MassBio

Insider

Winter 2020

An Inside Look at the #1 Life Sciences Cluster

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Susan Hockfield, PhD
President Emerita, MIT
Speaks at the State of Possible Conference *Gone Virtual*

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An Inside Look at the #1 Life Sciences Cluster

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I Have Never Seen Our Industry Work As Tirelessly

As we near the end of 2020, we can all look back at what has likely been the most disruptive year many of us have ever seen, and at the same time, breathe a sigh of relief. The biopharma industry has achieved an incredible feat—developing multiple COVID-19 vaccines that have upwards of 95% efficacy and may be approved before the year's end. In my 14 years at MassBio and many decades supporting the life sciences industry in Massachusetts, I have never before seen the industry work as tirelessly, pour more funds into R&D, and beat all odds for a development timeline that typically takes 5-10 years. So, thank you, to every single MassBio member who has contributed to the fight against the novel coronavirus, and to every single person who has lent their time and expertise, and sacrificed their health and safety for others.

Not only have our members played an indispensable role in getting us through this pandemic, their support of MassBio has never been stronger. Our members adapted alongside us as we moved our programming and services to a virtual environment and remained dedicated to our mission of improving patient lives. Looking ahead to 2021, we will continue to provide virtual services to our members, especially around events. Our first-ever Partnering Week, scheduled for January, reinforces MassBio's commitment to supporting our world-class startup community, and helping to ensure the best ideas leave the lab and reach patients' bedside.

MassBio looks forward to working with government, academia, and the entire life sciences cluster in Massachusetts to further the goals laid out in our 2025 State of Possible report, a roadmap on how to overcome challenges for the industry and capitalize on opportunities for growth. Equally important, we will continue to support our members as they seek to improve equity, diversity, and inclusion in their respective companies, and will work to promote positive change across the Commonwealth.

Thank you again for all you do for patients, the life sciences industry, and MassBio. I wish you a happy and healthy end to 2020 and an even better start to 2021.

words of welcome

Robert K. Coughlin, President & CEO, MassBio

MassBio Upcoming virtual events

MassBio Partnering Week
January 25-29, 2021

Policy Leadership Breakfast
January 27, 2021

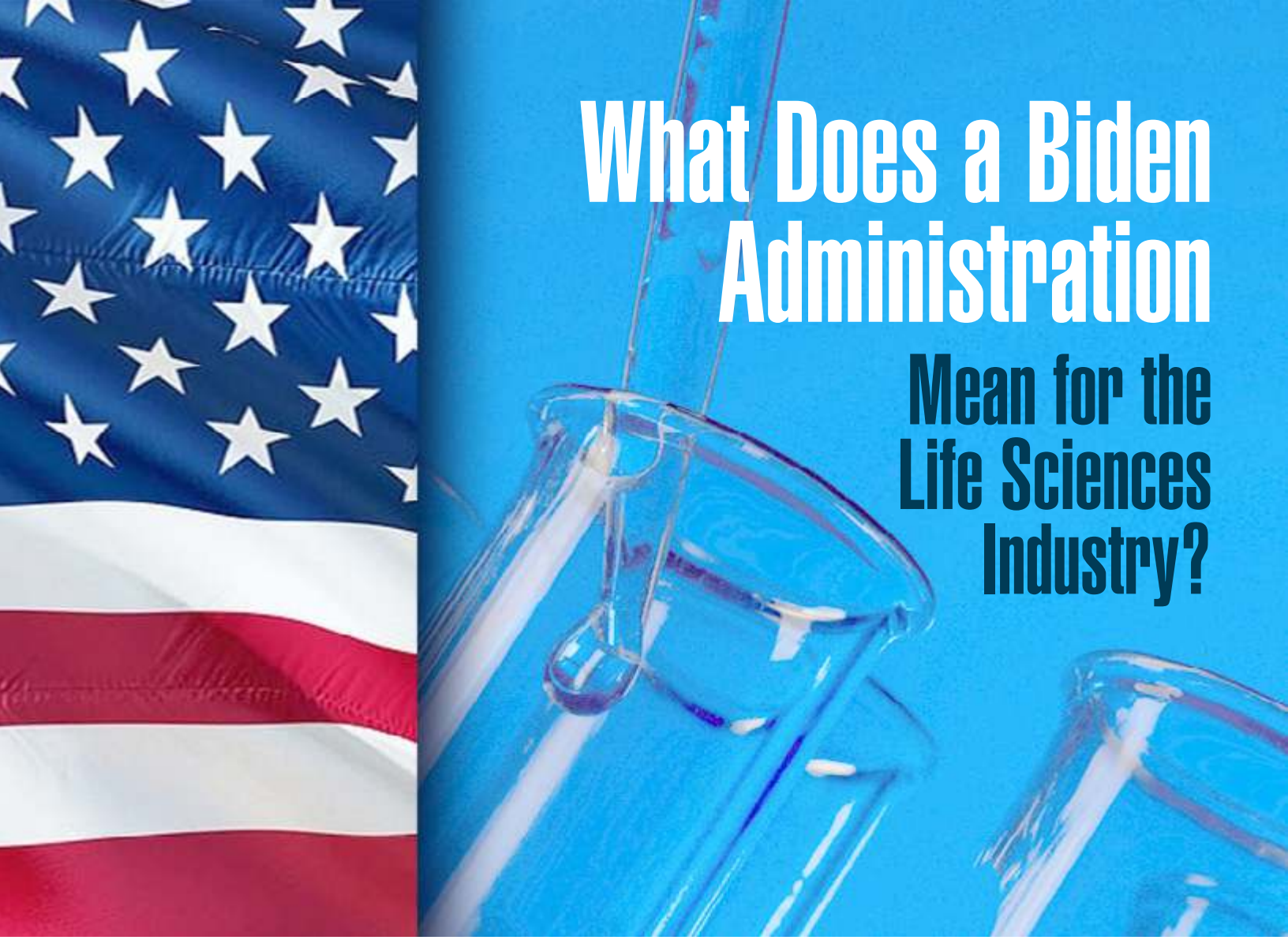
Rare Disease Day
February 26, 2021

State of Possible Conference
May 19, 2021

ED&I Conference
July 21, 2021

Patient Advocacy Summit
September 30, 2021

Digital Health Impact
November 4, 2021



What Does a Biden Administration Mean for the Life Sciences Industry?

By Susan Martin, Director of Government Affairs, MassBio

It's tough to make predictions, especially about the future. So, only weeks past a national election that saw historic voter turnout, do we have any idea how the election of Joe Biden as President will impact the life sciences industry? Based on how President Trump governed for the last four years and the platform Biden ran on, we can say for sure that things will change, and it will impact the biopharma sector in real ways. Will those changes be a net positive? We think so and here's why.

Drug Pricing Reform

Unexpectedly for a Republican, President Trump proposed and supported aggressive drug pricing reforms during his term, including a Most Favored Nation pricing proposal for drugs in Medicare Parts B and D, and allowing states to import prescription drugs from abroad. Trump also, on numerous occasions, publicly expressed support for various Republican-led legislative proposals on drug pricing reforms, even while he strongly opposed House Democrats' passed legislation, which included many of the same concepts. These were all policies strongly opposed by our industry.

On paper, President-elect Biden's policy positions on drug pricing are even worse for the biopharma industry. While he also supports some kind of international pricing index for Medicare drugs and drug importation, his proposals go a step further. Biden also supports policies that would allow the government to negotiate directly

with manufacturers in Medicare Part D, along with those that limit drug price increases to inflation across government payers. These are equally problematic for the continued success and growth of the biopharma industry.

VERDICT: *President-elect Biden will be worse on drug pricing reform for the life sciences industry than President Trump, but not by much.*

Trust in Science and Government Agencies

Throughout his Presidency, and especially during the COVID-19 pandemic, President Trump often questioned the integrity of government agencies and officials in charge of our country's public health. Whether the FDA, CDC, or Dr. Anthony Fauci directly, Trump impugned their trustworthiness and dedication to do what's right for the American people.

On the other hand, President-elect Biden has spent much of his campaign expressing strong support for our government agencies and its leaders, often arguing that we as the American people need to trust institutions like the FDA to do what's right, even if it takes longer than we'd want. Biden also led the Cancer Moonshot effort as Vice-President and believes in the power of government for good.

VERDICT: *President-elect Biden will lead a government that believes in science and trusts in its leaders and agencies to do what's right without politicization. He also has experience with and belief in supporting new and big government initiatives, such as the Cancer Moonshot, which could extend to other ideas that help advance future drug development.*

Immigration

President Trump spent his time in office supporting policies and signing executive orders that severely limit both legal and illegal immigration. From the "Muslim ban" to further limiting work (e.g. H-1B) and family visas, Trump has made it much harder, and much less desirable, for immigrants to come to America—even those with advanced degrees or specialized skills.

President-elect Biden has pledged to roll back many of President Trump's immigration policies immediately and will seek to reform the temporary visa system, along with expanding the number of high-skilled visas and eliminating the limits on employment-based visas by country.

