Winter 2019

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Insider

An Inside Look at the #1 Life Sciences Cluster

What's Next in Healthcare IT?

John Halamka, MD,
Beth Israel Lahey Health;
Harvard Medical School

- Bob Coughlin on Why MassBio is Opening a New Conference & Business Center
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Chrissy Farr of CNBC speaks at Digital Health Impact 2019

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Introducing MassBio*Hub* in the Heart of Kendall Square

Over the years, we've heard from our members again and again just how hard it is to find affordable meeting and conference space in Greater Boston and Cambridge. Most are using their office real estate for lab space, and when our members do need to book an event, they're hit with crazy hotel minimums or limited availability. We've heard your calls—and we're coming to you with a solution.

In May 2020, MassBio will open a brand-new conference and business center in Kendall Square, the MassBio*Hub*, exclusively for our members. These modern facilities will be available for rent at below-market rates and boast a unique and flexible space that can accommodate groups from 2-200 for all your business needs, from informal staff huddles to full-scale conferences and corporate events.

The *Hub* comes with on-site concierge, seasonally inspired catering and hospitality services, and state-of-the-art technology. Members will also have access to creative branding and visibility opportunities on-site for day of event and beforehand. We'll handle all of the logistics so you can focus on what you do best—improving patient lives.

To inquire about the MassBio*Hub* or to book your next event or meeting, email us at hub@massbio.org or visit hub.massbio.org.

words of welcome

Robert K. Coughlin, President & CEO, MassBio

MassBio

Upcoming events

Rare Disease Day
February 28, 2020

State of Possible Conference: MassBio's Annual Meeting
March 25-26, 2020

Visit MassBio.org/events for details

State of Possible Conference

MassBio's Annual Meeting

March 25-26, 2020 • Royal Sonesta • Cambridge, MA

"It's the Woodstock of Life Sciences, with all star performers playing their greatest hits all on one stage."

—2019 State of Possible Conference Attendee

What's Next in Healthcare IT?

John Halamka, MD,
International Healthcare Innovation Professor, Harvard Medical School

Having spent the past 25 years building electronic health records, personal health records/portals, and health information exchanges—I cannot predict what the next 25 years will bring—but I can predict what the next few years will bring with reasonable certainty.

1 Hospitals and provider offices will embrace the cloud, making EHRs a subscription service rather than licensed software running on local servers and storage. The cloud provides a higher level of redundancy, better security controls, lower total cost of ownership, ease of integration across vendors, and more robust operations than most healthcare organizations can provide. All the major cloud vendors will now sign HIPAA business associate agreements, keeping data private.

There will be two challenges—moving from capital expenditures (owning stuff) to operating expenditures (buying services) and figuring out indemnification protection for cybersecurity risks. CFOs are accustomed to giving IT departments a fixed amount of capital to buy an asset—hardware and software. The cloud model is a subscription for services that comes from the operating budget, not the capital budget. Although moving to a subscription approach is the right thing to do, it will cause a few rocky budget cycles during the transition. Cloud service providers typically will not indemnify customers in the case of a security breach. That means that healthcare cloud customers will most likely want to purchase cyber-liability policies to de-risk the consequences of cloud-based security incidents.

2 Patients will embrace more mobile and wearable healthcare. Patients don't ask for healthcare technology specifically; they want convenience, wellness, and lower costs. Apps will help patients/families get an appointment, manage their medical records, be compliant with care plans, minimize errors in medical decision making, and communicate with their care team. Wearables will gather telemetry on everything from vital signs, sleep, ECGs, weight, and even selected diagnostic tests. The challenge will be figuring out what to do with this data—who reviews it, who stores it, who is accountable for alerts/reminders/decision support generated from it? My prediction is that accountable care organizations will develop a new class of clinical staff—the care traffic controller/care navigator who, using innovative customer relationship management software, will ensure that a patient gets the right care, in the right setting at the right cost. Wearable data will be sent to the care navigator and not a specific clinician. Another challenge will be understanding the accuracy/precision of wearable data—what to act on and what to ignore. That's still a work in process for the industry.

3 Machine learning will change everyone's experience of healthcare. I do not believe that artificial intelligence will replace clinicians, but it will supplement many aspects of practice and enable them to better practice at the top of their license. Radiologists and pathologists will have new tools that help them focus on specific images with abnormalities. Primary care clinicians will be presented with a dashboard of potential diagnoses for each patient based on time of year, location, patients' genomes, historical patient experience, and local community disease prevalence. Patients will be sent to the right provider based on signs/symptoms.

4 Although EHRs will still be important, the agile innovation will come from apps and cloud services connected to EHRs using the Fast Healthcare Interoperability Resources (FHIR) standard for healthcare application programming interfaces (APIs). Hundreds of new companies will become part of a new app ecosystem that improves healthcare workflow for all stakeholders—payers, providers, patients/families, pharma, and researchers. Data will flow much more easily with better security using the Argonaut implementation guides for FHIR/OAuth/OpenID. Clinician burden of data entry and patient burden of care navigation will be reduced through this new app ecosystem and APIs.

5 Telemedicine will increasingly become the norm—only rarely will you need to drive to a clinician office, pay for parking, and sit in a waiting room. Imagine this new workflow:
You are concerned about a skin discoloration—is it cancer? You take a photograph of it with your phone and send it to a cloud hosted service which notes it is unlikely to be cancer but there are three specialists in your area who can see you to confirm. You review their experience and quality metrics. You pick one and book an appointment on your phone. In a few hours you have a remote visit and remote examination. A care plan is developed, and you are reassured. Costs are low, quality is high, and everyone wins.

As reimbursement moves from fee for service to value-based purchasing, incentives will be aligned to keep patients healthy. Such new workflows will be an essential part of reducing costs and ensuring wellness.

There you have it—if someone tells you that the future of digital health is cloud-hosted, mobile, wearable, machine learning enhanced, and API integrated, you can believe them! ■

John D. Halamka, MD,
leads innovation for Beth Israel Lahey Health. Previously, he served for over 20 years as the chief information officer (CIO) at the Beth Israel Deaconess Healthcare System. He is chairman of the New England Healthcare Exchange Network (NEHEN), and a practicing emergency physician. He is also the International Healthcare Innovation professor at Harvard Medical School. As the leader for innovation at the $7-billion Beth Israel Lahey Health, he oversees digital health relationships with industry, academia, and government worldwide. As a Harvard professor, he has served the George W. Bush administration, the Obama administration, and national governments throughout the world planning their healthcare IT strategy. In his role at BIDHS, Dr. Halamka was responsible for all clinical, financial, administrative, and academic information technology, serving 3,000 doctors, 12,000 employees, and 1,000,000 patients.

MassBio's 2019 Transportation Survey Reveals That

60% of Respondents Would Change Jobs for a Better Commute

The worsening transportation in Massachusetts is impacting employee satisfaction, hurting employers' ability to attract and retain talent

By Robert K. Coughlin, President & CEO, MassBio

Transportation is the first topic out of everyone's mouth no matter where I go. So, I wanted to quantify just how bad it's gotten—and what MassBio could do to help. That's why MassBio released a report on the state of transportation in Massachusetts, detailing the results of a survey of 2,133 professionals who work in or support the life sciences to better understand how transportation in Massachusetts is impacting the industry's ability to recruit and retain talent.

The verdict? We are in fact at a breaking point—traffic congestion and failures on the MBTA are worse than ever, and employees are making decisions on where to work based on their commutes. The majority of respondents said their commutes are worse than last year, and a whopping 60% of respondents said they would change jobs for a better commute. Nearly a quarter have even considered moving to a different state. Things are worse for those that take the commuter rail, as 76% of those respondents said they would change jobs for a better commute. That's not all that surprising given nearly 80% have been late for work due to delays on public transportation and almost 70% have been late for personal commitments.

Employers are taking steps to help ease their employees' commuting burden, but there's more work that can be done. Massachusetts is behind the nation in adopting formal work-from-home policies as only 4.7% of the workforce telecommutes full time, lower than in 19 other states, according to a report from FlexJobs. When asked about this in our survey, only 28% reported the ability to work from home at least one day a week, although 74% can work from home occasionally. Flex hours are more prevalent, with 75% of respondents utilizing this option, but this has limited impact as the rush hour in Greater Boston lasts longer than ever.

We, as employers and as a leading industry, can make a greater impact—at least in the short-term. MassBio recently enacted an employee mobility policy that allows our employees to work up to one day a week remotely while also allowing them to set flexible work hours to better avoid rush-hour. These are small steps and we are only 35 employees, so we asked our member companies to do the same, or adopt another policy that helps alleviate congestion and improve employee morale. The response was heartening. We heard that many of you already have such a policy, are already going above and beyond that, or are considering adopting something similar. If every company in Kendall Square and beyond agreed to take more employees off the road, we could make a collective impact on congestion without spending a penny.

