “It’s just an extremely lengthy, elongated and longwinded process navigating the French regulatory landscape,” reckons Emmanuel Eumont, general manager of Gedeon Richter. “First your product has to pass through the Agence Nationale de Sécurité du Médicament et des Produits de Santé (ANSM)’ to obtain the initial marketing authorization, next your dossier gets passed over to the Haute Autorité de Santé (HAS) and Transparency Commission for matters of health technology assessment and reimbursement, and then, after all of that, it goes over to the Comité Économique des Produits de Santé (CEPS) for pricing negotiations, with each of these individual steps requiring considerable discussions.”

Little wonder, then, that the process can be beset by long delays. “The average period between the European marketing authorization and the publication of the negotiated price in France lasts in excess of 500 days; France thus ranks a lowly 18th out of the EU27 in terms of time frames,” points out Biogen general manager, Martin Dubuc.

**DRUG REGULATION: RETOOLING THE MACHINE**

Even worse, many industry insiders believe the French health technology assessment (HTA) methodologies to be behind the times and fundamentally ill-suited to a future world of medicine characterized by sophisticated biologics and personalized, precision...
therapies. “The way products are assessed, is simply not fit-for-purpose in the current context of advancements in medicinal science. Treatment pathways are undergoing profound changes and the regulatory apparatus badly needs to catch up,” argues Jean Monin, former general manager of Amgen in France.

“It’s worth remembering that many latest-generation, breakthrough innovations today relate to tiny, niche indications where you simply cannot generate the level of data at the time of reimbursement that is being requested. What’s needed is a mid-term, provisional or conditional assessment to cover the period until full maturity of data can be attained. Right now, owing to the lack of complete data, these state-of-the-art therapies are being thoroughly undervalued in the assessment,” reflects Pierre-Claude Fumoleau, managing director at AbbVie.

“You only have to consider the huge discrepancies between FDA and French HTA decisions to realize how out of kilter the latter is: it’s the same molecule and same evidence in the dossier, but a totally different adjudication, which confirms there is a systemic failure of process,” he posits.

That has certainly been the experience of Nordic Pharma’s general manager Vincent Leonhardt. “We in-licensed an oncology therapy from a Japanese company, and the product attained approval across Europe, including in France. This product demonstrates exactly the same efficacy as equivalent therapies, but offers the advantage of having a far superior safety profile with fewer side effects for the patient. Nevertheless, the product was not even assessed by the French pricing authorities because it did not meet their eligibility criteria. This product was thus effectively locked out of the market, despite delivering clear added value to the patient in the sense of improving quality of life,” he recounts.

Stories of trouble bringing innovative molecules to market abound. “Sometimes we find ourselves unable to launch a particular molecule at all,” laments Lundbeck’s managing director, Lourdes Pla. "For example, we have a new schizophrenia treatment, in conjunction with Otsuka, that
we rolled out this year in the Nordics and will imminently arrive in the Dutch, Italian and Spanish markets, but we are still trying to figure out how and when to get it to France,” she admits.

“It took us nearly 1,000 days to get our product Kyprolis® for the treatment of multiple myeloma out into the marketplace last summer,” recalls Amgen’s former general manager Jean Monin, but what mystifies him the most are some of the obvious illogicalities in the assessment process. “Having a channel whereby a company can appeal the decisions of the committee makes very little sense if you are prevented from adding any new data and are appealing to the very same adjudicators who have denied authorization for your product the first time around… in the vast majority of cases, the new decision is already a foregone conclusion,” he exclaims.

France’s regulatory authorities do admit that the current system needs to be overhauled. “There are both technical differences in the way we consider cost-effectiveness and fundamental philosophical differences that distinguish us from more Anglo-Saxon approaches to drug pricing. For example, the British deploy the QALY (quality-adjusted life year) metric to measure a drug’s value whereas we don’t,” notes CEPS president Maurice-Pierre Planel. “As treatment pathways get transformed, we will obviously have to evolve accordingly.”