VERDICT: *President-elect Biden's approach of reversing President Trump's policies, while also seeking to increase the number of high-skilled visas, will be a major benefit to the full life sciences industry as it will allow organizations in the U.S. to better recruit the diverse talent necessary for us to continue to be the world-leader in the development of new cures and therapies.*

In summary, when considering just these three areas of Presidential policy that impact the life sciences industry, it's a bit of a mixed bag when assessing the impact of a Biden Administration on the life sciences industry. In many ways, our industry is doing better than ever this year, with record high numbers of IPOs and impressive funding amounts. Yet, in the long-term, our industry relies on a strong FDA, a widespread belief in science, and stability from government in order to be successful. When looking at Biden's policy positions and his past actions as Vice-President and Senator, we believe his presidency will be a net positive for our industry. ■

A Commitment to Action in a Time of Change

By Edie Stringfellow, Senior Director of Equity, Diversity, and Inclusion, MassBio

Early summer 2020 was a time of reckoning in America. A national conversation about race and racism was taking place with more widespread, serious attention paid across the country to these crucial issues than perhaps ever before. Businesses and organizations stood up and spoke out against racism and pledged to be better and to do things differently. Against the backdrop of our country's history and our collective recalcitrance to address this issue head-on, as a Black American, I felt hopeful but cautious. I've seen many times over how it's easy to be woke in the moment. It's harder to be committed to change and to be held accountable over time.

At MassBio, our equity, diversity, and inclusion initiative (ED&I) seeks to provide resources and direct assistance to our member companies of all sizes so they can launch or strengthen their ED&I strategies. These companies are working to improve diversity on their teams at all levels, to inspire an inclusive culture, and to provide an equitable environment where all are given the tools and resources to succeed. As our initiative has grown over the last four years, we regularly ask ourselves: are our efforts having an impact and are our members' initiatives making real, lasting change?

Measurement and accountability are critical pieces of any ED&I initiative. The latest step in our effort to provide a roadmap for success while ensuring accountability was the launch of our "Open Letter 2.0 – The CEO Pledge for a More Equitable and Inclusive Life Sciences Industry." This pledge, launched in July, called on CEOs across the life sciences community to join us and pledge their name toward a range of best practices designed to improve equity, diversity, inclusion, and culture inside their companies with a special focus on Black, Brown, and Indigenous People of Color. The pledge is designed to be flexible to fit all companies and circumstances with the understanding that there is no one-size-fits-all fix to ED&I.

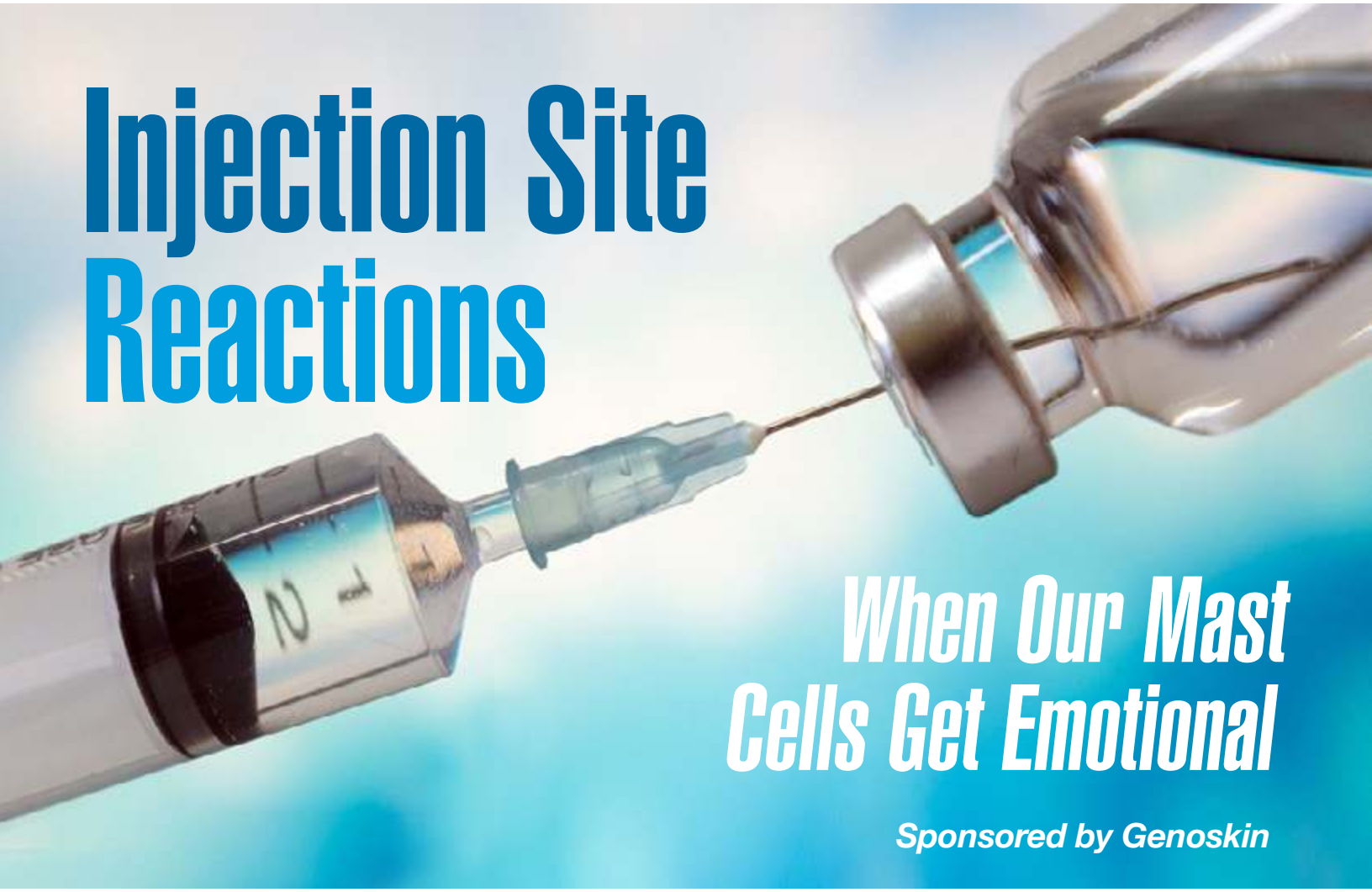
Signing the CEO Pledge is a public commitment to do better. In order to make sure that all signers, especially smaller companies, are successful, we are providing our full suite of resources to them upon signing, including 1:1 meetings to help develop and scale ED&I programs no matter the size of their company. We'll provide the resources, networks, and introductions to make sure they have the tools and contacts they need. And we will regularly check-in with them to assess progress and suggest further ideas to improve. We are also asking them to share their diversity data with us so we can measure change over time across the industry.

Since the launch of the pledge in July more than 200 CEOs have signed on. Of special note is the commitment by small and emerging life sciences companies to ED&I and to this pledge. Over 50 of these CEOs lead companies with less than 20 employees and over 40 have less than 50 employees. It is their recognition and leadership on ED&I from an early stage that has me particularly excited and hopeful about the future of our industry in Massachusetts.

Thank you to MassBio's Board members and ED&I leaders who were initial signers of this pledge—it's your early support that will give others the courage to sign on. I urge you to add your name as a CEO today or to encourage your CEO to sign. Together we will make a difference, be more successful, and ensure our companies look like the patient populations we all strive everyday to serve. ■

To learn more, visit MassBio.org/diversity

Injection Site Reactions



When Our Mast Cells Get Emotional

Sponsored by Genoskin

By Nicolas Gaudenzio, Ph.D., Chief Scientific Officer, Genoskin

Upon subcutaneous or intramuscular injection of therapeutic drugs a fast and local inflammatory reaction characterized by swelling, erythema, pruritus, even pain can develop surrounding the injection site and is commonly named "injection site reaction" (i.e., ISR, also known as pseudo-allergic reactions). Injection site reactions are the most commonly reported adverse event (10% of patients treated with biologics). Although clinical symptoms of immediate ISR can be similar to common allergy (or type 1 hypersensitivity), immediate ISR are quite distinct from IgE-mediated allergic reactions. The reaction arises for the first time, without prior sensitization or development of drug-specific antibodies (e.g., IgE). Many different drugs have been reported to trigger ISR. Drugs that usually exhibit basic properties with a tetrahydroisoquinoline (THIQ) motif were reported to stimulate the degranulation of skin mast cells and are often associated with the development of immediate ISR of moderate to severe intensity. Importantly, a greater risk to develop severe forms of ISR has been reported in patients with systemic mastocytosis (a proliferative disorder of hematopoietic mast cell progenitors leading to expansion and accumulation of excessive numbers of mast cells in multiple organs, including the skin). Indeed, anesthetic procedures in patients with systemic mastocytosis can more often lead to extreme reactions such as systemic hypotension, anaphylaxis, and coagulopathy often resulting in patient death. Reasons why certain drug formulations are capable of triggering ISR are still elusive and represent a major challenge in the field.