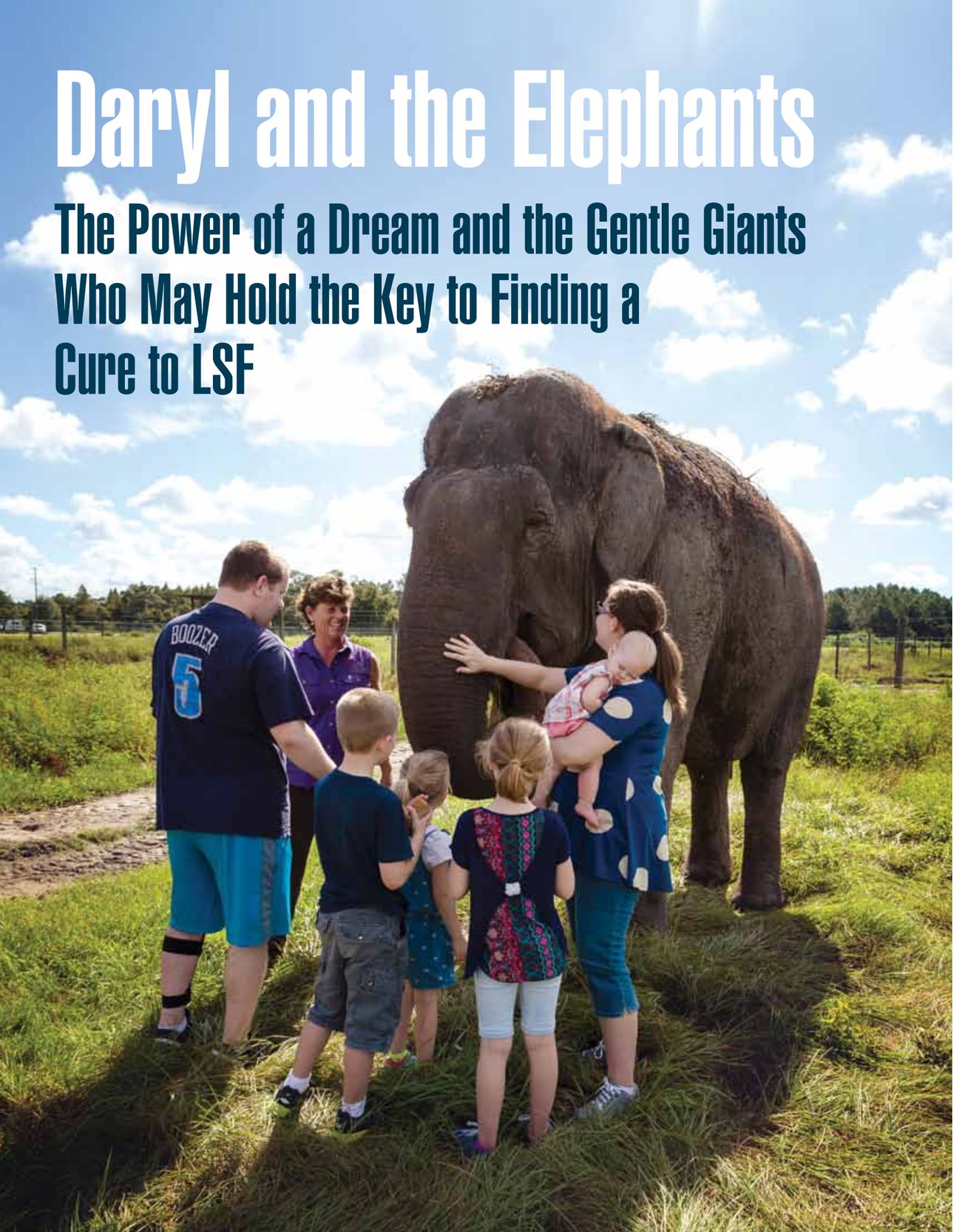
That doesn't mean MassBio won't do more to help address long-term solutions. MassBio is part of the Massachusetts Business Coalition on Transportation, led by the Greater Boston Chamber, that is seeking to develop solutions to the state's transportation issues. According to James E. Rooney, President & CEO of the Greater Boston Chamber of Commerce, "The findings from MassBio's survey are not unique to the life sciences, but reflect the experiences of those in all industries and show the urgent need for immediate, bold solutions. The business community is clamoring to be part of the solution and rightfully demand more accountability to ensure we get the world class transportation system our state needs to be competitive."

There is widespread support across the industry to come up with lasting solutions—not just to fix what's broken, but to support further growth. According to our survey, 82% of respondents do not think the Massachusetts state government is doing enough to improve the state's transportation systems, and 64% are likely or very likely to support increased taxes or fees to do so.

"MassBio's survey confirms what we've heard from employees across Kendall Square and across the Commonwealth: we are facing a transportation crisis and enough is enough," said C.A. Webb, President, Kendall Square Association. "We can't continue to drive Massachusetts' enviable economy forward if we don't fix our current transportation system and routinely plan for future needs. Ultimately, we can build the transportation system we all deserve and our economy needs. We must start now." ■

Daryl and the Elephants

The Power of a Dream and the Gentle Giants Who May Hold the Key to Finding a Cure to LSF



Daryl, a father of six children who had lost his mother to breast cancer when he was just 9 years old, had been living with cancer for several years. Tests conducted as part of his treatment had revealed the genetic mutation Li-Fraumeni Syndrome (LFS). People usually have two copies of a particular cancer-fighting gene but having LFS meant that Daryl had only one copy, making him more likely to develop the condition. Daryl was also devastated to discover that this genetic mutation was hereditary. Subsequent testing showed that three of Daryl and his wife Erica's six children also tested positive for LFS but were thankfully cancer-free.

The doctor responsible for Daryl's care, Dr. Joshua Schiffman, was at the time researching a possible cancer cure involving elephants as they have 40 copies of the cancer-fighting gene. Daryl provided blood samples that Dr. Schiffman was using along with others from people with LFS to study alongside the blood of some of the elephants at the Center for Elephant Conservation (CEC), a facility dedicated to the conservation, breeding, and understanding of elephants.

When Daryl learned that his cancer was terminal, he reached out to Dream Foundation, an organization that fulfills final dreams for terminally ill adults and their families across the nation. CEO Kisa Heyer explains, "Having a final dream come true can bring comfort or closure and plays an important part of a palliative care program. An independent study run in conjunction with the American Psychiatric Association showed that 80% of Dream recipients reported a greater sense of well-being after a dream."

Daryl told Dream Foundation that he wanted to take his family to the CEC to meet the gentle giants who could hold the key to hope for his children. Erica remembers the time very well.

What do you most remember about the time when Daryl received his diagnosis?

It was about a year and a half into our marriage. His mother had passed away from cancer, but he never got tested for LFS. I pushed him to get tested. He had all sorts of tests and an MRI showed that he had a brain tumor. It was a huge shock as he had no symptoms and we had no idea it was coming. We were both really young, we had an 8-month-old baby and we were expecting our second child. We thought we had a huge long life ahead of us. They told us that whatever treatment he had, it was a type of tumor that would keep coming back and that one day it would get the best of him. That was really disheartening.

Were there many treatments for him to consider?

We were cautious of chemotherapy and radiation. Daryl's father and two cousins had passed away from brain tumors and after they had chemotherapy their health spiraled down, so the only option was surgery.

Was there anything that offered Daryl hope or solace in his last few months/years?

Quite by chance, Dr. Joshua Schiffman, who was responsible for Daryl's care, was at the zoo one day with his children and they went to see the elephants. The elephant keeper mentioned that they rarely got cancer. Dr. Schiffman asked if he could have some of the blood that they drew from the elephants every day as part of their overall care program so he could start his research. First it was just Dr. Schiffman on his own, but now there's a whole lab and a team. Daryl wanted to be part of it, so his blood was used.

At one point when Daryl was really sick, we went up to visit the lab. I remember the look on Daryl's face. He was so excited.

On the morning that Daryl went in for his final surgery, Dr. Schiffer's research had been published in a renowned medical journal and there were hundreds of articles all over the world about it. Daryl got so excited. I think deep in his heart it was the right thing to give him hope.

And then there was Dream Foundation. A friend told me about this dream-granting organization for terminally ill adults that had made her husband's dream come true. I told Daryl, and he immediately said that he would love to go and see the elephants in Florida.

On that trip, I watched Daryl staring into the eyes of the elephant and petting its trunk and saw a flame of hope and gratitude in his eyes. That night, Daryl had a dream

that an elephant took its trunk and was able to go into his head and scoop out his brain tumor.

What do you and your children most remember about this trip to see the elephants and how did this impact your final moments with Daryl and your ability to cope with what was happening?

Daryl passed three months after that trip, nearly three years ago. The longer you go, the more you forget, and the kids just remember their dad being sick or sleeping or being in the hospital, so that trip stands out as the one really positive thing in that time frame of remembering their dad.

It was great to have that time to be together in the moment where cancer didn't matter, where we got to be mom and dad and the kids. Right after we got home, Daryl started spiraling down so it was probably the last time our kids interacted and had real conversations with him.

What is your advice for others out there who are either sick or have a loved one who is sick?

I think the greatest thing you can do in those moments when you're dealing with terminal illness is really just spend that time together. It's difficult to put things aside. You have to keep life running, but something I wish had done more was reach out to our family, community, and neighbors and let them deal with everyday things so we could just sit and be together. ■

Postscript

Erica Means gave birth to her and Daryl's seventh child three months after Daryl passed away. The infant tested positive for LFS. Dr. Schiffman's research continues.

By Kendalle Burlin O'Connell, COO, MassBio

The famous adage, "a rising tide lifts all boats," has never been truer in biotech. Our collective successes as an industry will benefit patients across the world, many of whom are waiting for a single viable treatment for an unmet medical need. That was the impetus behind the continued expansion of our member savings program, MassBio *Edge*, previously the Purchasing Consortium, which will now operate under New England *Edge* and aggregate the purchasing power of BioCT and Vermont BioSciences Alliance member companies. This new program will allow a greater number of life sciences companies and employees to benefit from competitive discounts and have a stronger presence in the marketplace, so they can put more money towards the innovation that will improve patient lives.

Founded alongside MassBio in 1985, MassBio *Edge* is now leveraged by 75% of MassBio's 1,200+ members and saves each an average of $100,000 a year. We've seen great success for our members, but to truly support the small biotechs that are the backbone of the industry, we wanted to bring the value of this program beyond the Massachusetts biotech hub.

For MassBio, BioCT, and Vermont BioSciences members alike, New England *Edge* ensures that even the smallest startups receive the same competitive pricing for goods and services, such as lab supplies, office supplies, bulk gas and compress gas, as the largest biopharma companies. Boasting an impressive roster of Preferred Partners, which were carefully selected to join the program, members will have premiere access to:

- Fisher Scientific *(lab supplies)*
- Veolia Environmental Services *(hazardous waste removal)*
- W.B. Mason *(office supplies)*
- Middlesex Gases & Technologies *(packaged cylinder & bulk gases)*
- LabCloud, Inc. *(e-Procurement)*
- UPS *(carrier shipping)*
- And many more!