Dominique Martin, CEO of the main regulator, ANSM, also speaks openly about the renewal that his organization is undergoing. “It used to be that, capriciously, authorization could take months or even years to obtain, so we are working hard to unblock these bottlenecks and are in the midst of refining our technical procedures… however we are not the only authority to have a say and interactions with other arms of regulatory apparatus can be cumbersome, so we still have to collectively identify a better way to streamline the process.”

Aspen: Embracing Unconventionality

“When we think of France, we’re talking about a highly regulated market, thick with complexity and this obligates us to be more creative and original in how we do business,” notes Aspen’s general manager, Franck Hamalian. Luckily the willingness and flexibility to do things differently is precisely what Aspen, one of very few high-performance pharma MNCs to herald from an emerging market, excels in.

“We deploy a unique business model bringing together innovative products and a traditional generics arm with a focus upon high quality, affordable, difficult-to-produce, specialty products which are transversal within a healthcare system. We are not an R&D company so we instead develop ourselves through acquisitions resulting in a brand-new portfolio that we fully integrate into our operations. This is actually quite unique for a pharmaceutical company. Indeed, in traditional pharma, companies merge and the portfolios are sorted and either hived off or retained whereas, at Aspen, we aim for very specific products and internalize production as quickly as possible with every decision driven by production capabilities and speed of acquisition,” reveals Hamalian.
Yet, for all of the challenges experienced over the past decade, there are undeniably still substantial profits to be made within French pharma. According to the LEEM’s own statistics, pharmaceuticals generated almost EUR 54 billion (of which 47 percent derived from exports) in 2017.

A MARKET OF ENDURING APPEAL

Moreover, there is a strong consensus that France possesses considerable latent assets that just need properly putting to good use. “All of the prerequisite ingredients for a great recipe are at hand and easily accessible: France demonstrates immense potential for innovation thanks to the country’s superb academic centers and hospitals, a strong legacy in medical science, an educated workforce, a solid economy and a strategically relevant geographic location, all of which can be harnessed to operate a high performance business,” affirms David Meek, CEO of Ipsen.

Indeed many multinationals seem ready to overlook the regulatory hurdles so as to focus on the greater prize. British pharma heavyweight, GSK, is a case in point. “GSK has been the biggest international pharma company in terms of footprint and employment in France for many years and has an integrated presence at all stages of the supply chain. Between 2018-2020, we anticipate

At the Sharp End of Vaccination

Some in big pharma might have been migrating away from the vaccines space, but Sanofi Pasteur, drawing upon the tremendously rich legacy of Louis Pasteur, very much bucks the trend: having posted robust growth of 8.6 percent in 2017 and now pressing ahead with ambitious plans to double its global output by 2023. Much of this success has come on the back of smart specialization with the Business Unit successfully innovating new generations of pediatric and epidemic vaccines to the point where it can now lay claim to 40 percent plus market shares across virtually all large Western countries.

“Specialization has become a necessity, rather than merely a strategy. The real challenge in vaccines development derives from the reality that this is a capital-intensive industry with high barriers to entry, as margins are not as high as in other fields, such as oncology, and it takes years and a EUR 500 (USD 564) million investment to build a single plant,” explains executive vice president, David Loew.

“Given these elements, accurate demand forecasts are essential to being able to plan production and avoid underutilization of capacity, which destroys profitability. The fact that each plant might be specialized in a single antigen adds a whole additional layer of complexity connected to the inflexibility of production,” he continues.

A further formidable barrier to ensuring smooth supply chains is tendering. “Tenders based on lowest prices might generate public expenditure savings in the short-term, however, it creates a significant problem for vaccine availability in the long run. Producers do not know in advance if they will win and thus the uncertainty related to capacity utilization is high. Governments, like the US, Germany and France, have understood this but the system remains prevalent in Italy, Spain and most emerging markets and this is one of the primary reasons why so many stock-outs happen in this industry,” argues Loew.
spending circa EUR 335 (USD 379) million on local R&D and manufacturing. France possesses excellent assets including continued public investment in health, an attractive R&D tax credit, highly skilled people and a quality infrastructure network,” discloses the company’s president, Josephine Yang Comiskey.