Mast cells are long lived hematopoietic sentinel cells located in areas of the body preferentially exposed to an external environment, such as the skin. Upon activation via a broad panel of surface receptors, mast cells release secretory granules enriched in pro-inflammatory molecules. Degranulation products act on neurotransmission to trigger itch and pain sensations, smooth muscle

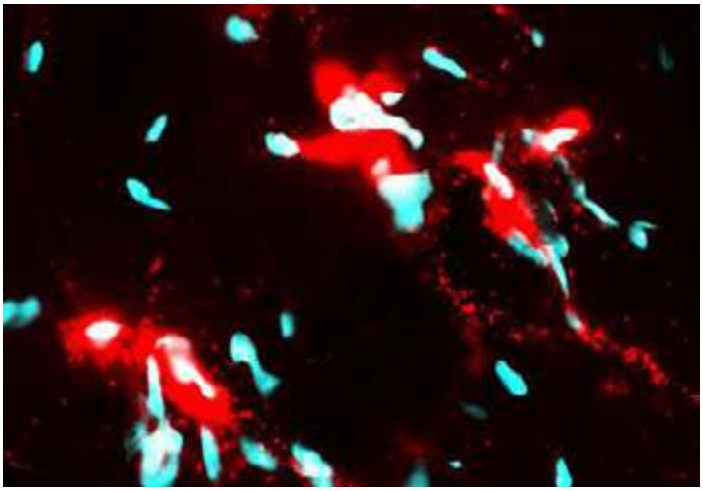
cells contractility, vascular permeability to cause edema and recruitment of inflammatory immune cells. Mast cells can be activated to degranulate in response to the engagement of numerous receptors, but among them, the mast cell-restricted Mas-related G-protein coupled receptor (MRGPRX2) has recently attracted substantial scientific and medical attention. MRGPRX2 has been reported to be expressed by human skin mast cells. Activation of human mast cells via MRGPRX2 has been proposed as a possible mechanism by which immediate ISR could develop in patients. Most of MRGPRX2 ligands share cationic chemical properties and belong to various categories of molecules, including synthetic drugs. Many of the FDA-approved drugs known to trigger immediate ISR in patients, share a basic THIQ motif and are reported to activate MRGPRX2, such as Angiopeptin, Hexarelin, Cetrorelix, Icatibant, Leuprolide, Octreotide, Sermorelin, Atracurium, Rocuronium, Tubocurarine, Ciprofloxacin, Levofloxacin, Moxifloxacin, Ofloxacin, Vancomicin, and Morphine. More are continuously discovered.

The key role played by MRGPRX2 in mediating most of the immediate ISR has been shown by engineering mutant mice that specifically lack MRGPRB2 (mouse MRGPRX2 ortholog). Interestingly, injection of Icatibant (a selective peptidomimetic bradykinin beta2-receptor antagonist reported to induce ISR in most patients) triggered a robust ISR in wild type mice that was significantly reduced in mice deficient in MRGPRB2. In vitro, basic molecules or drugs associated with pseudo-allergic reactions (e.g., atracurium, mivacurium, tubocurarine, rocuronium, iprofloxacin or levofloxacin) were found to activate wild-type mast cells, while mast cells that lack MRGPRX2 were unresponsive to these compounds. Other studies reported ISR with drugs used in the treatment of patients with schizophrenia, rheumatoid arthritis and chronic migraine. These results strongly suggested that MRGPRX2 expressed by skin mast cells could be efficiently activated by therapeutic molecules routinely used in clinic. Such IgE-independent MRGPRX2-dependent mast cell activation might represent a major mechanism of relatively fast occurring ISR and/or systemic pseudo-allergy development.

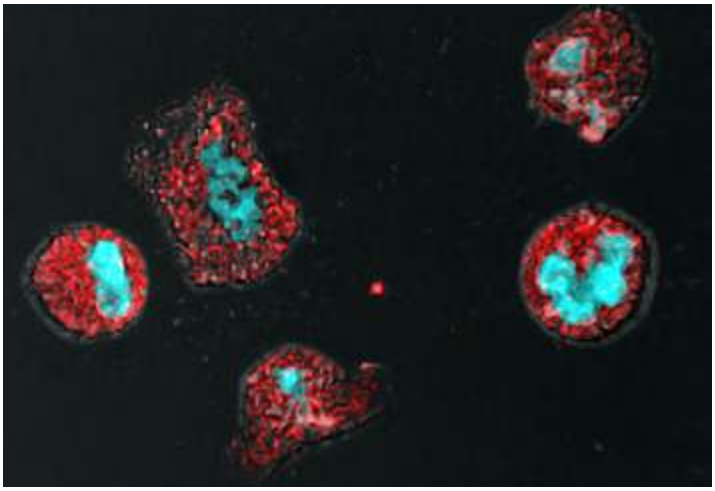
Fortunately, characterization of MRGPRX2-dependent adverse skin manifestations can now be performed on human skin explants when immunocompetency is maintained and confirmed in complementary translational screening assays developed in MRGPRX2-expressing cell lines as well as human primary mast cells. ISRs can be studied on Genoskin's HypoSkin® to avoid MRGPRX2-dependent reactions, optimize drug formulations to avoid MRGPRX2-dependent reactions and accelerate drug development. ■

Dr. Nicolas Gaudenzio is a rising Assistant Professor in the field of neuro-immunology and allergic skin inflammation. His work has contributed substantially to identify molecular and cellular targets involved in allergic skin inflammation (such as atopic dermatitis) and to develop new intravital and multiplex imaging methods to probe neuro-immune interactions in preclinical skin models.

Learn more about our ISR services at
bd@genoskin.com



Imaging of activated mast cells in HypoSkin® assay. Staining with DAPI (nuclei) and Avidin (granules).



Imaging of resting mast cells in culture. Staining with DAPI (nuclei) and Avidin (granules).

The Race for a Vaccine Began in the 19th Century

Here's How Far We've Come

By Cayley Moynihan, Senior Associate of Public Affairs, MassBio

At the onset of the COVID-19 pandemic, all eyes turned to biopharma companies to deliver a safe and effective vaccine and to do so at record-breaking speed. As of writing this, Pfizer and BioNTech reported their COVID-19 vaccine candidate is safe and 95% effective, and Massachusetts-headquartered Moderna reported its vaccine candidate to be 94.5% effective, both in early analysis. To truly understand how colossal of a feat vaccine development is, we looked at the vaccines that changed history and how they compare to the innovation of today.

Smallpox was one of the deadliest diseases known to humankind. While its origin is largely unknown, the earliest written description of a disease that resembles smallpox dates back to the 4th century. Centuries later in 1796, Edward Jenner observed that milkmaids who previously caught cowpox did not catch smallpox. Using this observation, he demonstrated that "inoculated vaccinia protected against inoculated variola virus" and introduced the smallpox vaccine—the first successful vaccine. People were vaccinated through "arm-to-arm inoculation," taking fluid from a blister of someone infected with cowpox and injecting it into another person's skin. Smallpox remained widespread until 1966, until it was officially declared eradicated on May 8, 1980—one of the greatest public health victories of our time. But the history of this disease also demonstrates just how long it can take to research,

develop, and produce an effective vaccine. Like smallpox, many other notorious viruses articulate a similar story.

At the height of the polio epidemic, parents lived in fear for their children. The first major epidemic in the U.S. occurred in 1894 and by the late 1940s, polio outbreaks in the U.S. increased in frequency and scope, affecting more than 35,000 people each year. Research for a vaccine began in the 1930s, but early attempts were rendered unsuccessful. Nearly two decades later, Jonas Salk introduced an effective vaccine, the inactivated polio vaccine (IPV), in 1953. Clinical trials for the vaccine began in 1954, enrolling over one million schoolchildren, and in April 1955 it was announced that the vaccine was safe and effective, sparking a nationwide inoculation campaign.

Salk's vaccine was unique—unlike vaccines that used a weakened version of the live virus, like the vaccines for measles or mumps, the polio vaccine used an inactivated version. Because the immune system cannot distinguish between an activated virus and an inactivated one, when someone was injected with the polio vaccine the immune system still recognized the intruder and created antibodies to protect that person from future infections. Despite Salk's resounding success, it took roughly 20 years from when research first began to develop an effective polio vaccine. Though polio has not been completely eradicated, cases have decreased by over 99% since 1988 because of widespread vaccination. Contrary to these two viruses, influenza cases spike every winter with vaccines serving as a means for yearly protection, versus a one-time solution.

The flu has been around for over 100 years. In fact, the first mention of influenza appeared in a public health report during the 1918 flu pandemic. Roughly 20 years later and with the support of the U.S. Army, Thomas Francis, Jr. and Jonas Salk—the same doctor that developed the polio vaccine—developed the first inactivated flu vaccine using fertilized chicken eggs, the method used to produce most flu vaccines today. A vaccine that offered protection against both strains of influenza was discovered and developed later in 1942. A few years later and decades after the world learned of influenza, a flu vaccine was officially licensed for use in people. However, the flu virus does not behave in the same way as smallpox or polio. It frequently acquires new mutations—even from season to season—forcing scientists to analyze the virus each year and modify the vaccine to best protect people.

Each of these three viruses highlight the arduous path from the discovery of a virus to vaccine development and approval. They also paved the way for the innovative vaccines we are seeing today that are being developed and tested at unprecedented speeds.