To learn more about how we can help your company focus on innovation, not budgetary confinements, visit **MassBio.org/purchasing-and-rewards.**

Breaking into the MA Innovation Ecosystem
The Journey of a MassCONNECT® Grad

Q&A with Kareem Barghouti
Co-Founder and CEO, VastBiome

As the #1 life sciences cluster in the world, Massachusetts presents a unique allure to life sciences companies across the globe. But, for startups on the path to commercialization, breaking into this ecosystem—without access to its expansive network—can often coincide with the "valley of death." To get a first-hand look at this journey, MassBio's Chief Business Officer, John Hallinan, recently sat down with Kareem Barghouti, Co-Founder and CEO of VastBiome, a recent MassCONNECT graduate.

Why did you decide to come to Massachusetts when you're based in the Bay Area and got your start in Houston? What does the MA ecosystem offer that these areas don't?

We started in Houston as part of the Texas Medical Center Innovation Institute (TMC). Our company spun out of the TMC Biodesign Fellowship program where we spent a year identifying unmet needs within the Houston medical community. VastBiome was born through a number of discussions and observations with various medical professionals expressing the value of understanding the role of the microbiome in treating disease.

Following the fellowship, we moved to the Bay Area, where most of our team is from, to establish ourselves and participate in a number of accelerators (Illumina Accelerator and Plug and Play).

What intrigued us about coming to Massachusetts was the biotech ecosystem that Boston/Cambridge has built and the various microbiome companies that have established themselves in the region. We had not yet made an effort to grow our network in MA until a friend of ours who completed the MassCONNECT program recommended we apply. Once accepted, we thought it would make sense to participate and hopefully make a few strong local connections.

Looking back after completing the 8-week MassCONNECT program, this may have been the most productive and fruitful two months of our company's existence. The sheer density of top-quality talent and expertise is second to none. Within a few square miles, you have access to industry leaders who are willing to meet and mentor you. We spent months prior trying to get in front of various prospective partners based in MA. Spending the summer establishing our MA network had accelerated our company's trajectory for fundraising, product, and business development strategy. While other cities have the resources, the energy and community that MA has built around the biotech industry is truly special. A company's chances to succeed are exponentially greater when instilling themselves within the MA network. The knowledge and resources at your disposal from both academia and industry enables a company to fast track their learnings and prevent inevitable mistakes by connecting with various individuals to provide high-value mentorship.

Biotech is a highly competitive industry. The MA ecosystem allows companies and individuals to open up and support one another's growth. You can't enforce such actions. They are built over time, trust and respect.

Your technology represents the convergence between digital tech and biotech—how has this presented both challenges and opportunities for your company and for patients? How are you getting the expertise needed on both sides?

VastBiome's core competency sits at the intersection of computation and science. Until recently, it was not possible to sequence and analyze data at an affordable and scalable rate. These technological advancements have enabled various opportunities for tech to drive science into new territories of exploration and for small companies, like VastBiome, to have a fighting chance to make it in what is known to be a capital- and resource-intensive field. That said, with the barrier of entry reduced, this opens up the field for various new companies to enter the market, including us. While great for the field, this comes with significantly more competition and what could be considered "noise." The terms "artificial intelligence" and "machine learning" have begun to lose their allure and are considered buzzwords that, in most cases, are not being applied to the level of driving true value creation. Industry partners and investors have become numb to these technologies, setting a high bar to get buy-in—especially when some groups have abused the value of what technology can bring to the field of medicine, promising more than they can deliver. This is increasingly concerning when it comes to trusting these technologies in treating patients, which is why we all got involved with the field to begin with. While technology is powerful and can lead to great discoveries, keeping the trust of the patients and ensuring they are not put in a potentially harmful situation is critical. VastBiome addresses these concerns by ensuring that we are surrounding ourselves with both tech and biotech professionals. These industries take very different approaches to solving large unmet needs and their business models are quite different. Tech expects short-term returns while biotech companies are expected to wait 10 years to commercialize a potential asset. Understanding those differences and creating a hybrid approach to minimize risk as best we can is something we spend a lot of time on at VastBiome.

How has the MassCONNECT program helped accelerate your path to commercialization?

MassCONNECT has helped us develop a clear understanding of our path forward in both the short-term and long-term. Being put in front of 15 mentors weekly for eight weeks and presenting to them a number of burning questions is an invaluable opportunity. You are getting access to the experience and knowledge of industry veterans who have held senior positions at Fortune 500 companies. Companies typically spend tens of thousands of dollars to get access to this type of network. In addition, the variety of perspectives helps paint a picture of how key stakeholders think about specific questions/ideas that we've raised. It is up to the company to digest the feedback and decide what is the best action forward.

VastBiome has accelerated our path to commercialization by having determined where to spend our capital and time to deliver the highest value to best position us for our seed-round milestones. We have refined our business model to clearly illustrate our value and how we plan to grow into a full-fledged drug developer. Furthermore, we have established a strong network of individuals who are willing to support our efforts, covering the entire spectrum of expertise needed to build a drug discovery company (e.g., business development, IP strategy). Overall, we would not be where we are today if we had not participated in MassCONNECT.

Kareem Barghouti is the co-founder and CEO of VastBiome. He received his BS in Biological Sciences at UC Irvine and an MBA from Duke University with a concentration in Healthcare Sector Management. His background is in Strategy and Operations, and Sales and Marketing. Kareem joined the fast-growing startup Wildfire Interactive as a Business Development rep which was acquired by Google for $450M. He spent five years at Google leading the data integrity of its advertising business, managing teams of 100+, and was part of the core product team to launch an online education portal to millions of users globally. He was recognized as the top sales rep globally (1000+ reps) for the New Business Sales team. Before Google, he co-founded an online marketplace (StingyCampus.com), having raised $250K in seed funding and launched the program across twelve universities in Southern California. Most recently, Kareem participated as a Biodesign Fellow at the Texas Medical Center Innovation Institute in Houston, Texas, where VastBiome was born. ■

Value of Health Series

Breaking Down the Value Equation to Ensure Patient Access to Breakthrough Therapies

MassBio's Year-long Initiative Explores the Future of the Biotech Industry Through the Prism of Drug Pricing

By Jennifer Nason, Director of Communications, MassBio

For a growing number of therapies, especially specialty drugs, manufacturers can no longer assume payer coverage and patient access based on regulatory approval alone. Increasingly, manufacturers are being asked to first demonstrate the "value" of the drug—to patients and to the healthcare system—and thus, justify its price, before being added to a payer's formulary.

This demonstration of value will ultimately determine who has access to new therapies, but it also determines how patients gain that access. Value determinations can influence whether payers add additional barriers to certain drugs, such as requiring prior authorization from physicians or implementing step therapy, which forces patients to fail first on certain drugs before trying others. The challenge? There is no objective or shared measure of value.

That's the impetus behind MassBio's Value of Health Series, a year-long initiative to explore the future of the biotech industry through the prism of drug pricing. The goal of the series is to educate biopharma companies, especially those in early and mid-stages, about the current and expected environment they're operating in, and considerations these companies must make to ensure patient access when their drug comes to market. Drawing insights from a MassBio advisory group and thought leaders in the industry, we've released three separate whitepapers on the issue and as of this writing, have held two of the three related events.

Part I of MassBio's Value of Health series examines the value equation from the perspectives of the various healthcare stakeholders, the increased use of health technology assessments (HTAs) in the US in determining value, the current state of innovative payment methods with private payers, and the importance of real-world evidence in demonstrating the long-term value of therapies. We looked at real-world case studies from companies who are leading the way in value-based agreements, and the increased use and impact of HTAs, like the Institute for Clinical and Economic Review (ICER), in determining the cost-effectiveness of new therapies.

While Part I focuses almost exclusively on how to best demonstrate value to private payers, the public insurance market in the US is a critical piece to understand as it covers more than one-third of insured Americans. So, in Part II we explore this in detail, breaking down Medicare and Medicaid in the US, existing access and reimbursement issues for innovative therapies and cures, proposed changes to those programs, and barriers that exist to innovative payment models. We looked at how Gilead Sciences was able to overcome some of those barriers and engage in a subscription model with Louisiana state to treat its hepatitis C (HCV) patients, as an example of how innovative payment models can be executed within state Medicaid programs.