On top of those very alluring market fundamentals, France’s prowess at the pioneering cusp of many therapeutic areas renders it of great strategic importance to firms operational in those spaces. “France has dedicated neurosciences centers of outstanding quality in nearly all areas of neurological disease, so clinically speaking, the country offers a strong value proposition for an outfit like Biogen that strives to become the frontrunner in combatting neuro-degenerative conditions. As such, our own in-country clinical trials have increased nearly 30 percent over the past three years and we are proud to say we currently have 21 ongoing clinical trials with more than 2,000 patients involved,” discloses Martin Dubuc.

Aspen’s in-country investment has followed a similar logic. “Commercially the affiliate is a EUR 90 (USD 100) million operation and our Notre Dame de Bondeville facility constitutes a key production platform, but beyond that, France plays a significant role for us because it is a top five market in most therapeutic areas, especially anesthetics. There is access to renowned KOLs in anti-thrombotics and anesthetics who can play a critical role in the discussion of how to develop these areas in other markets,” reflects Franck Hamalian.

The same could be said for companies focusing on immuno-oncology; yet another area where France is an undisputed regional leader with world-renowned institutes such as Gustave Roussy. “We have created a new drug development entity called the Department of Therapeutic Innovation and Early Trials which is the only division of its kind in Europe,” enthuses the institute’s general director, Alexander Eggermont. “Our system brings between 3500 and 4000 patients into clinical trials every year, which makes it the largest such program in Europe and, for early clinical trials, one of the top five programs worldwide.”

Moreover, the somewhat surprising election of Emmanuel Macron as President in May 2017 has unleashed a plethora of unexpectedly wide-reaching reforms that are now promising to propel the French life sciences sector right back to its former glory at the pinnacle of European medical scientific advancement. “The central idea behind last year’s historic breakthrough 8th Strategic Council for the Healthcare Industries (CSIS) has been to send a forthright and unequivocal message to the international investor community that France seeks to become a country of innovation, research, and global leadership in life sciences again. It signaled that we are open for business. We want elite pharmaceutical actors to know that they can operate here and generate a fair price for their work,” proclaims Julien Borowczyk, secretary of the Social Affairs Commission at the Assemblée Nationale.

What’s more, Macron has made an overture to domestic pharma and medtech developers to let them know that this is a pro-business government that understands their needs. “What has been so very distinctive about this CSIS has been its sheer pragmatism with an actionable set of goals and a dedicated partnership with representatives of private enterprise who will be able to determine if we are correctly implementing our policies and establish whether we are on track,” attests Thomas Courbe, general director at the Direction Générale des Entreprises (DGE) within the Ministry of the Economy & Finance.

What, then, have been the concrete takeaways from the event? “The main achievement has been the recognition of the healthcare sector as a strategic element of this country’s economic fabric
WE BELIEVE IN A WORLD IN WHICH NO ONE SUFFERS OR DIES FROM A VACCINE PREVENTABLE DISEASE.

For over 100 years, Sanofi Pasteur, the vaccines global business unit of Sanofi, has been committed to extending the life-saving power of vaccination as broadly as possible. We are proud our vaccines can help protect us and our loved ones from a wide range of severe infectious diseases, at every stage of life.
by the government. This is a big practical step forward, as we have not been considered as a strategic asset in the past, but as a cost burden. This re-conceptualizes the way that policymakers view public health: namely repositioning it as an area that is worthy of sustained investment,” opines FEFIS’ Bélingard.

“The change in the rules of economic regulation that we obtained is also hugely significant as, up to now, each of the envelopes – hospital and retail – were financially separate... This implied that even if you over-performed in savings with one envelope, you might still have been eligible for paybacks by poor performance with the other. From now on there will be a single envelope for the two markets which should give our industry better visibility and predictability,” reflects the LEEM’s Philippe Lamoureux.

This is certainly an issue that is close to the heart of many of the LEEM’s members.