In early January, Chinese scientists published the SARS-CoV-2 sequence. In less than 10 weeks, a team from the National Institute of Allergy and Infectious Diseases and a Massachusetts-headquartered biopharma company had a vaccine candidate ready for a Phase 1 clinical trial. While some approaches to vaccine development still use a weakened or inactivated form of the virus, as was done with smallpox and polio, the innovative techniques today enable scientists to bypass this lengthy process and instead look to the virus's genetic sequence to accelerate vaccine development. Prior to COVID-19, the current record for vaccine development was four years—mumps — but with the degree of contemporary scientific progress, coupled with past triumphs and failures, and immense levels of funding and collaboration, a COVID-19 vaccine could potentially receive FDA approval in less than 12 months. However, the next, and perhaps greatest, hurdle will be distribution.

The next several weeks, months, and year are shrouded in extraordinary levels of uncertainty. Once a vaccine is approved and available for public distribution, the Centers for Disease Control and Prevention (CDC) has outlined preliminary plans to provide healthcare workers, those with high-risk medical conditions, people over 65, and essential workers first access to the vaccine. However, state and federal health officials are also taking into account how to legally provide early access to minority populations and those disproportionally affected by the novel coronavirus. It will take a concerted effort between federal and state public health agencies to distribute COVID-19 vaccines to as many people as possible as quickly as possible. While many questions remain around prioritization and distribution, one thing is certain: we are fortunate to be living through an era of unprecedented innovation and it is science that will lead us out of this pandemic. ■

How the Right Choice of Primary Packaging Can Save Time and Money During Drug Development

By Dr. Abizer Harianawala, Ph.D., US TEC Site Leader, Stevanato Group

The container closure system is a crucial element of parenteral drug delivery—so the selection of primary container packaging is one of the most fundamental considerations in the drug development process. Making the right choice early in the development process can save significant time and resources further down the line, as well as helping to smooth the path to eventual regulatory approval globally.

Plan for Success

Selecting a primary container for injectable drug development needs careful planning and is best done in the early stages of a project to streamline the whole development process. Simply choosing a container by default, without clear technical rationale, can lead to realization at a late stage of the drug development cycle that the container is not the right one to take to market. This can have serious implications in terms of cost and time—and may even lead to the drug having to be reformulated.

It is important to consider all aspects of the drug product before deciding on the primary container and closure system. Biopharmaceutical developers, for example, often work with complex, unstable, but very promising compounds that have the potential to transform the lives of patients with chronic diseases. Complex compounds are more prone to interactions—so a rigorous assessment of the primary packaging is an essential early step in the drug development process. The overall container closure system is a combination of multiple elements, which means a range of analytical techniques are required to characterize the primary packaging and ensure the best option is chosen for each specific drug product.

Factors to Consider

The choice of a particular primary container can be affected by a range of factors related to both the integrity of the drug and the container itself. Drug administration factors include stability and sensitivity, which will affect

the type of container that should be used. Container-specific factors include cosmetic appearance, filling equipment, chemical durability, and container closure integrity. Manufacturability must also be considered to ensure compatibility across all these parameters—and thorough testing is key to address this. Long-term life-cycle management of a drug product is another vital consideration. For example, if a drug is initially introduced in vial format, it may eventually be converted into a different container—such as a prefillable syringe—due to market preferences. Risk assessment during early phases of product development should be conducted to account for all the factors to minimize risk and reduce development time and costs.

Safety and Regulatory Considerations

Meeting global regulatory requirements is crucial for any primary packaging container—and pending regulatory changes also need to be addressed to ensure the container system meets future requirements. The shift away from hospitals and clinics to home healthcare is a further consideration for drug companies. Medications for chronic diseases such as rheumatoid arthritis, along with a wide variety of biological preparations, are being developed for administration at home—either by the patient themselves or a home healthcare worker. This has implications for the choice of primary container. In the past, systems may have evolved from a vial to an autoinjector over time. However, drugs are now being developed directly into prefillable syringes to meet the increasing demand for home care.

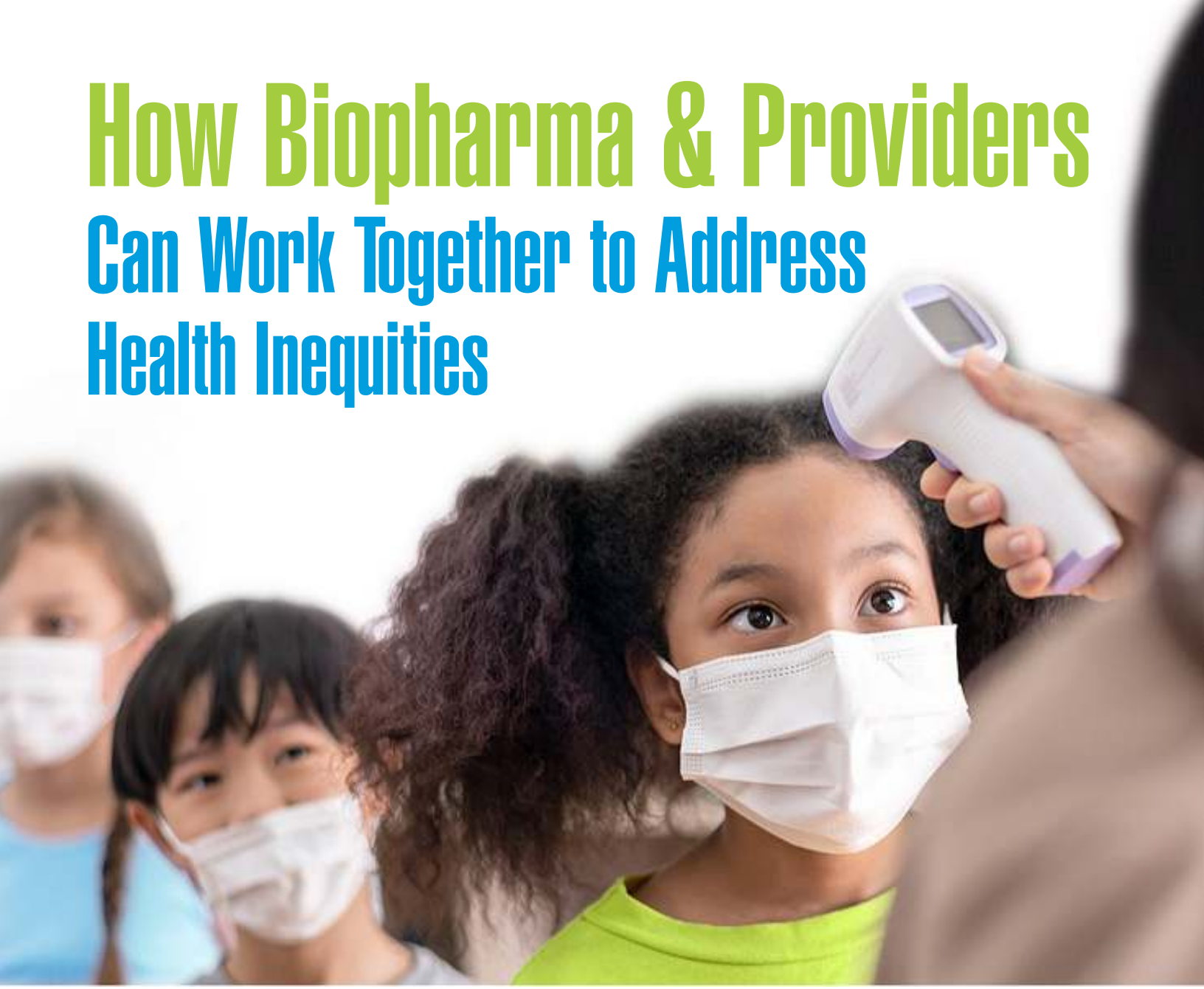
Right First Time

Drug developers risk losing millions of dollars if a product launch is delayed—even by a month—due to packaging issues. So, there's a lot at stake when choosing the right primary container to support requirements from early-stage formulations through to future integration into delivery devices. Getting that decision right first time is now more crucial than ever. ■

Abizer Harianawala is the Site Leader for Stevanato Group's US Technology Excellence Center located in Boston, MA. With a Ph.D. in Pharmaceutics from the University of Connecticut, Dr. Harianawala has more than 20 years of extensive CMC experience in developing and commercializing oral, parenteral, and combination products. His areas of expertise include formulation design, manufacturing process development and optimization, container closure characterization, and preparation of CMC documentation for global regulatory submissions.

To find out more about SG's Analytical Services, visit
stevanatogroup.com/en/offering/analytical-services/

How Biopharma & Providers Can Work Together to Address Health Inequities



By Zach Stanley, Vice President of Public Affairs, MassBio

The COVID-19 pandemic has put a spotlight on health inequities in our country, as minority populations are getting sick and dying at a disproportionate rate. Healthcare and life sciences industries, and organizations like MassBio, are increasingly making health equity a strategic priority to ensure everyone has equal access and opportunity to quality healthcare. But it's clear there is much work to be done.

That's why MassBio's new podcast, the State of Possible Podcast, put the issue front and center. For our October episode, Bob Coughlin, MassBio's President & CEO, was joined by Kate Walsh, President and CEO of Boston Medical Center Health System (BMCHS), and Chirfi Guindo, Executive Vice President and Head of Global Product Strategy and Commercialization at Biogen, to explore how the biopharma industry and providers can work together to address health inequities.