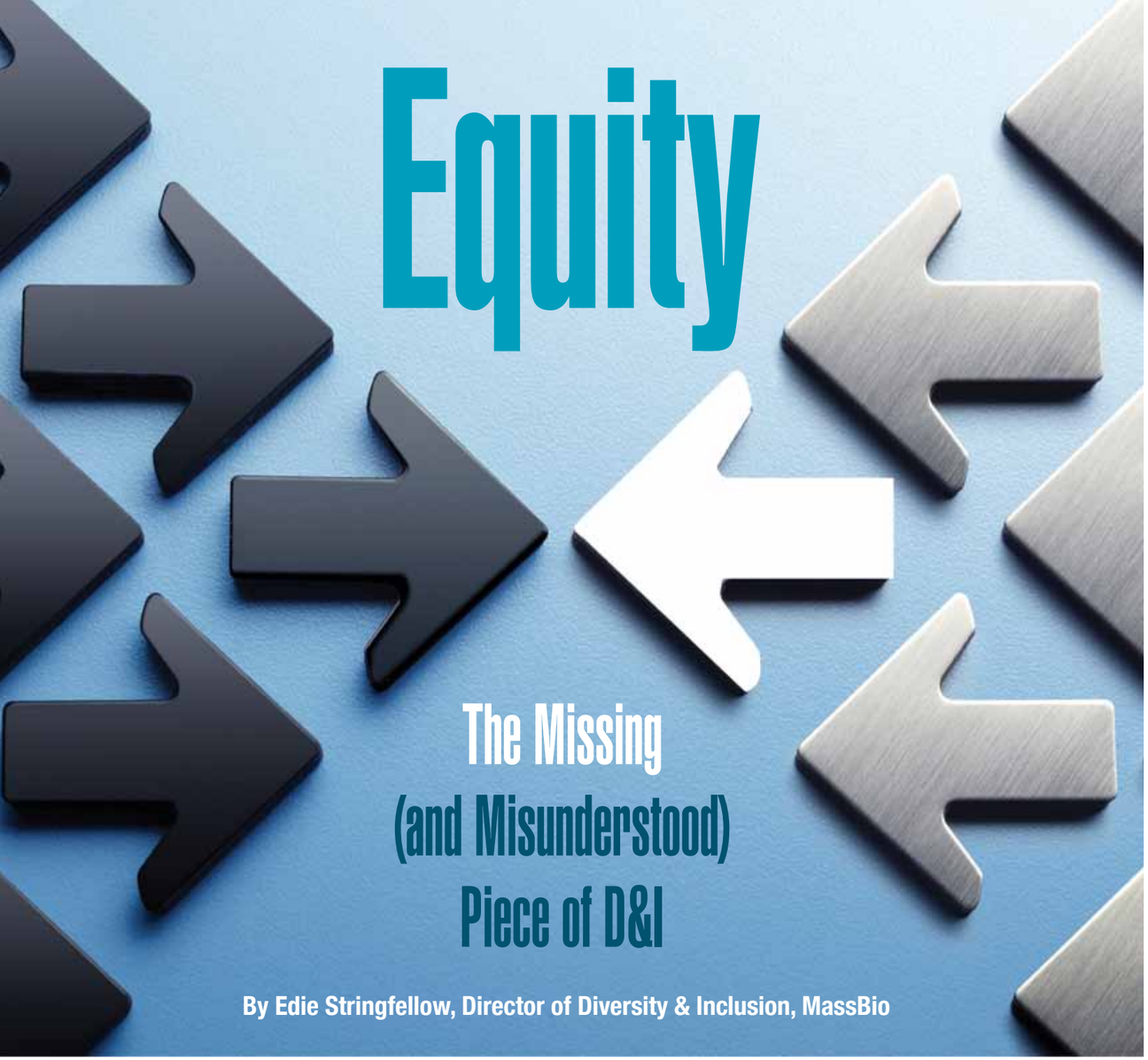
We also considered those key international models of assessing value and determining access and what the US may be able to learn from them. In the highly developed healthcare markets within Europe and elsewhere, single-payer or government-directed payers each have their own processes and metrics, but through broad reference pricing across markets, have important influences both within and beyond each country's borders. Emerging markets like China are evolving at an incredible pace, presenting new opportunities for manufacturers to engage, but also posing challenges around how best to do this. We found that successfully bringing new therapies and cures to patients, regardless of the market or country, shares a common theme: clearly demonstrating the drug's value to payers is critical to ensuring access and reasonable reimbursement. Public payers are no exception and while the need, methods, and opportunities to show that value differ, together Medicare, Medicaid, and international markets strive to provide the best value to the people they cover and the governments they work for.

Lastly, Part III of the series considers disruptions to the ever-evolving value, access, and reimbursement landscape as new players enter the arena and others consolidate. What impact will mergers between payers and PBMs have on drug pricing, patient access, and the ability to track and analyze data? How are digital technologies changing drug discovery and development and how patients access treatment? What new players are disrupting the value equation? Part III attempts to answer those questions and more.

For MassBio and our membership, this issue is especially timely. We're at an inflection point, with science that's been tested for decades finally becoming a reality for patients and a payer system that has not caught up with this level of innovation. Myriad factors are combining to alter the status quo—with policymakers demanding action, and healthcare stakeholders agreeing that we must come up with real solutions. If we don't come together and address the value equation as an industry and ensure patient access to all new therapies, government or other stakeholders will—and they will likely get it wrong. It is our belief that the sooner companies start thinking about value and how they will demonstrate it, the fewer hurdles to patient access will exist. ■

Learn more at **MassBio.us/value-of-health**

"This demonstration of value will ultimately determine who has access to new therapies, but it also determines how patients gain that access."



Equity

The Missing (and Misunderstood) Piece of D&I

By Edie Stringfellow, Director of Diversity & Inclusion, MassBio

By many measures, life sciences companies have made progress improving diversity and inclusion (D&I) across the industry. However, one component of a comprehensive D&I strategy is often misunderstood at best and forgotten about at worst: equity.

Diversity, inclusion, equity, and equality are often used interchangeably, but they should not be—each has a specific meaning. After meeting with dozens of company leaders I've found that some are confused by the distinction and some have difficulty using these terms to generate meaningful, lasting action. For the purpose of this article we are going to focus on equality vs. equity and discuss why equity is important, and how it impacts our industry.

Equality is when everyone is treated the same way and has equal opportunities to contribute and excel.

However, equality is based on an assumption that we all started at the same place and need the same tools to be successful. That's where equity comes in.

Equity is about leveling the playing field recognizing that we all don't begin at the same point and some people do have built-in advantages. Without it, you can't effectively acknowledge structural 'isms' (sexism, racism, ableism, ageism) that negatively impact our industry and our communities, ultimately making D&I initiatives less sustainable. After all, you can't fix a problem without addressing the root of the issue.

Why does 'equity' matter?

Equity is not about compliance (reactive and transactional). It is about commitment (proactive and transformational). Equity, diversity & inclusion (ED&I) is about the full deployment of a variety of resources for better outcomes that benefit everyone in a sustainable way —employees, the organization, and thus, the patient community.

What impact does equity have on the industry and society?

Without a proactive commitment to equity, a company's D&I efforts will not be impactful or sustainable. Hiring and retaining diverse talent is only the first step. Equity will ensure that all employees have the same opportunity for promotions and future success.

With so many organizations in the life sciences focused on improving D&I, we must consider why we haven't achieved the depth of change that we seek. Inequity exists as a result of years of hiring practices that have historically excluded marginalized groups. Creating an inclusive culture through equity requires an intentional, adaptive, and transformational approach that impacts behaviors and mindsets, as well as policies, practices, and programs.

Equity means more than treating persons in the same way—it requires action on and the accommodation of differences. At a minimum, an equitable workplace has transparency in hiring, promotions, consequences, and rewards. But it must also provide the training and professional development opportunities needed to correct the conditions that created disadvantages for these marginalized groups. Only then can a workplace encourage a sense of belonging and authenticity for all its employees.

Equality doesn't mean Equity

Here are some real-world examples from MassBio members:

- Member A reported that their equity efforts have led to:
  - *A 95% retention rate of those who participated in a 6-month mentoring program;*
  - *The appointment of two senior female executives to corporate boards; and*
  - *An increase in the number of diverse Medical Science Liaisons to work with clinical researchers and drive more diverse participation in clinical trials.*
- Member B's 'Sponsorship Plan' develops senior leaders and advances careers. Over the course of a year, the person being sponsored meets with an executive coach once a month and shadows key leaders and receives their feedback. The goal of this initiative is to bridge the skills gap and diversify the senior ranks.
- Member C is transparent about reporting ED&I metrics across business units and feels that this brings accountability. Internal transparency builds trust and confidence, while external transparency allows a company to lead by example.

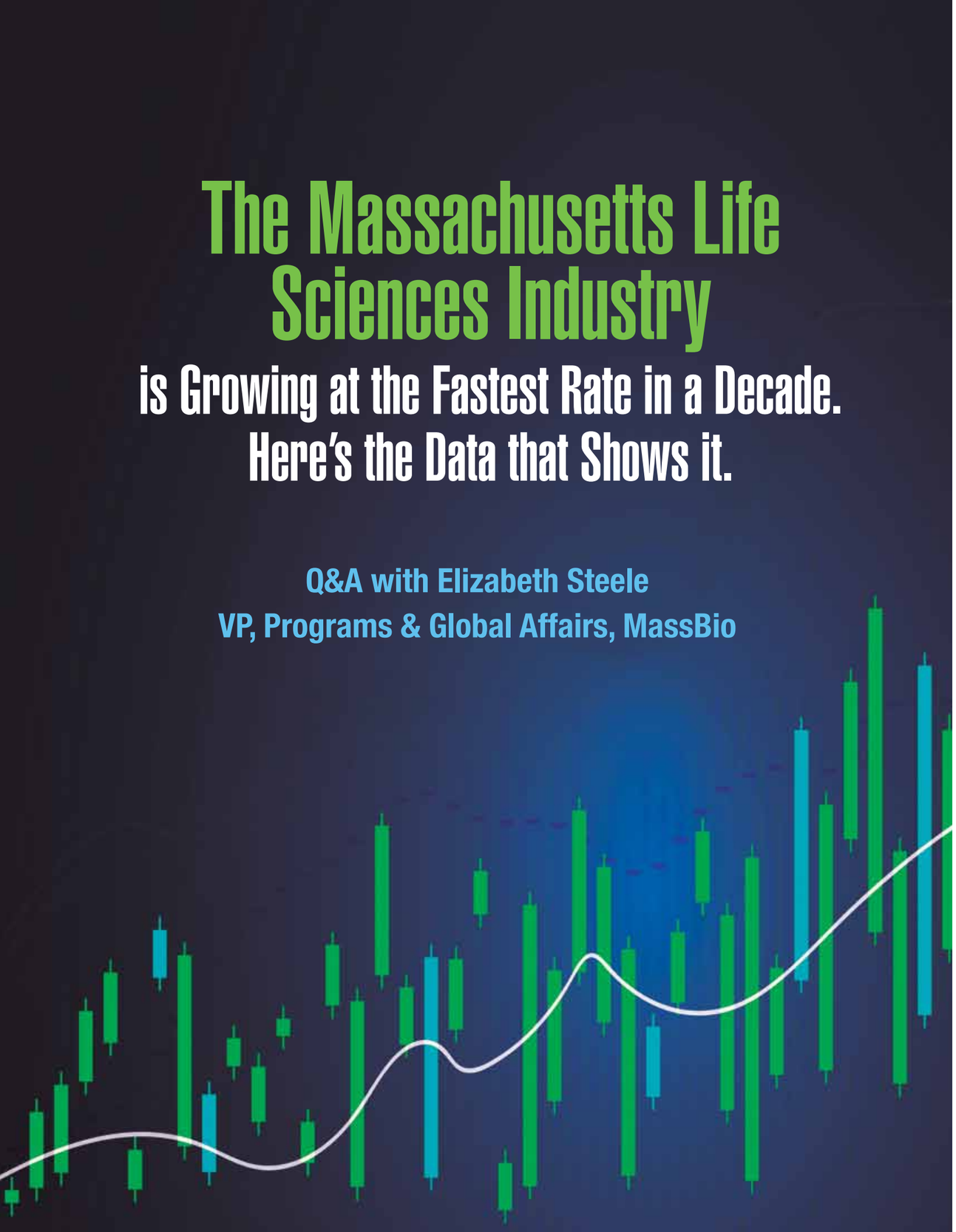
Having a diverse workforce within an inclusive and equitable environment contributes to increased retention, innovation, and productivity; an organization's responsiveness to an ever-evolving diverse world; and the industry's ability to cope with change. By building inclusive cultures through equity, we can begin to dismantle the structural 'isms'. Only then will we truly live up to our missions. ■

Learn more about MassBio's D&I efforts at MassBio.org/diversity

The Massachusetts Life Sciences Industry

is Growing at the Fastest Rate in a Decade. Here's the Data that Shows it.