“In the past, the French market suffered a lot because of insufficient visibility, but the proposed changes should make it substantially easier to plan investment and the attached ROI,” ventures Gedeon Richter’s Emmanuel Eumont. “This is the game-changing aspect, because the innovative pharma industry is inherently a long-term venture: ten years are required to develop a product marketed for at least another ten years, so it is a cycle of roughly 20+ years. If you do not have any visibility, then you cannot invest because the risk exposure is too great.”

COMPRESSED APPROVAL TIMES

Measures have even been outlined to try and fix France’s creaking market access system with a pledge to shorten the time period for reimbursement and pricing decisions down to the EU recommendation of 180 days and a popular commitment to extend out France’s much-loved Early Access Program: the Autorisation Temporaire d’Utilisation (ATU) mechanism.

“Our neighbors in adjacent markets envy the unique ATU system that we have in France, with good reason! It’s a fantastic system that enables patients to get access quickly to innovative treatments, and without a doubt this mechanism has saved and improved many lives,” exclaims Franck Puget, managing director at CSL Behring.

To date, the mechanism has tended to cover only around ten percent of the patients clinically eligible for the drug and has not been designed to take into account the whole target population, but one of the big outcomes of the CSIS has been a promise to extend its usage. “Right now, only a limited number of molecules actually meet the stringent eligibility criteria for the ATU: it has to be an indication of high unmet need and, up until now, second and third indications are disqualified. It’s most definitely a positive step forward to allow further indications because even incremental innovation can deliver substantial benefits, but we’re advocating for it to be transformed even further into a proper accelerator,” says Emmanuelle Quilès, CEO of Janssen.

One company that will be watching very carefully to see how the ATU is being reformed is German plasma protein supplier, Biotest. Some of their products made from specific plasma proteins are highly innovative with no two formulations exactly the same and catering to niche markets thus well placed to benefit from the mechanism. “France is the second European market for plasma products just after Germany and demand for those products is still increasing especially for innovative treatments in rare diseases. In cases of high medical need, the French Authority has really led the way by introducing ATUs prior to registration to allow a quick access to innovative treatments,” recounts Biotest’s managing director, Olivier Samama. “Both our Cytomegalovirus (CMV) and varicella specific immunoglobulin treatments are submitted to ATUs... and we also put in place a Protocol of Therapeutic Use (PUT) [a recent procedure required for ATU products under which the indications for which the product can be used are restricted – Ed.].”
Leveraging a rich industrial tradition that originated in the silk and chemicals sectors, Lyon has managed, over the years, to successfully cultivate a fully-fledged life sciences ecosystem that today encompasses an entire value chain of actors including start-ups, manufacturing facilities, research labs, hospitals and clinics. Along with the fruits of industrialization, however, have arisen fresh challenges. “While my predecessor, Gerard Colomb, made clear headway in putting Lyon on the map and establishing a consistent brand for the region, nowadays we are making subtle changes to our development model, conscious of the possible dangers of becoming victims of our own success,” explains David Kimelfeld, president of Lyon Métropole. “We have entered a new phase in our development lifecycle in the sense that we now strive to privilege quality of growth over quantity.”

Paris is, very much, an example of what not to do. “Indeed we have welcomed a great many Parisian firms complaining of expensive rents, poor infrastructure and congestion who had got to the point where they were searching for an alternative location and base to operate from. We thus have to remain vigilant about the environment, transport and the overall urban texture all of which have a big impact on the quality of everyday life. After all, the life sciences industry is an environmental and health-oriented sector at the same time,” contends Kimelfeld.

Jean-Charles Foddis, Executive Director of Adery, the agency for the economic development of the Lyon region very much agrees. “We’re not looking to attract absolutely every company, but rather only those related to our focus sectors. We are also attentive to ensuring that companies are supported in their set-up here so that Lyon can be ready to welcome in new entities without compromising the development of the firms already present.”