We started the conversation around the root of health inequities in our country, from a culture of mistrust originating from the Tuskegee Syphilis experiment that began in the 1930s, to the lack of access to quality care for minority populations. According to Guindo, this trust deficit

is a longstanding issue and there is no magic solution. We need diverse representation within the healthcare workforce, both at biopharma companies and providers and at the leadership levels, to really change this perception. Walsh agreed, noting that as a safety-net hospital, the COVID-19 pandemic has disproportionately impacted BMC's patients, serving as a current example of just how prevalent these health inequities are in the U.S. However, she argued that institutions like BMCHS have an incredible opportunity to improve this reality since the organization works hard every day to earn the trust of minority populations through their long-standing commitment to caring for Boston's most diverse residents.

Before COVID-19, 50% of BMCHS's discharges were patients of color, and 10% of patients in BMC beds were homeless—a percentage that rose to 80% and 18%, respectively, during the pandemic. According to Walsh, COVID-19 made them step back and ask how we can address these root causes. It became clear that we can all do better to involve patients earlier in the process, and to stop making assumptions about why people do what they do. It's so much more complicated than not wanting to take time off work to participate in a clinical trial. Patients can't worry about their health if they don't have a roof over their head, argued Walsh. How we engage patients earlier on in study design, considering their specific needs, while also recognizing that the organizations that serve minority populations are often under-resourced is no small task. So, what can we do?

Access to clinical trials is a major component of health inequity that our industries can take the lead on addressing. A clinical trial should be representative of the patient population, but we know this isn't always the case, as there are real barriers to accessing these trials and to diversifying the participants. According to Guindo, "There is a tension between the urgency with which we develop programs and the need to have diversity." While we all agree it is important, it takes more work, and the incentives are not quite aligned. Do we sacrifice the speed in which we're developing therapies to find more diverse participants? The simple answer is yes—but nothing in the drug development process is simple. Back toGuindo's earlier point, it takes having diverse frontline workers to do it right, including clinical research teams and people who are interacting with the providers and the patients.

That's where institutions like BMCHS come in again, since these organizations already have a long history of building trust with these diverse and underrepresented patient populations. Biopharma needs to take advantage of these relationships, and not just round up the usual subjects for clinical trials. "We need to find willing partners on the life sciences side who will take a risk and work with us," said Walsh. She recognized that BMC must also do better to make sure their patients are represented in clinical trials. Fortunately, we're at an inflection point where it seems there's a true commitment to do just that. To Kate's point, the industry came out of the COVID-19 surge and into Black Lives Matter. We cannot let go of this momentum and the incredible collaboration we've seen during the pandemic.

MassBio is committed to being part of the solution and to push on our members, who represent both the biopharma and provider side, to use 2020 as a catalyst to create lasting change. We must double down on our commitment to diversifying the life sciences workforce, and share the good work that Biogen, BMCHS, and others are doing to address the very disparate needs of patients—from housing to food to health. "We need to go beyond curing disease to promoting health and promoting equity—and that includes human health and also environmental health," said Guindo.

Recognizing that the speed in which we were able to get clinical trials off the ground for vaccines is the result of decades of research and experience, we must take this same long view to health inequity. "We have a long way to go but I think our commitment is the fuel to accelerate our desire to diversify clinical trials and really address health disparities in our country," said Walsh. ■

You can listen to MassBio's "State of Possible Podcast" on MassBio.org/podcast and all major podcast platforms.

Sustaining the Legacy of the Massachusetts Life Sciences Cluster

A Q&A with Chuck Wilson
Chair of the MassBio Board

Earlier this year, MassBio released its State of Possible 2025 report, a five-year strategic plan for Massachusetts and MassBio to ensure the life sciences cluster's long-term success. To learn more about how this report will serve as a roadmap for the next five years and beyond, we sat down with MassBio's new Board Chair, Chuck Wilson.

MassBio's State of Possible 2025 Report identified four key opportunities for the Massachusetts life sciences industry to achieve balanced growth in the coming years. Which opportunities do you believe you can make the greatest impact on during your time as chair?

We must focus on the opportunities that will best support our sustainability. To get there our efforts should be concentrated on the growth of commercialization and manufacturing of therapies here in Massachusetts, and the expansion of the cluster beyond Cambridge/Boston. Our strength in R&D has cultivated an ecosystem of innovation that is the envy of the world and led to improved outcomes for patients. Similarly, the life sciences industry is highly concentrated in Kendall Square. In both cases, without growth it's hard to envision a truly sustainable ecosystem.

In the past, Massachusetts leadership in new drug development has not translated at scale to commercial operations as small and emerging companies often did not bring products to market themselves. That dynamic is changing, but intentional steps must be taken to get companies to invest their commercial operations in our state. What's farther along is biomanufacturing. Massachusetts is already home to multiple manufacturing facilities and has received significant investment in new and expanded facilities. As curative and next-generation therapies come down the pipeline, creating new opportunities for biomanufacturing, Massachusetts' cohort of innovators and availability of specialized talent are ripe to position the state's manufacturing as an area of strength. Additionally, as our 2025 Report lays out, growing existing and establishing new "mini-clusters" across the state will allow our industry to thrive. It's time we transform the dynamic of our industry, so innovation isn't solely centered within a few square miles, but instead exists in even the smallest communities.

What are some of the biggest challenges facing the life sciences industry right now?

Right now, our industry's growth is being crippled by our aging transportation and housing infrastructure and our limited ability to recruit and retain top talent, especially from diverse backgrounds. Traffic may not be bad right now because of the pandemic, but hoping it stays this way is not a long-term solution. It is very difficult for people to leverage our robust network of resources when they cannot access the Cambridge/Boston core on our decaying roads and public transportation or afford to live near it. However, the shift to remote work in response to the pandemic provides an opportunity to rethink how we can address our infrastructure challenges, and part of that may be changing the way we work. If we don't fix things now, we are going to be worse off once normality is

restored, and that applies to our talent pipeline as well.

Society is commanding we take action in response to the recent protests and decades of systemic racism that has plagued our country—it is time we hold ourselves accountable. Our industry is not as diverse as it could be and that is a disservice to the patients we serve. Boston and Cambridge are an academic mecca of many people who come here for education, training, and experience, but we need to retain that talent to stay, work, and live here. We also need to significantly broaden the industry's talent pipeline outside of the usual places so we can identify more diverse talent and get them to Massachusetts. We are the world-renowned leaders in the life sciences, and we need the best talent and the greatest minds from across the globe to live, work, and innovate here in Massachusetts. The onus is on us to harness the opportunities before us that will bolster our cluster's leadership. This journey will begin with a focus on our workforce and our infrastructure.

Looking ahead, what will be your biggest priorities as Chair to help ensure Massachusetts remains the best place in the world for the life sciences?

While addressing systematic racism will remain a focal point while I'm Chair of the MassBio Board, we must also navigate the new realities brought on by COVID-19. This pandemic is fundamentally changing the way we work, while also shining a light on the brilliance of the life sciences industry. Our industry's response has been incredible, but we need to harness the lessons learned from this pandemic as we drive forward. What will the next disruption be? Are we taking the appropriate steps to identify and address it? That resiliency to combat the world's greatest disruptions will be the key to sustaining our leadership as the best place in the world for the life sciences.

As we move forward, we must broaden our credibility as the best place in the world for the life sciences. A place beyond just R&D where all pieces of industry exist and reach across the state—not just in Kendall Square and Boston. Currently, Massachusetts is highly focused on academic innovation and venture-backed development of therapies and products, and while that has led us to greatness it will not sustain our legacy. Expanding our manufacturing of therapeutics is a critical component to our success. I also hope to work with MassBio's extensive membership to improve people's daily lives so they aren't stuck in traffic every day or delayed by public transportation failures. We're looking ahead to support the next decade of biotech innovation, one with significant opportunities for Massachusetts to sustain its foothold as the world-renowned leader in the life sciences and healthcare innovation. Together, we will make sure Massachusetts remains the best place in the world for the life sciences and talent, and I look forward to help lead this charge. ■

To read the full State of Possible 2025 report, visit MassBio.org/2025-report/

State of Possible

PODCAST

Featuring

Bob Coughlin

MassBio President & CEO

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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.

Cancer Action Network™ American Cancer Society®

Q&A

In July, we spoke with Shalini Vallabhan,
Managing Director, Government Relations for ACS CAN. Shalini has been with the American Cancer Society and ACS CAN for more than 15 years serving in leadership roles to lead advocacy efforts across the New England states and cancer control work across Asia.

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

Treating and reducing pain and suffering from cancer relies as much on public policy as it does on science and medicine. Lawmakers and policymakers at all levels of government play a critical role in decisions that help save lives. The American Cancer Society Cancer Action Network (ACS CAN), the advocacy affiliate of the American Cancer Society, works tirelessly to educate the public, elected and government officials, and candidates about cancer's devastating impact.

We have a nationwide volunteer structure with a presence in all 435 congressional districts. We empower our advocates to influence public policy that will reduce the burden of cancer. We are nonpartisan and the only side we take is the side of cancer patients.

Our public policy priorities are to accelerate cures, improve access to quality care, and reduce tobacco use. Achieving health equity has been and will be the north star of ACS CAN's work.