Q&A with Elizabeth Steele
VP, Programs & Global Affairs, MassBio



In August, MassBio released its annual Industry Snapshot report, examining the life sciences industry in Massachusetts throughout all of 2018 and the first two quarters of 2019. To get a better idea of the results and what they mean for the future of the industry, we sat down with MassBio's VP of Programs & Global Affairs, Elizabeth Steele, who authored the report.

How has employment in the biotech industry grown in recent years and do you predict this trend will continue?

In the last decade, the Massachusetts biopharma industry grew by 35%, adding close to 20,000 jobs. This includes 4,300 jobs that were added from 2017 to 2018, an increase of 6.4% and the highest year-over-year employment growth in over 10 years. If we dissect those numbers further, we noticed incredible growth of the biotech research and development sector. R&D jobs grew by 50% in the last decade, highlighting the pace of innovation and the overall capacity of the industry to bring new, breakthrough treatments to patients around the world. However, continued growth is threatened by our state's aging transportation system and lack of affordable housing. Traffic congestion has reached unprecedented levels and the MBTA is in a state of disrepair. To ensure Massachusetts remains the best place in the world for the life sciences well into the future, we must take action now to not only fix what's broken, but to also create the transportation system we need for the next 20 years. That's why MassBio is part of the Massachusetts Business Coalition for Transportation, joining business and community associations across the state to develop lasting solutions (learn more on page six).

MassBioEd predicts that by 2024 there will be 12,000 additional jobs that need to be filled. What is being done to ensure we have a robust pipeline of talent?

Talent remains Massachusetts' greatest natural resource. Yet, with tens of thousands of jobs to be filled in the coming years, we need to look everywhere to ensure the best and brightest have access to our industry. That's why MassBio is a founding member of Project Onramp—an internship program that connects first-generation and/or low-income college students to the biotech industry. The first class of 52 students were placed in paid internships in 30 life sciences companies, and the program is set to continue through 2020 and beyond. This is in addition to the great work that MassBioEd, our sister organization, is doing to get young people excited for careers in the life sciences.

Did you notice a shift in the venture capital landscape for 2019?

Investment in biopharma reached an all-time high in 2018, with $4.8 billion of venture capital investment in Massachusetts biopharma companies. When looking at the first two quarters of 2019, Massachusetts biopharma companies raised just under $1.5 billion—a dip from the first half of 2018 but still in line with past years and an impressive amount overall. Of those investment dollars, Cambridge-based biotech companies received 63% of all biotech VC investment to the state in 2018.

Cambridge and Boston are home to some of the most renowned universities, hospitals, and biopharma companies in the world, but we're actively working to spread the success of this industry throughout the Commonwealth. Mini clusters are beginning to sprout across Greater Boston and out to Worcester and beyond. We're hopeful that in next year's snapshot we'll see VC funding invested in companies throughout the state.

In what ways do patients benefit from Massachusetts' thriving life sciences cluster?

Massachusetts-headquartered companies have developed therapies that treat patient populations of up to 265 million patients in the United States, and 2 billion patients around the world. For sick people, Massachusetts represents the greatest hope for new tomorrows.

Based on the data, the impact to patients of drugs developed in Massachusetts is only going to grow. Massachusetts is home to four of the top five NIH-funded independent hospitals and receives 56% of all NIH funding to independent hospitals. On top of that, 18 of the top 20 biopharma companies in the world have a physical presence in the state. As a result, much of the R&D of cutting-edge, new therapies is happening right here, with many nearing approval. Massachusetts-headquartered companies currently have 37 drug candidates pending FDA approval, and 2,253 in the pipeline. This represents 14% of the US pipeline for new therapies—all from one state. Massachusetts truly is the State of Possible. ■

To learn more about the state of the industry in Massachusetts, visit MassBio.us/industry-snapshot

Policy Spotlight

If Healthcare Cost Growth Has Slowed, Why Are Health Insurance Costs Skyrocketing?

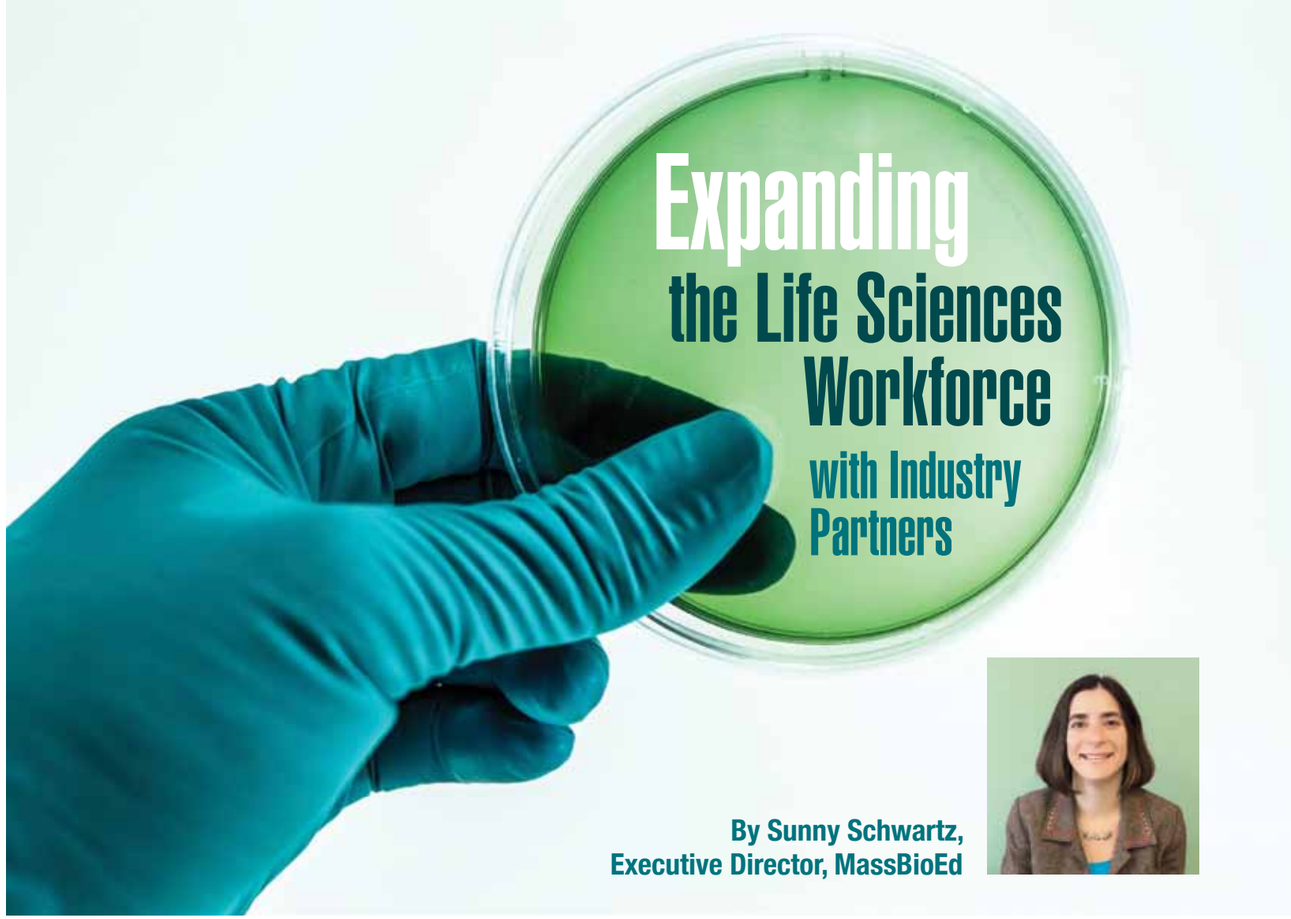
By Zach Stanley,
VP of Public Affairs, MassBio

In October, CHIA, Massachusetts' healthcare data collection agency, released their Annual Report examining healthcare spending, coverage, data, and related trends. The report's primary goal is to assess if total healthcare expenditures grew between 2017 and 2018 and by how much. The verdict: "Total health care expenditures (THCE) totaled $60.9 billion in 2018, or $8,827 per capita;" equaling a 3.1% growth rate between 2017 and 2018. That's the third year in a row spending growth has been at or below the benchmark growth rate set by state regulators—unqualified great news.

Yet, despite this consistent restraint in spending growth, health insurance premiums are growing rapidly. Between 2017 and 2018, when CHIA measured the THCE growth rate at 3.1%, fully-insured premiums increased on average by 5.6%. The average premium for a family in Massachusetts is now over $20,000 a year (the third highest rate in the country), and for one in 10 families, total premiums exceed $30,000. A 5.6% average increase in premiums may not seem like much but it has a real impact on patients and families across Massachusetts. The data shows Massachusetts employees directly paid 26-30% of their total premium costs—equaling more than $1,500 a year, on average, for one person and up to $9,000 for a family.

For years, payers have explained away significant increases to consumers' premiums and out-of-pocket costs by blaming the rising costs of pharmaceuticals. This message has resonated with policymakers who are also hearing from their constituents about how expensive their prescription drugs are at the pharmacy counter. This year's CHIA report shows both assertions to be false: spending on prescription drugs only grew 3.6% between 2017 and 2018 and is the third smallest category of healthcare spend, and the fourth smallest contributor to the change in total healthcare expenditures in Massachusetts during that time period.