Christophe Cizeron, president of Lyonbiopole, meanwhile, is trying to encourage Lyon to broaden its horizons. “Our aim is to position the pole as an international reference point... Medical research and innovation are becoming increasingly globalized and we have to align with these tendencies and be able to accompany life science entities wherever they need to go,” he urges.
Furthermore, the momentous signing of a “Strategic Contract of the Health Industries and Technologies” between the Macron administration and the life sciences industry on the 4th of February 2019 has given further substance to the original aspirations of the CSIS. Standout objectives include cultivating France’s innovative drug development base by “increasing bio-pharma manufacturing productivity by a factor of 100 in a decade,” and “nourishing homegrown health technology champions able to hold their own on the international stage.” “With this strategic contract we are seeking to usher in a new era of French industrial conquest through innovation,” confirms Agnès Pannier-Runacher, secretary of state for the Minister of the Economy and Finance.

Already signs are afoot that France is ramping up its participation in bio-pharma with the news that Novartis has selected France as the first host country to conduct CAR-T cell therapy manufacturing outside of the United States and Switzerland by signing an agreement to acquire French state-owned entity, LFB’s contract manufacturing subsidiary, CELL for CURE.

“CELL for CURE is an industrial platform located in Les Ulis, near Paris. This site is the first and largest in Europe for the production of cell and gene therapy drugs. This is clearly a momentous occasion and positive signal that France has been chosen as the host country for Kymriah (tisagenlecleucel) manufacturing. This is a big vote of confidence and emblematic of our manufacturing bases’ ability to scale the value chain,” opines LFB’s chief executive, Denis Delval.

Frédéric Revah, president of YposKesi, an expert industrial platform exclusively dedicated to manufacturing and development of gene and cell therapy products, believes that it is a very smart move on the part of the authorities to encourage local production of complex biologics. “Gene therapy has a unique feature: a very large part of the final value derives from manufacturing. The costs are spectacular. If we are not able to innovate in manufacturing, products invented in France will have to be produced elsewhere and that would constitute an incredible loss in economic value for our country,” he asserts.

COMPETING ON THE WORLD STAGE

In a sign, perhaps, of things to come, high-performance French mid-cap pharma firms are increasingly asserting themselves abroad and scaling the value chain by branching into ever more sophisticated medicines. Ipsen probably best exemplifies this approach, having taken the bold steps of appointing their first ever non-French CEO, penetrating new power markets across the world and building from scratch an entirely new oncology franchise. “At the outset, we were very clear that we needed to unleash a profound and ambitious transformation right at the heart of the company that would equip Ipsen to face the future with zeal and confidence. This entailed reconfiguring the leadership team, instilling a biotech mindset and nurturing a culture of external innovation with a view to driving our research, development and commercialization. I have been very pleased with this growth profile, especially in the way that we are broadening our reach globally: the US market now makes up 25 percent of our overall business and our Chinese affiliate is also thriving,” details their American CEO, David Meek.
The Strategic Contract’s explicit emphasis on fostering local champions, meanwhile, is, of course, music to the ears of French manufacturers. Pierre Moustial, CEO of the renowned wound-care medtech company, Urgo, certainly believes that it is about time for the state to do more to bolster homegrown medical device players. “It is unfortunate that there is, of yet, no French company within the top 30 medtechs worldwide despite our country exporting some EUR 7 (USD 7.9) billion worth of medical devices each year, but the reality is that younger medical devices companies require a strong performance at home as a shop window when exporting their products, otherwise they lack the credibility of their own market and that inhibits their development. They could definitely do with some support and it is positive that the state is finally beginning to appreciate this,” he insists.

“Macron has spoken extensively about the time being ripe to restore momentum and confidence to national industry and consequently launched the ‘La French Fab’ initiative to promote a home-grown industrial base made up of mid-sized exporting firms,” notes Stéphane Mathieu CEO for Europe, South & West operations at SIGVARIS GROUP, a company that straddles both the textiles and medtech sectors having pioneered the concept of compression stockings as a complete alternative to treating venous insufficiency with medicines. “This ‘French Fab’ movement is essentially about uniting French industrialists under a common banner able to spread the excellence of French manufacturing abroad. SIGVARIS is participating out of a desire to become a flag bearer of the ‘Made in France’ brand and because companies like ours are actively doing a great deal to reinvigorate the national industrial ecosystem and foster quality job creation,” adds Mathieu.