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

In 2015, ACS CAN launched the One Degree Campaign. At that time, federal funding for medical research had dropped by 24% over 12 years. The campaign set a bold goal – a $6 billion increase for the National Institutes of Health (NIH) which includes a $1 billion increase for the National Cancer Institute (NCI). Within three years, we surpassed this goal! We are witnessing a revolution in cancer diagnostics, immunotherapy, and targeted treatments which has improved patient outcomes. Scientific breakthroughs, which can take decades to see progress, is rooted in NIH and NCI's investment in scientific discovery.

Reducing the cancer burden depends on access to meaningful health coverage for all. ACS CAN has a long track record of success when it comes to increasing access to quality cancer care within Medicare, Medicaid, and private health insurance. ACS CAN advocates to ensure that patients have affordable, timely access to diagnostics and personalized medicine for their cancer diagnosis.

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

Clinical trials enrollment is a challenge for both industry and patients. Enrollment in clinical trials does not accurately reflect the population of cancer patients in our country. Diversifying patient participation in clinical trials is critical to improving patient outcomes – especially outcomes for traditionally underserved populations. ACS CAN has introduced The Henrietta Lacks Enhancing Cancer Research Act to address barriers faced by underrepresented populations to participate in clinical trials. We are also working with Congresswoman Diana DeGette and Congressman Fred Upton on their Cures 2.0 legislation to address needed changes to ClinicalTrials.gov and address

routine costs for clinical trials that are a barrier to patient access to trials.

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

ACS CAN is committed to protecting NIH and NCI funding from cuts that would squander years of progress. Our advocates are in regular communication with their members of Congress reminding them of these critical investments. As the future of the Affordable Care Act is considered, ACS CAN is committed to ensuring lawmakers maintain patient protections that are so important to cancer patients and survivors and ensure insurance coverage is affordable.

In addition, we are excited about projects that will facilitate clinical trial participation. For example, the first project we are looking to launch is to develop a "Blue Button" functionality that will enable site-agnostic trial screening from inside any electronic medical record system. Second, we are focusing on ways to decentralize certain aspects of trials to address the travel and geographic restrictions that often keep patients from participating in trials. The goal is to allow patients to receive as much of their care as possible in the closest appropriate setting, whether that is a local facility or even their home. The regulatory flexibility and site innovation in response to the COVID-19 pandemic has moved the needle on such practices rapidly, and our work will focus on patient-centric needs within the public policy realm to facilitate longer term continuation of best practices.

We are stronger with more voices and we urge anyone interested in supporting this lifesaving mission to visit www.fightcancer.org or to reach out to me at Shalini.Vallabhan@cancer.org. ■

COEUS consulting group, LLC

Q&A

In August, we spoke with Marc Hixson,

President & CEO of Coeus Consulting Group. Marc began his life sciences career on the commercial side of the business in sales and marketing, realizing early on that the ever-changing managed care environment would have a greater impact on the way pharmaceutical products and medical services were delivered. Gaining knowledge and insight through experience, he has directed his efforts to the successful creation and execution of multiple market access strategies and teams for various clients.

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

Founded in 2009, Coeus Consulting Group is a boutique life sciences market access strategy consulting firm offering clients commercial support across the entire development spectrum. Our market access practice works with clients to develop their managed care and pricing and contracting strategy, market access insight generation, HEOR strategy and tactics, launch readiness, account management, operational support, patient access, distribution, and trade and advocacy/policy work. It is our mission to work with clients to show true value of their products to all stakeholders in the healthcare ecosystem to ensure that every patient has access to the therapies they need.

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

Defining value is key to keeping the healthcare ecosystem healthy and viable, Coeus works with all stakeholders to ensure that products are appropriately available to patients when they need them. As cell and gene therapies become the next medical frontier, innovation is needed around the distribution, payment and patient support models that currently exist today.

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

The COVID-19 pandemic has changed the ways in which manufacturers engage with their patients, HCPs and payers, however, there seems to still be a large gap between manufacturers and the stakeholders they engage with.

Going forward, the new normal will entail utilizing the various data sources they have access to, which may have been previously unavailable, and will be key towards closing those gaps and obtaining reimbursement for their products.

What's next for your organization? What are you focused on in the coming year?
Our 3rd party adjudication platform for value and/or outcome-based agreements, Coeus Healthcare is starting to expand rapidly and we've just completed a merger with Mirador Global who enhances our current offerings and expands them outside the US. Later this year we have plans to offer additional services in the agency space under our Coeus Holdings umbrella of companies. It is our pledge to continue to innovate on behalf of our life science clients to ensure a healthy healthcare ecosystem. ■

MBI Q&A

In September, we spoke with Jon Weaver,
President & CEO of Massachusetts Biomedical Initiatives (MBI). MBI is the longest running life sciences incubator in the Commonwealth and has supported over 150 early stage companies that employ over 800 in our regional life science cluster.

Tell us about your organization, its mission, and current initiatives.
At Massachusetts Biomedical Initiatives (MBI), our mantra is "All About Our Companies." MBI is the longest running life sciences business incubator in the Commonwealth and as a non-profit we measure our success through the success of our companies.

MBI has supported over 150 early stage startups with success stories like T. Breeders (acquired by Perkin Elmer), Coley Pharmaceuticals (acquired by Pfizer), Convergent Dental, and Blue Sky Bioservices (acquired by Lake Pharma). We pride ourselves on the success of our entrepreneurs, the jobs they create, and the key role they play in growing the regional life science cluster.

MBI is also an economic development and workforce development advocate. MBI supported projects like the Worcester Biomanufacturing Park, Gateway Park, and supports workforce development efforts like our Increasing Diversity in STEM partnership with Worcester State University & Quinsigamond Community College.

How does your organization's activities help patients now and into the future?
At MBI, our role is to empower the entrepreneurs who bring new hope to patients.

We achieve this primarily through our StartUp & ScaleUp Innovation Centers. Our StartUp Centers provide individual, private labs ranging from 100 – 600 square feet for early stage startups. Our ScaleUp Center provides lab & office suites up to 3,000 square feet to help companies grow to scale. We provide access to shared equipment, CORE facilities, and environmental health & safety support. Most importantly, we view ourselves as an extension of our companies and partner to support their success and overcome challenges.

After maintaining 99% occupancy for over three years, MBI was proud to cut the ribbon on our new StartUp & ScaleUp Centers at 17 Briden Street on August 25, 2020. Through the support of the Massachusetts Life Sciences Center and our State & Local officials, this facility doubles MBI's capacity to support early stage companies.

What do you see as the biggest challenge facing the life sciences industry today?
On the innovation front, MBI is focused on closing the gap between developing great science and building a great company. To do this, our Board of Trustees established a Business Strategy Committee and engaged

Dr. Dana Ono, a Founding Member of MassBio, as an Entrepreneur in Residence (EIR).

The Business Strategy Committee provides a multi-disciplinary evaluation of companies, offers strategic advice, and helps makes connections wherever possible to advance companies on a path to commercialization.

Through our EIR program, Dr. Ono provides more direct tactical support to help our companies achieve fundable milestones. Dr. Ono started several companies of his own, spent two decades in venture capital, and has a great perspective on where the gaps are and how to fill them. We launched this effort in March and have already celebrated a seed round and a pharma collaboration through the program.

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

At the Ribbon Cutting, 17 Briden Street was already home to 17 companies, exceeding our year 1 goal. Over the next year, we'll continue to onboard new companies and then dig in to support their success. Through our Business Strategy Committee and EIR efforts, we're focused on fundable milestones!

With WuXi's $60M biomanufacturing facility underway and Galaxy Life Sciences $50M biomanufacturing project shortly behind it, Worcester and Central Massachusetts are gaining momentum as a critical node in the regional life sciences cluster. Our strategy is to identify opportunities for Greater Worcester to complement the success of the regional cluster in biomanufacturing, supporting startups, contract research, and drug discovery. Supporting Worcester's Biomanufacturing Park and helping promote the proposed biotech buildings around WooSox Stadium will be key priorities. ■

mdbiosciences. Neuroscience Discovery Services

Q&A

In October, we spoke with Anya Moradian,
Vice President of Business Development, MD Biosciences Neuro, who leads business development locally in Cambridge and across the United States.

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

MD Biosciences Neuro is an industry-leading provider of preclinical and translational research services focused on the development of pharmaceutical and diagnostic assets. Established in 2006 in Israel, we recently inaugurated our business presence in Cambridge, MA to interact more closely and directly with our sponsors and partners. We provide services across a range of therapeutic areas and specialize in neurology and pain. MD Biosciences Neuro aims to develop predictive and translational preclinical models and methodologies to improve approval rates in clinical trials and provide safe and effective treatments for patients. As a leader in this field, we are continuously advancing our depth of services and are currently focused on growing partnerships locally in Cambridge.

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

We are expanding our range of translational models so that our partners can use preclinical research as a reliable predictor for clinical trial results. We have established models and methods that help build a complete story of neurological disease and effective treatments with the potential to impact patients immediately. We regularly perform in vivo electrophysiology in our rodent models, which provides quantifiable measures for neurological disease severity, pain side effects, and treatment effects.

We also heavily emphasize our translational pig models, as this species shares valuable similarities with humans, including skin and neurological characteristics. We have developed models for neuropathic pain, post-operative pain, and wound healing and have helped our partners achieve success in clinical trials. These results are directly impacting patients and we are looking forward to more opportunities in advancing translational research.