As policymakers in Massachusetts and D.C. consider legislation that seeks to lower the cost of prescription drugs, we urge them to recognize that the market is already working well to restrain drug cost growth. Instead, they should be examining why health insurance out-of-pocket costs and premiums are growing at twice the rate of inflation and substantially faster than the state's at-benchmark 3.1% total healthcare expenditure growth rate. ■



Expanding the Life Sciences Workforce with Industry Partners

By Sunny Schwartz,
Executive Director, MassBioEd

I am thrilled to join the Massachusetts Biotechnology Education Foundation (MassBioEd) as its Executive Director. I have spent my career in the workforce development field, both developing and implementing education and training programs as COO of the Asian American Civic Association and serving as a funder, partnership builder, and policy leader as the President and CEO of the MassHire Metro North Workforce Board. I am excited to bring that workforce development expertise to MassBioEd as we seek to grow and enrich talent for the Massachusetts life sciences workforce.

Given the historically low unemployment rate in Massachusetts, combined with an industry that has grown by 35% in the last decade, life sciences companies are struggling to find and retain the workforce they need to be productive. MassBioEd is poised to work with industry and academic partners to expand the life sciences workforce pipeline to alleviate this gap in labor supply and demand. While assisting companies in hiring and developing a talented workforce, MassBioEd seeks to expand opportunities for residents to enter the biotech field so they can lead prosperous careers in an industry that helps patients and addresses unmet medical needs.

Through our valuable network, MassBioEd directly engaged 1,000+ students in exploratory college and career experiences in 2018-19, of which 92% reported an increased interest in pursuing a career pathway in the industry. We couldn't do this without the continued support of the life sciences industry—and I thank you for all you do. If we work together to engage and excite the next generation of life sciences professionals, I'm confident the future for patients around the world will be even brighter. ■

Learn more about MassBioEd and get involved at MassBioEd.org.

Member Spotlights

Each month, MassBio spotlights a member company and the great work they do to advance the life sciences industry and support the patients we serve.

Lyndra Q&A

In July, we spoke with Tyler Grant,
Director, Product Engineering at Lyndra Therapeutics. Tyler was one of the first two people to join the company after its founders. He has a PhD in Biomedical Engineering from the University of Oxford and completed a Postdoctoral Fellowship at the Langer Lab at MIT.

Tell us about your organization, its mission and current initiatives.
Lyndra Therapeutics aims to change how people take medications to improve healthcare. Non-adherence to medication is a global epidemic that costs up to $300 billion a year in the US alone. Rather than try to change patients to improve adherence, we're changing the pill. Our mission is to make daily pills a thing of the past by developing the first ultra long-acting oral pill that lasts a week or longer.

Our near-term focus is on small-molecule drugs where there's an unmet need in therapeutic performance. Real-world outcomes in many diseases depend on taking the right amount of medication, with consistency. Lyndra's platform could help ensure that patients consistently get the right dose, improving health outcomes.

How do your organization's activities help patients now and in the future?
Our current pipeline is focused on developing drugs for diseases where replacing a daily pill with a weekly or monthly dose would improve adherence and outcomes. For instance, as many as 75% of patients diagnosed with schizophrenia relapse as a result of medication non-adherence. As we advance toward filing an application with the FDA to begin clinical trials for a once-a-week dosage of risperidone, a medication used to treat schizophrenia, we're moving closer to getting a long-acting oral treatment to patients.

We're also working on a sustained-release drug for Alzheimer's with our partner Allergan to extend the time a patient can perform simple daily tasks without full supervision, alleviating stress for caretakers, who are often unpaid family members.

Globally, our therapies can also help address public health issues. We're developing a weekly dosage of ivermectin, a drug that targets malaria-transmitting mosquitoes, that could boost the efficacy of population-level drug administration and help eliminate malaria.

What do you see as the biggest challenge facing the life sciences industry today?
One of the biggest challenges is the need to get therapies to patients faster. There are so many potentially life-changing drugs in development today, but it can take years to get them to the people who need them most. As a biomedical engineer, I believe traditional bioengineering techniques hold at least part of the answer.

For example, at Lyndra we're developing formulations that can deliver drugs for up to a month following a single dosing event. Existing stomach models are designed to test immediate release dosage forms and testing in preclinical models is a slow, complicated process. We needed a faster alternative, so we designed a first-of-its-kind simulated gastric model that can replicate complex gastrointestinal motility patterns. The model enabled us to accelerate mechanical testing and improve product development cycle times. Currently, we are able to collect 30 days' worth of data in just one day of testing.

What's next for your organization? What are you focused on in the coming year?
Most recently, we announced that we're developing an ultra long-acting oral treatment for opioid use disorder. Medication-assisted treatments have been proven to decrease opioid use, but most people who could benefit don't receive MAT treatment. There's a critical need to make it easier for patients to access treatments and we're looking forward to continuing our work with the NIH to

get these treatments to patients.

In the second half of 2019, we're expanding our Phase I pipeline and moving toward advancing multiple drug candidates, including our own therapies and drugs developed with partners, through clinical trials starting with an Investigational New Drug Application for schizophrenia. We're also advancing toward a planned Phase II clinical trial starting in 2020 and scaling up our manufacturing capabilities as we continue to focus on drug development. ■

Albireo Q&A

In August, we spoke with Ron Cooper,
President and CEO of Albireo Pharma. Ron is a seasoned biopharma leader with a track record of growing businesses, brands and organizations in the US and Europe. Before joining Albireo in 2015, Ron worked in five different countries and held positions of increasing responsibility in sales, marketing and general management with Bristol-Myers Squibb, culminating in his role as President of Europe.

Tell us about your organization, its mission, and current initiatives.

Albireo is focused on addressing unmet needs in the treatment of liver diseases. We do this by leveraging our bile acid modulation technology to develop ileal bile acid transporter (IBAT) inhibitors. The lead product candidate, odevixibat, is an IBAT inhibitor currently being evaluated in a Phase 3 program in its lead indication, progressive familial intrahepatic cholestasis (PFIC). Odevixibat could be the first medicine approved to treat this rare and life-threatening pediatric liver disease. We're also working to realize odevixibat's potential in other rare pediatric cholestatic liver diseases. We plan to initiate a trial in biliary atresia and explore other indications in 2020. At the same time, we're expanding the potential of our IBAT platform to address a large unmet need in non-alcoholic steatohepatitis (NASH).

How do your organization's activities help patients now and into the future?

Imagine your child scratching herself so incessantly throughout the night that she wakes with blood-stained sheets. She has a life-threatening liver disease, and it is getting worse. This is what the parents of children with PFIC experience. For them, Albireo represents hope. We are committed to advancing odevixibat, which has the potential to become the first approved drug therapy for this devastating disease for which there are no pharmacologic therapies. In addition to moving forward with the PEDFIC 1 clinical trial, which has over 40 sites recruiting worldwide, we have collaborated with patient organizations to support their work in community building and raising awareness through education. To this end, we launched PFIC Voices, a multimedia effort to increase understanding of PFIC together with the PFIC Resource & Advocacy Network (pfic.org).

What do you see as the biggest challenge facing the life sciences industry today?

For rare disease drug companies, a challenge—and an opportunity—exists in formalizing the patient voice in drug development. While many are already working closely with patient groups to better understand rare disease patient populations and address their unique needs, integrating these insights and context about the patient journey should be a key part of the process for identifying and evaluating a therapy's ability to offer meaningful benefit to patients.

Another challenge is bridging the gap between innovation and the patient. While the pace of breakthroughs in our industry has accelerated, the unmet medical needs remain critical in rare disease and in other areas. Society demands more and better healthcare but must reconcile the cost with willingness to devote the resources needed to develop new treatment options and cures.

What's next for your organization? What are you focused on in the coming year?

This is an exciting time to be at Albireo. From a clinical perspective, we are moving towards completion of PEDFIC 1, the Phase 3 trial of odevixibat. We're actively discussing studying odevixibat in other rare pediatric liver diseases and expect to begin a trial in one of the largest rare pediatric cholestatic liver diseases, biliary atresia.

We also have multiple programs underway to help realize the potential of our bile acid modulation science in NASH. Simultaneously, we're preparing for growth and the potential commercial launch of odevixibat.

We're looking to grow our talented team in Boston and beyond. We are even relocating to a larger office space to accommodate this growth. ■

cyclerion Q&A

In September, we spoke with Andy Busch,
PhD, Chief Innovation Officer at Cyclerion Therapeutics. Andy has extensive R&D and portfolio leadership experience across a broad range of therapeutic categories, including significant expertise in rare and orphan diseases and in the discovery and development of sGC stimulators. Before joining Cyclerion, Andy was chief scientific officer at Shire Plc; head of drug discovery at Bayer; and global head of cardiovascular research at Hoechst and Sanofi-Aventis, where he started his work with sGC modulators and brought the first sGC stimulator to the clinic.

Tell us about your organization, its mission, and current initiatives.

Cyclerion is a biotech company focused on discovering, developing, and delivering medicines that treat serious and orphan diseases. Our efforts are centered on a powerful target, called soluble guanylate cyclase (sGC). sGC is located throughout the body and is a critical regulator of a number of biological functions that are essential for human health, such as blood flow, metabolism, inflammation, and fibrosis. We are working to develop therapies called sGC stimulators that enhance these positive effects in specific tissues or organs—for example, the vasculature, the liver, the lungs, and the brain.

We are currently developing potential treatments for diabetic kidney disease (Phase 2), heart failure with preserved ejection fraction (Phase 2), sickle cell disease (Phase 2), neurodegenerative diseases (Phase 1), liver diseases (discovery), and lung diseases (discovery).

How do your organization's activities help patients now and into the future?

Every day, we're working toward getting clear answers about the efficacy and safety of the potentially life-changing medications we're developing. Our goal is to get one or more of these products approved as quickly as possible so they can make a difference for patients living with debilitating, often life-threatening, under-served conditions.

We try to help patients in other ways as well. We make sure that the patient voice is incorporated into everything we do, including the strategic decisions we make as a company and how we design and run our trials. For example, in our clinical studies we frequently use patient-centric endpoints that evaluate patients' reports of their symptoms and functional or quality of life measures, studying improvements in the symptoms that patients indicate are the most important to them. We also have a patient advisory committee that has significantly informed our understanding of sickle cell disease and the needs of the patient community.