**UNSHACKLING ENTREPRENEURIALISM**

Meanwhile small and medium sized life sciences enterprises are also in line for some much-needed relief courtesy of the Ministry of the Economy & Finance’s freshly unveiled flagship Action Plan for Business Growth and Transformation or ‘Loi PACTE.’ “This is a broad-sweeping initiative that should lighten the administrative burden and lift any disincentives to growth for SMEs in the health technology sector. This basket of measures is partly intended to encourage the recruitment of new employees by streamlining workforce thresholds and facilitate the creation of start-ups and spin-offs by researchers,” outlines Frédérique Vidal, minister of higher education, research and innovation.

After all, France is a hot-bed of entrepreneurs, with more than 550,000 business creations a year. “The PACTE bill also streamlines the patenting process to simplify public-private partnerships and unleash our budding entrepreneurs and researchers to focus on discoveries that improve patients’ lives,” affirms Minister of Health and Solidarity Agnès Buzyn.

“I think small and mid-sized businesses will ultimately be emboldened by the Macron administration. This is a presidency that really appears to understand what is needed to push a business and the sorts of constraints that one feels on a day-to-day basis as an entrepreneur,” suggests GREENTECH’s Jean-Yves Berthon.

“I wouldn’t be surprised if nowadays biotech companies choose to remain in France, whereas in the past they tended to leave the country after finding success. The PACTE and fiscal measures like the innovation tax credit and the extension of the research tax credit should certainly help to foster a more welcoming environment,” predicts Nicolas Bardonnet, general manager at Promega, a Wisconsin-based supplier of enzymes and molecular biology who’s ‘For You Program’ enables small labs to have access to their latest technologies without having to invest in instrumentation outright.

**MIXED OPINIONS**

Not everyone is completely happy with the overall thrust of the new reform package, however. Instead, certain specific segments of the life sciences industry feel left behind and rather excluded from the general euphoria. “When looking at CSIS, the project of the current administration has been very much to promote and support innovation in France, but it is important to understand, however, that this sort of innovation is very different from the R&D concept we have in place at Nordic Pharma, as we focus on incremental innovations and drug rediscovery,” points out Vincent Leonhardt.
These may not be breakthrough innovations as expected by the administration, but smaller innovations, which deliver a significant benefit for the patient in terms of comfort, efficiency and compliance, and therefore correspond with unmet medical needs. Consequently, I am concerned that the policy in place does not sufficiently reward incremental innovation as it focuses almost exclusively on developing highly expensive, breakthrough technologies that grab headlines. SMEs that are engaged in different types of innovation such as modifying the delivery mechanisms for existing medications to make them more patient-friendly or enhancing user adherence are the unsung heroes of the drug development sector and need to be rewarded too,” he concludes.

In a not entirely dissimilar manner, some generics producers have also been slightly disappointed. “While innovative researchers are turning more and more to biology for innovative drugs, the majority of drugs consumed will be chemically synthesized and treat rather common ailments. Generic drugs are exceptionally valuable when this is taken into account because we can affordably produce quality drugs that provide treatment for many illnesses, freeing up funding for intense R&D,” reminds Catherine Bourrienne-Bautista, delegate general of the generics association, GEMME. “The CSIS was supposed to be focused on innovation and industry, however, we noticed there was a much larger emphasis on innovation than on industry. There have not been many industrial reforms that especially concern classic small molecule manufacturing,” she sighs.