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

One of the challenges we have in the life sciences industry is improving the approval rate of neurology and pain treatments. While our understanding of neurology is growing, there are still several limitations. Compared to other therapeutic areas, there are fewer predictive models, fewer known biomarkers that help quantify disease, and a narrow scope of human data. Consequently, the development of neurological treatments heavily depends on qualitative and subjective measures, which is an inadequate predictor of disease and treatment. MD Biosciences Neuro is combatting this challenge by developing novel quantitative measures that help de-risk clinical trials. Our translational models are more predictive than traditional rodent models, and we expect improved approval rates as a result.

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

We are currently expanding our services in Cambridge and are interested in pursuing collaborative opportunities that will advance drug discovery in neurology and pain. We are continuously developing novel models and methodologies that will help our partners achieve preclinical translatability and clinical success. ■

SERVIER

Q&A

In November, we spoke with Rekha Paleyanda,
Director, Servier BioInnovation. Rekha heads business development locally in Cambridge and across the US with a focus on in-licensing innovative therapies from early-stage through clinical proof of concept.

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

Servier BioInnovation (SBI) was founded in February 2018 as the early-stage partnering engine of the Servier Group. Servier Group is an independent, global pharmaceutical company governed by a nonprofit foundation headquartered in France, with revenues of $5.2 billion and subsidiaries in the United States and China. SBI is a joint initiative between the Group's global Research & Development and Business, Development, and Licensing functions, a one-stop shop for partnering that covers the U.S. from Cambridge, Massachusetts.

Servier BioInnovation was set up in the heart of Kendall Square to foster strong relationships within the world's most innovative ecosystem. Our team is responsible for creating awareness and identifying R&D opportunities, while expediting business development and licensing activities in the U.S. We also actively sponsor emerging science and entrepreneurship at leading biotech launch pads and science incubators, while also supporting the next generation of scientific leaders via educational and postdoctoral programs with local universities and medical centers.

Indeed, we do not innovate in isolation: Servier currently has more than 30 active partnerships worldwide and over 40 research collaborations. Our research focuses on oncology, neurodegenerative, and immune-inflammatory diseases; together with lifecycle management of our established cardiometabolic and neuropsychiatric products. In addition to a commercial footprint in 150 countries, Servier operates four research institutes and 15 clinical research centers across the globe. Locally and across

North America, we are now equipped to offer clinical operations via the Servier Pharmaceuticals subsidiary in Boston's Seaport District.

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

Servier medicines treat well over 100 million patients every day on a worldwide basis. Patients are at the center of our focus as we are committed to therapeutic progress to serve their specific needs in every country in which we are present. Servier is dedicated to supporting patients, caregivers, families, and patient organizations. Some of our U.S. initiatives include our Patient Engagement Teams, Patient Services Program, and Patient Advocacy Program. We work with foundations to serve our patients' needs, for example, we sponsor the LLS COVID-19 Patient Financial Aid Program.

Our unique governance structure allows us to dedicate a significant portion, 25%, of our revenue from innovative products annually to R&D activities, demonstrating our commitment to drug discovery and development for critical unmet needs. Collaborations are a core part of our business, and at Servier, we partner for a purpose: our patients. We actively foster and promote emerging science through partnerships and alliances with scientists, physicians, entrepreneurs, disease-oriented non-profit organizations, and patient groups.

Patients are at the heart of every decision we make, and we seek partners who share our values and commitment to improving the lives of the patients, caregivers, and families we serve. We partner with others for the advancement of research, while leveraging our strengths as a private company with more than 60 years of success in drug development. Due to our worldwide commercial presence, we are able to increase the global reach of innovative drugs for our partners to far corners of the globe and therefore, treat a large diverse patient population.

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

Advances in science and medicine are occurring at a very rapid pace. As early-stage innovators, a big challenge for our industry is keeping abreast of these developments, deciding what is therapeutically actionable, and implementing new programs with the right partner. Moving at the speed of science requires large organizations to constantly evolve their ways of working, to ensure investment and engagement with the needed agility. By building true collaborations with partners, Servier is focused on finding the right modality for the right target in the right patient population at the right time.

Another challenge facing our industry concerns talent, especially ensuring a diverse and inclusive environment for STEM professionals. Our commitment to finding solutions has led us to being very intentional locally, we actively support organizations such as Women in the Enterprise of Science and Technology, the World Frontiers Forum, and have signed the MassBio Pledge to Create a More Equitable and Inclusive Life Sciences Industry.

It will likely take us several years to realize the full impact of the COVID-19 pandemic on our industry. Factors such as delays due to implementing new safety procedures, in the clinical trials process, in patient diagnoses and treatment, along with reduced R&D and commercial output could impact future revenues. There has been a rush on developing COVID-related therapeutics, and this refocused attention will pose a challenge on the funding and progress of non-COVID programs.

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

Servier Group is undergoing a transformation to become a leading provider of therapies in oncology, including hard-to-treat and pediatric cancers. Servier's pipeline currently includes innovative approaches targeting solid tumors and hematological malignancies with 15 projects in clinical development and 24 projects in drug discovery, encompassing a multitude of therapeutic modalities, including small molecules, biologics, and cell therapies. Our focus is on two main areas of oncology, immuno-oncology and cancer-cell signaling, in hematological malignancies and certain solid tumors. Servier is committed to investing around 50% of the annual R&D spend in generating innovative and specialized oncology therapeutics. We are committed to partnering in the Americas to expand our presence and drive our portfolio forward for the benefit of patients. We are actively looking to strengthen our portfolio by licensing-in or acquiring late-stage products in this space. We also have an interest in expanding our translational medicine efforts.

Our group supports innovation in local ecosystems be-

yond Massachusetts. On this side of the Atlantic, a good example is the construction of the Servier Paris-Saclay Research Institute. This state-of-the-art facility will include an incubator for local biotech companies and house around 1500 people dedicated to the discovery and development of innovative therapeutic solutions. This investment is another testament to Servier's commitment to furthering life science innovation and entrepreneurship. ■

Note: This Spotlight article was written by the local members of the Servier BioInnovation team

Cure Duchenne Q&A

In December, we spoke with Debra Miller,

Founder and CEO, CureDuchenne. She and her husband started the organization in 2003 after their only son, Hawken, was diagnosed with Duchenne. Debra and her team have brought many firsts to the Duchenne community, including a Duchenne-focused venture philanthropy model, an annual nationwide community education event tour, a Duchenne Physical Therapy Certification program, and an open access Biobank.

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

CureDuchenne's mission is to improve the lives of everyone affected by Duchenne through accelerating research to find the cure, redefining care, and empowering the Duchenne community. Duchenne is a devastating muscle disease and is the most common form of muscular dystrophy, occurring in approximately 1:5,000 male births. Those affected with Duchenne lose their ability to walk, feed themselves, breathe independently, and ultimately succumb to heart and respiratory failure. But there's hope through new pharmacological and gene-based therapies.

CureDuchenne is recognized as a global leader in research, patient care, and innovation for improving and extending the lives of those with Duchenne. Our actions fuel hope for families, enable progress for drug development, and extend ambulation for patients in collaboration with biopharmaceutical companies, medical and healthcare professionals, our scientific advisory board, and our board of directors.

CureDuchenne breaks the traditional charitable mold and balances passion with business acumen. Our innovative venture philanthropy model funds groundbreaking research, early diagnosis, and treatment access. With pioneering education and support programs, our organization drives real change for those with Duchenne muscular dystrophy and their loved ones.

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

"Embrace this Moment" has been a mantra we've lived by, especially this year as we've focused our CureDuchenne Cares program on providing virtual events due to COVID-19. While we usually go around the country creating support networks of families, clinicians, and others, this year we've done that through Zoom, having coffee (or cocktails) with families. We're able to keep that sense of being together and give them what they need: conversations about mental health, clinical trials, nutrition, school, physical therapy, insights from all kinds of providers. We've even done it in Spanish, reaching global Spanish-speaking communities this year. We're always looking for life's silver linings and appreciating all its precious moments!

We've also kept moving forward with our venture philanthropy efforts, making several investments this year—never stopping in our relentless pursuit to find treatments and cures. CureDuchenne Ventures deploys donor dollars to fund Duchenne muscular dystrophy drug development with a vision of bringing transformative treatments and cures for everyone with Duchenne. Our robust pipeline of therapies comes from a diverse array of cutting-edge technologies used to treat all aspects of Duchenne. CureDuchenne Ventures' funding of companies at early and critical times helps their Duchenne projects obtain the important data necessary for those companies to attract

future investments. We measure success by seeing a company demonstrate the evidence that its approach is a viable one to take in tackling Duchenne, and then see that company raise the capital needed to continue their progress and conduct clinical trials. In this way, a successful CureDuchenne Ventures investment is a success for the entire Duchenne community.

For example, one of CureDuchenne's recent investments includes Myosana Therapeutics, which is developing a virus-free gene therapy delivery platform to deliver full-length dystrophin and overcome many of the immune system limitations of viral-delivered gene therapy. CureDuchenne also invested in Dyne Therapeutics (headquartered in Waltham, MA), whose FORCE ™ platform enhances the delivery of exon skipping therapeutics to skeletal, cardiac, and smooth muscle with the potential to improve efficacy and reduce dosing frequency.