What do you see as the biggest challenge facing the life sciences industry today?

A significant challenge for biopharma is addressing the very different, but equally important, needs of large, heterogenous patient populations and rare disease populations. Forty years ago, pharma was focused almost exclusively on developing blockbuster medications to

serve large patient populations. Then, rare diseases came into the spotlight and many companies began developing specialized medications for smaller patient populations, including well-defined sub-populations of large diseases. The pendulum has swung to the point that much of the innovation is now happening in smaller patient populations. With 7,000 rare diseases, many of these conditions are still underserved—but there remain significant unmet medical needs in larger disease areas as well. It's difficult for many companies to serve both small and large populations well. At Cyclerion, our goal is to "follow the science" and pursue the indications that we believe our molecules can best impact. We then employ a "best owner" approach. For orphan and rare indications, we have the expertise and resources to commercialize the medicine ourselves. For larger disease spaces, we intend to partner with leaders in the therapeutic area to get the medicine to patients as efficiently as possible.

What's next for your organization? What are you focused on in the coming year?

The next 12 months will be very exciting for Cyclerion. We have four key clinical trial read-outs. Our Phase 2 diabetic kidney disease and HFpEF trials, and the Phase 1 study of our molecule for neurodegenerative diseases, will all read out by the end of this year. Our Phase 2 sickle-cell disease results will be coming in mid-2020. All of these projects are part of the "dowry" we received when we spun out of Ironwood Pharmaceuticals in April 2019.

At the same time as we're focused on executing well on our near-term clinical studies, we're just getting started with our new biotech start-up. We have a fabulous team and an opportunity to build an iconic company by establishing a strategy and culture that supports ongoing innovation. ■

Bristol-Myers Squibb Q&A

In October, we spoke with Saurabh Saha,

MD, PhD, SVP, Global Head of Translational Medicine and Cambridge Site Head at Bristol-Myers Squibb. In his role, Saurabh leads the hundreds of world-class researchers that make up Bristol-Myers Squibb's translational medicine team. This innovative group aims to integrate scientific understanding into portfolio decision-making in order to enable and expedite the discovery and development of new medicines for patients. Prior to joining Bristol-Myers Squibb, Saurabh served as a venture partner at Atlas Venture, holding leadership positions across many of its portfolio companies, including CEO of Delinia.

Tell us about your organization, its mission and current initiatives.

Bristol-Myers Squibb is a global biopharmaceutical company whose mission is to discover, develop and deliver innovative medicines that help patients prevail over serious diseases. Our newest R&D site, sitting in the heart of Cambridge's vibrant innovation ecosystem, is home to a fully integrated drug discovery and translational medicine organization. With state-of-the-art laboratories, a team of leading researchers and close proximity to academic and industry partners, our Cambridge facility is well-positioned to deliver on our vision of transforming outcomes for patients. In addition to our Cambridge R&D site, Massachusetts is also home to our Devens facility, which supports process development as well as clinical and commercial manufacturing for biologics medicines.

How do your organization's activities help patients now and into the future?

Our teams are spearheading the most nascent stages of drug discovery, through molecular discovery technologies and discovery platform chemistry. Our translational medicine team is improving confidence in clinical outcomes, providing new scientific insights and accelerating our hypothesis-to-data time to make informed decisions with hopes of increasing the speed and success of drugs in the clinic. A site-wide commitment to collaboration and information sharing means no insight is being evaluated in a silo—our teams work diligently to put the larger puzzle pieces together, in hopes of discovering new therapies that can help patients in need.

What do you see as the biggest challenge facing the life sciences industry today?

One of the largest hurdles facing the industry is sustaining sufficient innovation to rapidly address patient needs. At Bristol-Myers Squibb, we're utilizing emerging technologies (e.g., artificial intelligence and deep learning, genomics, imaging, digital pathology, bioinformatics and novel diagnostics, etc.) to address this issue by enabling scientists to gather and analyze the most accurate, comprehensive data in the shortest amount of time. This helps us validate hypotheses, reimagine clinical trial designs, select novel endpoints and gather essential insights to predict outcomes more accurately and quickly. Ultimately, we believe the combination of these efforts and what we learn from them will inform strategic decisions and lead us to our goal of uncovering life-changing treatments for some of the most challenging diseases.

What's next for your organization? What are you focused on in the coming year?

Since leading the development of the first immuno-oncology treatments, we continue to ask, "Why do some cancers remain unresponsive to immuno-oncology therapy?" One of our main focuses in our Cambridge site, and at other R&D sites across the country, is answering that question. Investments in next-generation science as I mentioned earlier, including experimental research, clinical mechanisms, bioinformatics and pathology, will enhance our understanding of resistance mechanisms and help uncover why some patients never respond to I-O therapy, and others stop responding after a period of time. Paired with some of the nation's top cancer research institutions in our backyard, our Cambridge presence allows for rapid knowledge sharing with other leaders who are also working to tackle this challenge. ■

BeiGene Q&A

In November, we spoke with John V. Oyler,

Chairman, Co-Founder, and CEO of BeiGene. Over the years, Mr. Oyler has led a variety of healthcare companies including BioDuro, LLC, a drug discovery outsourcing company, Galenea Corp., a biopharmaceutical company dedicated to the discovery of therapies for central nervous system diseases and Genta Inc., an oncologyfocused biopharmaceutical company. Mr. Oyler joined the Board of Directors of the Biotechnology Innovation Organization (BIO) 2019 and is a member of its Health Section Governing Board.

Tell us about your organization, its mission, and current initiatives.

BeiGene is a fully-integrated, commercial, global biopharmaceutical company with broad capabilities spanning research, clinical development, manufacturing and commercialization. Our mission is to build the first next-generation biopharmaceutical company—one that expands the highest quality therapies to billions more people—through courage, persistent innovation, and challenging the status quo.

Over the last nine years, the company has developed a broad portfolio of six internally developed, clinical-stage, oncology drug candidates, including three in late-stage clinical development, zanubrutinib (BTK inhibitor), tislelizumab (anti-PD-1 antibody), and pamiparib (PARP inhibitor).

We also cultivated partnerships around the world, in-licensed seven drugs and drug candidates, including three marketed drugs in China (ABRAXANE®, REVLIMID® and VIDAZA®) under an exclusive license from Celgene Corporation, and four clinical-stage drug candidates with development and commercialization rights in China and other select countries in the Asia-Pacific region.

Our tagline—*Cancer has no borders. Neither do we*—speaks to BeiGene's unique model and opportunity. We operate with a collaborative spirit born out of our joint Chinese and American heritage and we are established as a leader in running China-inclusive clinical trials that can accelerate global drug development. We're currently running 60+ clinical trials, with 26 trials in Phase 3 or potentially registration enabling, across our

broad pipeline.

Founded in Beijing in 2010, BeiGene listed on NASDAQ in February 2016 and on the Hong Kong Stock Exchange in August 2018. Currently, the company has a team of approximately 3,000 employees across the globe, including approximately 600 employees in our four U.S. offices.

How do your organization's activities help patients now and into the future?

With approximately 4.3 million new cancer patients in 2018, China has as many cancer patients as the US and EU combined and is the second largest pharmaceutical market in the world by revenue. It's a dynamic market with expanding reimbursement, increasing patient affordability, and evolving regulatory policy that complies with international standards.

We're working to create impactful medicines that will be affordable and accessible to far more cancer patients around the world. One of the ways we do this is by applying cutting-edge science for the development of life changing therapies. We're proud of our efforts and the work of our innovation team which consists of 300 dedicated scientists who are challenged with the mission of developing tomorrow's medicines.

We are focused on cancer and advancing a pipeline of novel oral small molecules and monoclonal antibodies. We hope that what we do at BeiGene will contribute to improving the lives of patients who are diagnosed with cancer.

What do you see as the biggest challenge facing the life science industry today?

It is a very exciting time to be working in the life science industry, but one of the biggest challenges we see today is access. That is why our work at BeiGene is rooted in a simple yet comprehensive philosophy—BeiGene medicines must be accessible to patients who need them. Cancer is a disease that afflicts people irrespective of age, sex, socioeconomic background, ethnicity or where they live. It is our responsibility to patients and to society to bring innovative, quality treatments to markets at a price that is affordable.

What's next for your organization? What are you focused on in the coming year?

One of our lead investigational compounds, zanubrutinib, a potent and highly selective BTK inhibitor designed to maximize BTK occupancy and minimize off-target effects, has shown potential in a number of B-cell malignancies. Our New Drug Application (NDA) was accepted and designated Priority Review by the U.S. FDA for the treatment of patients with relapsed/refractory mantle cell lymphoma, an aggressive form of lymphoma. We are focused on successfully bringing this treatment option to patients who may benefit from it. We are excited to work with the FDA as it reviews our submission and are building our commercial team to execute a world-class launch of this important medicine. ■

Alnylam® PHARMACEUTICALS Q&A

In December, we spoke with Barry Greene,

President of Alnylam Pharmaceuticals. He joined Alnylam in 2003 and has more than 25 years of experience in healthcare, pharmaceutical, and biotechnology industries. In his current role, he provides key oversight and leadership in the commercialization of RNAi therapeutics around the world.

Tell us about your organization, its mission, and current initiatives.

At Alnylam, we have led the translation of RNA interference (RNAi) into a new class of innovative medicines with the potential to transform the lives of people afflicted with rare genetic, cardio-metabolic, hepatic infectious, and central nervous system/ocular diseases. Based on Nobel Prize-winning science, RNAi therapeutics represent a powerful, clinically validated approach for the treatment of a wide range of devastating diseases with high unmet medical need.

In 2018, we launched ONPATTRO® (patisiran), the first-ever RNAi therapeutic to be approved for the treatment of the polyneuropathy of hereditary ATTR (hATTR) amyloidosis in the U.S., European Union, Switzerland, Canada, and Japan. Today, our second investigational RNAi therapeutic givosiran is under review by the US Food & Drug Administration and the European Medicines Agency for the treatment of acute hepatic porphyria (AHP). With several other investigational therapies currently in late-stage development for the treatment of the cardiomyopathy of ATTR amyloidosis, primary hyperoxaluria type 1 (PH1), hemophilia and hypercholesterolemia, we are rapidly delivering on the promise of this new class of medicines.