CONTRASTING FORTUNES

The ascension of Agnès Buzyn to the Health Ministry has also translated into diverging fortunes for different therapeutic areas. The signing of a National Rare Disease Plan, has, for instance, been embraced by firms active in that space such as CSL Behring. “This plan is well-crafted, and we welcome it with open arms. The first merit of the plan is to recognize the..."
WE LIKE TO SEE YOU EXCITED ABOUT PURSUING YOUR DREAMS

That is why, for almost 90 years, we have been providing the therapies you need: cutting edge treatments for disabling conditions and rare diseases, as well as treatments for everyday illnesses. On all 5 continents around the world, we want to encourage you to live life fully, TO LIVE... AND TO DREAM.
 fact that it can be difficult for patients afflicted by rare diseases to get access to effective treatments. Secondly, it affords structure to the provision of medical care to these patients. Lastly, it raises awareness among the larger population about the fact that there are a lot of different and serious rare diseases, more than 7000 in fact,” notes Franck Puget.

The local women’s health segment is also buoyant after the National Committee for Ethics (CCNE) delivered its stamp of approval for Medical Assisted Procreation. “This is an important step that affords women access to fertility services, in the same way of other EU nations. These patients will no longer have to travel to other countries to have a baby,” affirms Gede-on Richter’s Emmanuel Eumont.

Also overjoyed are vaccine developers after the Ministry authorized pharmacists to administer vaccinations in a bid to enhance accessibility and turnaround a strong anti-vax movement that has two-fifths of the population doubting that inoculations are safe. “Minister Buzyn has been commendably assertive and introduced scientific reasoning into the dialogue, backing the government’s preventative healthcare strategy with hard scientific data,” applauds David Loew of Sanofi Pasteur, which is investing EUR 170 (USD 193) million in extending its influenza immunization production capacity at its Val de Reuil facility in Normandy.

Neuroscience specialty companies, by contrast, have been hit hard by the controversial decision on the part of the Ministry to no longer reimburse Alzheimer’s therapies on the grounds that they don’t demonstrate sufficient proof of efficacy during clinical trials. “We believe that this is a distinctly unsound decision that effectively abandons an entire stratum of patients and the people caring for them. It sends out completely the wrong message at a time when these patients require reassurance and hope,” warns Lundbeck’s Lourdes Pla.

“The pathways of neurodegenerative disease are still relatively poorly understood, but we are beginning to discover that they manifest themselves in very individualized ways. Our therapies therefore lend themselves to the new era of personalized medicine but will not necessarily generate the same clarity of clinical trial results as traditional, classic molecules in clear-cut therapeutic areas like hypertension,” she elaborates.

**LAST CHANCE SALOON?**

With so much having been promised and considerable expectations now hanging on the execution of the reform agenda the mood tends towards cautious optimism.

“Many businesspeople have been rather impressed by the clear strategic direction emanating from the new Presidency. Already the new administration has enacted a plethora of changes to the labor code and tax regime with a view to reducing the burden on companies and drawing in new tranches of investment. I believe these steps are indeed serving to nurture a more business-friendly operating environment and to conjure a much more attractive picture of France abroad. The very strident pro-innovation and pro-technology orientation of the Edouard Philippe government has also created a strong sense of optimism within the life sciences industry at large. However, a transformation of this magnitude cannot happen overnight and there is a need to properly manage expectations,” counsels LFB’s Denis Delval.

Others draw attention to the sheer significance of what is happening. “There is substantial goodwill riding on this particular attempt to get the life sciences industry back on track so the time has come to start converting promises into actions. You could say we are in the last chance saloon because global management boards have been won over by the eloquent speeches and are now waiting for the substance. If those promises fall through, then reputations will be tarnished and attaining real progress will become even more difficult than before. That said, we are feeling the momentum and continue to believe,” contends Pierre-Claude Fumoleau.

“Frankly, there are alternative ecosystems out there at the moment that are managing to achieve more with a comparable resource base. At the end of the day, investment flows will always gravitate towards the markets offering the optimal value proposition, so there is no room for complacency or false dawns if France, or indeed any country, seeks to remain in the game... This is the golden opportunity, and it must be seized!” urges Ipsen’s David Meek. 🌟

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**Plant Power**

GREENTECH – founded in 1992 – is one of Europe’s foremost plant biotechnology pioneers; investigating and producing plant-derived molecules for a wide range of therapeutic areas, as well as across different industries.