Finding and supporting innovation to build chances for the future while embracing this moment is CureDuchenne's constant path to hope.

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

The ability for systems to keep up with innovation and ensuring accessibility and meaningfulness to patients and their families are the biggest challenges the life sciences industry faces today. Old ways of developing and paying for treatments cannot be sustained in a world where the FDA is being inundated with several hundred new cell and gene therapies and other innovative treatments per year. The treatments are not the only things that are innovative, either. It is also innovative to include patients and caregivers' insights, experiences, and preferences as integral decision-making components of drug development. It is innovative to find new ways of thinking about manufacturing more efficiently and precisely. Increasingly accessible genetic testing and diagnostics is innovative. Finding new ways to base payment arrangements on value is innovative. CureDuchenne is excited by the prospects of all this innovation coming together, has pioneered some of it, and will be there to solve some of it as well. We work to bring every step of this innovation back to the Duchenne community.

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

Getting to the other side of COVID-19, to the new ways of being together with the Duchenne community, our other colleagues and friends, is certainly a focus, along with bringing the community back together when it's safe. We will move ahead with the next strategic phases of the CureDuchenne Biobank, which include continuing to collect samples of skin tissue and blood samples, as well as data from Duchenne patients made accessible to qualified Duchenne researchers. Continuing to embed health equity efforts more deeply into everything we do is paramount. In 2021, we'll be prioritizing policy strategies around newborn screening and access as well as supporting initiatives including outcomes-based agreements, PDUFA VII, and precision medicine. We look forward to a year of coming together, progress, innovation, and of continued partnership with our biotech and pharmaceutical colleagues in doing all we can to accelerate research and innovation and empower the community with positive solutions. ■

MassBio's Industry Snapshot Affirms the Strength of the Life Sciences—Despite COVID-19 Uncertainty

By Ben Bradford, Vice President of Membership and Economic Development, MassBio

In late July, MassBio released its 2020 Industry Snapshot report which analyzes the strength of the Massachusetts life sciences industry through 2019 and the first half of 2020. Despite the economic downturn caused by the COVID-19 pandemic, the life sciences cluster in Massachusetts remains a powerhouse.

In the first half of 2020, Massachusetts biopharma companies raised $2.1 billion in venture capital investment—nearly as much as was raised in all of 2019 ($3.1 billion). Additionally, the IPO market in Massachusetts remains prominent, helping to fuel the strength of the IPO market we're seeing across the United States this year. We saw seven IPOs from Massachusetts biotech companies in the first half of 2020, making up 33% of all US-based biotech IPOs, and raising an average of $187 million. In comparison, through all of 2019, there were 10 biotech IPOs from Massachusetts companies in 2019, accounting for 23% of all US-based biotech IPOs. Of those, 64% were located outside of Cambridge, demonstrating the migration out of the Cambridge epicenter and into mini-clusters throughout the state—a positive trend for our industry as we continue to promote the growth of the suburbs.

When taking the differences between 2019 and the first half of 2020 into account, we must consider the severe drug pricing legislation we saw in 2019—both in Massachusetts and federally. As we predicted, these harsh regulations led to a decline in investments until the pandemic hit. The outbreak of COVID-19 put a spotlight on the true value of the biopharma industry and the outsized role Massachusetts life sciences companies are playing in the pandemic. Over 90 companies in Massachusetts alone are working to develop diagnostics,

therapeutics, and vaccines to bring us out of this pandemic.

Much of this success can be attributed to the incredible talent Massachusetts boasts and the influence this cluster wields in R&D. As our Industry Snapshot shows, biotech R&D jobs reached 46,000 in 2019, an increase of 18% from 2018, and industry jobs as a whole reached nearly 80,000 in 2019, the largest year-over-year growth since 2007. In the last 15 years, industry jobs have increased 94%. This growth is remarkable, but it also presents challenges. As we look to better recruit and retain the best and brightest from around the world, we need to get students excited about careers in the life sciences to grow our pipeline, while also ensuring we have a diversity of talent that represents the patient populations we serve.

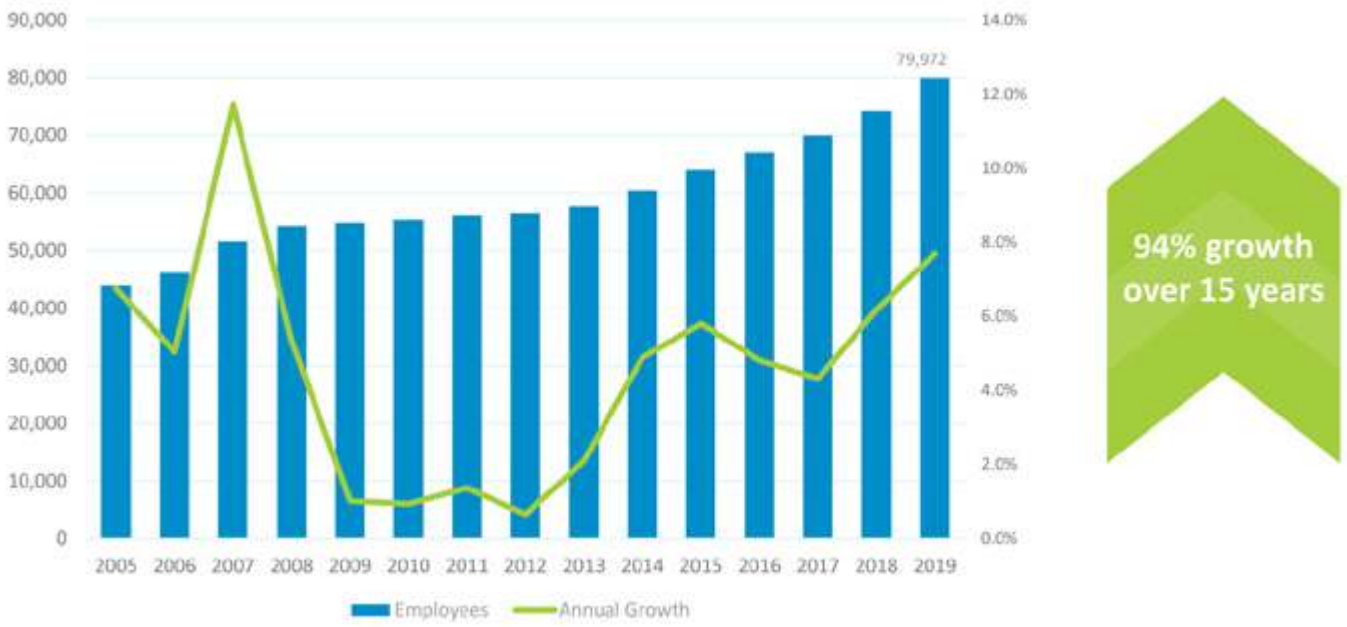
This has been a historic year and as we continue to navigate this crisis, we look forward to working with our vast membership to sustain our industry's strength and ensure we remain the best place in the world for the life sciences. ■

To download the full report, visit
MassBio.org/industry-snapshot/

Massachusetts Biopharma Industry Employment

Industry jobs grew by **94%** in the last 15 years adding over 38,000 jobs, and by **45%** in the last 10 years adding over 24,500 jobs.

In 2019, biopharma employment grew at **7.7%**, the fastest year-over-year rate since 2007.



Source: Privately owned companies, U.S. Bureau of Labor Statistics, Quarterly Census of Employment and Wages (QCEW)

MassBio's State of Possible Conference 2020 *Gone Virtual*

By Laura Rudberg, Director of Events, MassBio

The outbreak of COVID-19 brought a lot of change to 2020—and with it a complete transformation of events as we know it. This year's State of Possible Conference, MassBio's Annual Meeting, was moved to a virtual platform for the very first time and, though we missed the opportunity to convene in-person, we were excited to engage with the virtual world and gather the best and brightest minds in the life sciences from Massachusetts and beyond. We heard inspiring Possible Talks®, panels, and keynotes from patients, students, and life sciences professionals, and sparked thoughtful conversation around how this pandemic could reshape the perception of our industry.

We also honored Dr. Abbie Celniker (Third Rock Ventures) and Dr. Kenneth Anderson (Dana-Farber Cancer Institute & Harvard Medical School) with our Henri A. Termeer Innovative Leadership Award and State of Possible Award, respectively, for their incredible work contributing to the growth and success of the life sciences industry in Massachusetts and the well-being of its citizens. Overall, we were heartened by the support and engagement from our attendees as they adapted with us to this new reality.

As an added component to the State of Possible Conference, MassBio appointed seven new life sciences executives to its Board of Directors, increasing the diversity of the MassBio Board and better positioning our organization to capitalize on the opportunities to sustain and grow the Massachusetts life sciences industry. However, in a bittersweet moment, we also said farewell to our past Board Chair, David Lucchino, who served MassBio for more than two years, and made a greater impact on our organization, the industry, and our ability to support our members' diverse needs than he'll ever know. While we relished the last two years with David at the helm, we look forward to our future under the leadership of our new Board Chair, Charles Wilson, and are excited to see what the next two years will bring.

We continue to be blown away by our industry's prowess during such an unprecedented time. It is because of our 1300+ members that Massachusetts is the State of Possible, and we cannot wait to see you all again (in-person) soon. ■

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