How do your organization's activities help patients now and into the future?

Ever since we saw the therapeutic potential of RNAi in early experiments in roundworms, we have been singularly focused on transforming this groundbreaking science into medicine for patients as quickly as possible—holding deep belief in our science even when others lost hope. This same tenacity drives everything we do as a company and our work in service of patients.

Developed even before we launched our first medicine, our Patient Access Philosophy is the compass that guides our mission to get medicines to people who need them most. For example, we partner with patient advocacy groups, healthcare providers and payers to support disease awareness, diagnosis, and access efforts and establish responsible pricing practices. Additionally, through our support program, Alnylam Assist®, we offer services that guide patients through treatment with our medicine, including financial assistance options and a dedicated team of counselors that help patients make educated decisions about their treatment and care. We make every effort to ensure that our treatments fit the lives of patients, and not the other way around.

What do you see as the biggest challenge facing the life sciences industry today?

Our industry faces many challenges, but I think the most pressing issue is the need for greater diversity and inclusion within our industry leadership. Creating the next generation of medicines means we must bring in creative minds regardless of race, gender or sexual orientation, and it's incumbent upon us leaders to set an example from the very top.

At Alnylam, we've been an ardent proponent of promoting diversity in the life sciences and I'm fortunate to serve as co-executive sponsor of our diversity and inclusive initiative. We've also implemented unconscious bias training to all employees, starting with senior executives across multiple functions. These efforts and others by organizations like MassBio—which is actively promoting diversityby prohibiting all-male panels at its programs and events—can make a real difference in ensuring we have the best and brightest minds the field has to offer to help us continue to innovate.

What's next for your organization? What are you focused on in the coming year?

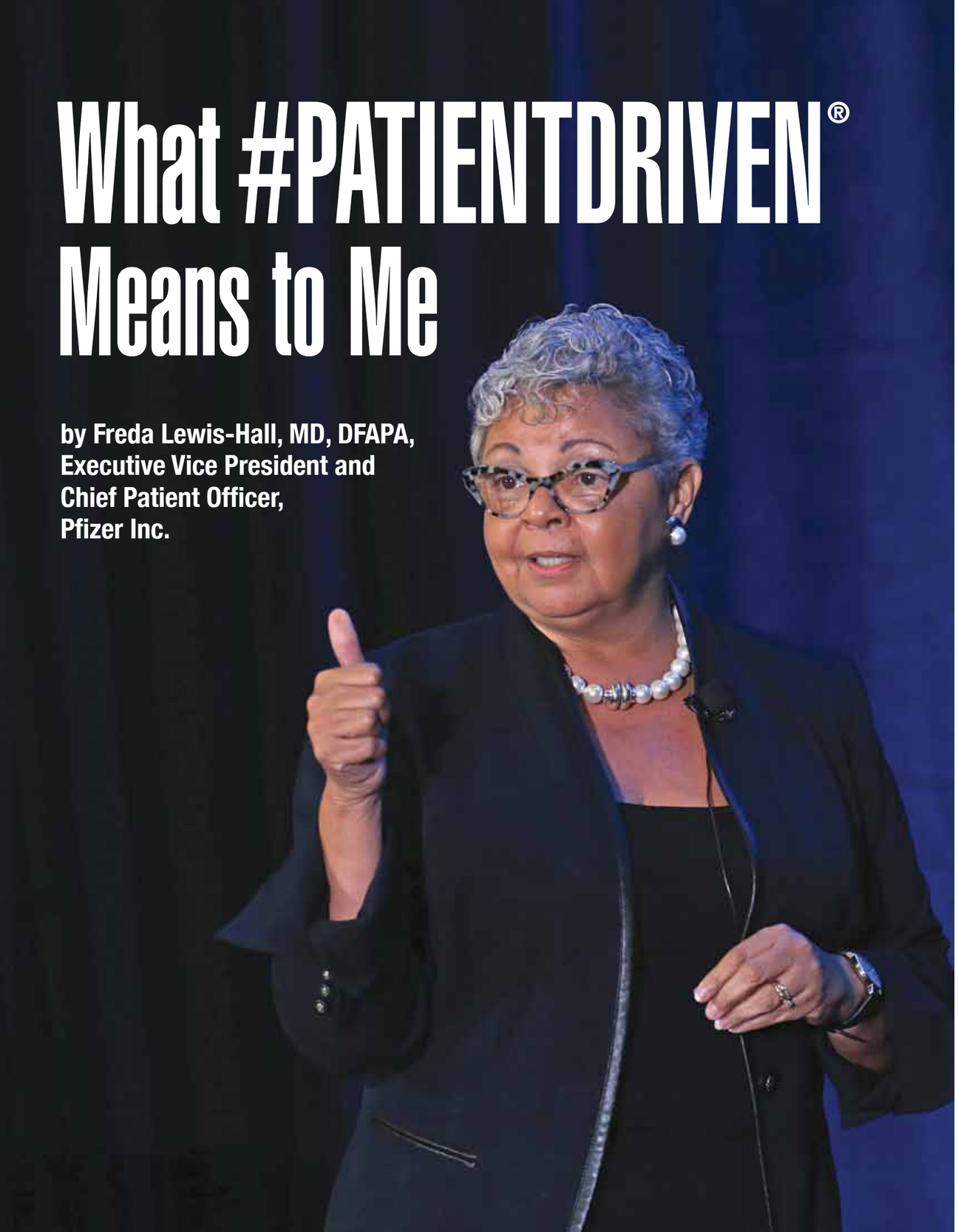
Today, our focus is on achieving our 2020 goals of building a multi-product, global commercial company with a deep clinical pipeline for continued growth and a robust product engine for sustainable innovation.

We're focused on delivering ONPATTRO® (patisiran) for hATTR amyloidosis to patients around the world, rapidly preparing to launch our investigational RNAi therapeutic givosiran for AHP, and advancing late-stage trials for lumasiran, an investigational RNAi therapeutic for the treatment of primary hyperoxaluria type 1 (PH1).

All the while, we're pushing the boundaries of RNAi by discovering new medicines for hard-to-treat diseases of the central nervous system, eye and liver, in conjunction with our partner Regeneron. We're excited about the future of RNAi, and the opportunity to harness the power of this revolutionary science for other, more common diseases as well. ■

What #PATIENTDRIVEN® Means to Me

by Freda Lewis-Hall, MD, DFAPA, Executive Vice President and Chief Patient Officer, Pfizer Inc.



Recently, I had the opportunity to visit Pfizer colleagues in three European cities—Berlin, Madrid, and Rome. I talked with them and with select groups of patient advocates about what it means to be focused on the patient, and to affirm how and why patients must be the North Star of healthcare. I emerged from these talks with fresh energy but also with eyes wide open. We are at an inflection point in the patient-centricity movement. To put it simply, it's up or out. Deliver on the potential of this movement, or fade away.

Already, some patient advocates are avoiding the term "patient-centric" because they feel it is has fallen into the chasm of "corporatespeak." I understand and respect their concerns. I've witnessed plenty of worthy concepts in medicine and business that became the equivalent of the "flavor of the month"—that is, lots of energy early on but then the hard work sets in. Then energy fades and commitment with it.

For those of us who are dedicated to the ideals of patient-centered healthcare, the hard work has set in. In healthcare systems around the world, patients are feeling that theirs is just one voice among many, and not a powerful one at all. Patients see that their access to health, and especially, to the fruits of biomedical research, is increasingly circumscribed by forces beyond their control or determined by formulas slanted against them. There is palpable concern among those who are waiting for new treatments or cures, either for themselves or for their children or other family members. People see news of potential breakthroughs regularly in the media, but approved therapies and access to them seems so far away.

In this difficult climate, though, I am heartened by the commitment of so many who are firmly on the side of patients. One of my priorities as Chief Patient Officer is to enlist our colleagues as actors and ambassadors in the drive towards a more patient-centered approach to healthcare. In Europe I was energized by stories of colleagues who went beyond the call to both listen to patients and take action with them, in one case, actually walking alongside them as patients pressed for greater recognition of their need and concerns. With both colleagues and patients, we discussed the goal of "end to end" advocacy, where a company like Pfizer goes well beyond patient-focused drug development, vital as that is. "End to end" advocacy calls for meaningful patient engagement in every major decision that begins with a scientific insight and ends with a therapy that people can both obtain and afford.

So that's what #PATIENTDRIVEN® means to me. "End to end" advocacy, arm in arm with patients, so that they benefit now from the fruits of biomedical research and can offer hope to the next generation that they, too, will benefit. ■

During her 35-year career in medicine, Freda Lewis-Hall has been on the frontlines of health care from the standpoints of a clinician, a researcher, and a leader in the biopharmaceuticals and life sciences industries. The common thread throughout has been her passion to advocate for health equity and improved outcomes for all patients. Trained as a psychiatrist, Dr. Lewis-Hall began her medical career in patient care and became well known for her work on the effects of mental illness on families and communities and on issues of health care disparities. She has held positions of leadership at the Howard University Hospital and College of Medicine, Vertex, Bristol-Myers Squibb, Eli Lilly and Pfizer. Some of her many achievements include founding the Lilly Center for Women's Health in the 1990s; serving on the board of the U.S. Patient-Centered Outcomes Research Institute (PCORI) since its inception in 2010; creating Pfizer's public health information program Get Healthy Stay Healthy in 2012; launching the industry's first public compassionate access request portal, PfizerCARES, in 2015; and spearheading the creation of SpringWorks Therapeutics, a new company working to develop promising new treatments in underserved areas of urgent medical need, in 2017. From 2009 to 2018, Lewis-Hall served as Pfizer's Chief Medical Officer, responsible for the safe, effective and appropriate use of Pfizer's medicines and vaccines, and in this role she reshaped Pfizer's medical policies and practices to intensify the company's focus on patient engagement and inclusion. In her current role as Chief Patient Officer of Pfizer, Lewis-Hall is working to extend the reach of Pfizer's patient-facing health information and education and amplify the voice of the patient inside and outside Pfizer.

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