CEO Jean-Yves Berthon explains GREENTECH’s mission thusly, “Essentially, we see our job as delving into the science and investigating traditional herbal-based medicine to ascertain the active compounds present in different plants and understand their corresponding therapeutic qualities. Then, harnessing state-of-the-art technologies usually only deployed in pharmacy, we are able to transform and convert these naturally occurring substances into active product ingredients.”

With 27 years of experience behind it, Berthon feels that GREENTECH now stands apart from its phytopharmaceutical competitors, noting that, “We are providing simultaneous solutions for the same marketplace, but from different origins – plants, algae and biotech – and can mobilize our expertise around the different disciplines to identify novel solutions.”
Despite major advances in the orphan drug development process, and recent important FDA drug approvals for patients with cystic fibrosis (CF), there is still a high unmet medical need among CF patients and a critical need for new treatments, particularly those with nonsense mutations who often have the greatest burden of disease and few, if any, treatment options. Increased collaboration and open communication between patient advocacy groups, industry leaders, and regulatory authorities can and must be a priority to accelerate research and development in CF, with the hope of getting new treatments to patients who need them as quickly as possible.

CF is a rare genetic condition with devastating physical and emotional impacts on patients and their families. Despite currently approved FDA-therapies, there are still no approved drugs for CF patients with nonsense mutations. These patients represent about 10% to 13% of the overall CF patient population.

At Eloxx Pharmaceuticals, we have a commitment to working with patient advocacy groups who share a similar vision of addressing this underserved patient segment.

At patient advocacy group meetings, we can learn so much from individuals with CF, their parents and caregivers, CF clinicians, other biotech companies who share their perspectives and experiences with the disease, currently available therapies, and future drugs in development. I’ve heard directly from patients and parents about the substantial effects CF has on their daily lives. I’ve also heard what patients would value in future treatment options.

Emily Kramer-Golinkoff is a CF patient who has the rare nonsense mutation, and none of the approved therapies have been able to help her. In one patient advocacy meeting I attended, Emily described her arduous daily routine, and shared that she has just learned from her physician that she had suffered a partial lung collapse. Currently, Emily spends countless hours a day in breathing treatments. In December 2011, Emily founded Emily’s Entourage, a nonprofit organization that fast-tracks research for new drugs and, hopefully, a cure for nonsense mutations of CF.

“The single biggest asset that we have is desperation,” said Kramer-Golinkoff. “It spurs creativity, it spurs disruptive thinking and innovation, and instead of condemning it, we should flip it and turn it into a valuable asset. It can be really challenging to keep pushing through. At Emily’s Entourage, we will keep fighting until each patient with CF has a long, healthy, and productive life.”

Last year, the median age of death for CF patients was 30 years, according to the CF Foundation.

As the chairman and CEO of Eloxx, a global biopharma company, I realize that we are in an urgent race against time to help these CF patients who currently have no options. We recently initiated a new program focused on inherited retinal disorders, and entered into a wide-ranging partnership with the Foundation Fighting Blindness. Its urgent mission is to drive the research that will provide preventions, treatments, and cures for the entire spectrum of retinal degenerative diseases. Our partnership with the Foundation Fighting Blindness will include broad scientific engagement supporting the Eloxx ocular portfolio development through scientific consultation, advisory support, clinical protocol reviews, and other activities.

Industry, regulators, and patient advocacy groups are in a unique position to provide insights and recommendations and work together to help accelerate drug development. We must encourage greater collaboration and new models, and the active participation of patients is an important step in overcoming many of the existing challenges in clinical development. It is vital to consider the particular needs of all stakeholders to ensure benefits from this increased engagement.

Transparency and open communication throughout the R&D process are essential. Orphan drug development has been a long, uphill battle, but due to recent legislation and greater collaboration between advocacy groups, industry, and government regulators, today, there are unprecedented opportunities for advancement. As a community, we must seize these opportunities with a goal of rapidly improving the lives of patients and their families.
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