Vol. 11, No. 3 | A publication of the Massachusetts Biotechnology Council | Winter 2018

MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

THE VALUE OF HEALTH

MassBio positions itself as a thought leader around drug pricing

By Meaghan Casey

As the debate over drug pricing intensifies, MassBio is refocusing the discussion around the value of health. In 2019, through a three-part series of white papers and events, MassBio will explore how to define value, how public payers are impacting policy and access, and the disruptors set to change the healthcare system. The series will look at what's working well and what needs to change in order to both reward breakthroughs and lower patients' out-of-pocket burden.

MassBio's President and CEO Bob Coughlin put the need for this work succinctly: "If we don't come up with market-based solutions now, the government will. And they'll get it wrong. It's too complicated, and it's too important. Now is the time for leadership."

Coughlin says the true value of medicine lies in the outcomes achieved, and the industry needs to be doing more to highlight that.

"Prescription drugs save lives," said Coughlin. "They keep people healthy, make them productive members of society, and they save costs across the healthcare system."

Coughlin, whose son has cystic fibrosis, understands all too well the daily toll that is put on families and the healthcare system when the underlying cause of a disease is left untreated.

His son takes dozens of pills each day, receives X-rays and chest physical therapy and is frequently admitted to the hospital. Treatments

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MassBio's Patient Advocacy Summit focused on showcasing best practices of how life sciences companies can more fully incorporate the patient voice into the work they do — not just approaching regulatory applications or at commercialization, but throughout the drug development cycle. Above, Erin Mistry, Managing Director of Pricing & Market Access at Syneos Health, speaks about the changing landscape of value and access.

See story, pages 4-5

MassBio news

A PUBLICATION OF:
Massachusetts Biotechnology Council
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617-674-5100 ■ www.massbio.org

PRODUCED BY:
GRANT COMMUNICATIONS CONSULTING GROUP
www.grantgroup.com

Keeping Massachusetts at the top

ROBERT K. COUGHLIN

The value of MassBio membership has never been higher. From financial savings through our Purchasing Consortium, to unparalleled professional development and networking events, to our on-the-ground support of early stage innovators and our non-stop public policy advocacy, our members benefit in myriad ways.

In 2018, we celebrated the State of Possible at the BIO International Convention to recognize the incredible contributions of those professionals that make up the Massachusetts life sciences cluster – a theme that will carry through 2019 as we rebrand our Annual Meeting to the State of Possible Conference. But we won't just look back at our progress, we'll also tackle the most pressing challenges facing our industry in 2019. We plan to strategize how Massachusetts can lead the future of digital health, and we'll be convening a series of events and white papers on the Value of Health around prescription drug pricing and access.

Earlier this year, we made history as the first-ever trade association to hire an externally facing Director of Diversity & Inclusion whose job is to help our members plan and implement strategies to improve diversity at their companies. Next year, our D&I strategy will expand while being more focused on measuring progress and holding our members accountable.

We'll continue to grow our existing support for members by adding new vendors and services to our Purchasing Consortium. We've hired a Vice President of Consortium Operations to lead this expansion and to ensure our members receive far more value and savings in the years ahead.

Our Innovation Services team is set to expand as well with more offerings for early-stage entrepreneurs and by growing the outreach and engagement with academic medical centers and universities. And our public policy efforts will be more significant than ever as both state and federal policymakers have ramped up the frequency and intensity of their attacks on the life sciences industry.

Together, we'll work to keep Massachusetts the best place in the world for life sciences and we'll continue to put patients at the heart of everything we do.

Robert K Coughlin is President & CEO of MassBio.

Committed to building our workforce

DAVID LUCCHINO

With MassBioEd forecasting an additional 12,000 jobs by 2023, it's clear that our industry must look to all available talent pools to fill these positions. We also know that Massachusetts can only remain the best place in the world for life sciences if companies hire the best and brightest, regardless of gender, sexual orientation, race, ethnicity and more. Unfortunately, the data shows we're not doing a great job currently. Women, African-Americans, Latinos/Hispanics and other minorities are woefully underrepresented in leadership positions, which is why MassBio has taken such a strong stance on improving diversity and inclusion – a cause I am fully committed to as Chair.

I've always believed great things happen when people figure out how to work together, so I identified those groups that were already focused on workforce development and getting young people excited about career opportunities in the life sciences to see how we could combine our forces to enact real change. To that end, I'm proud to announce Project Onramp, a collaboration between MassBio, MassBioEd, the Massachusetts Life Sciences Center, Life Science Cares and Bottom Line to connect underserved students with industry internships so they are competitively positioned to join these companies upon graduation.

Why is this necessary? For students seeking a career in the life sciences, there is nothing as effective as an internship at a local biotech company. But at many companies, internship opportunities go unpublicized, and many are reserved for those with personal connections. This effectively shuts out promising students from underserved communities – so we're going to change that. Furthermore, internships and future job opportunities in non-science positions abound in our industry and will be part of Project Onramp.

The life sciences industry has become a tremendous economic engine for greater Boston and Massachusetts. Now's the time to spread the wealth and opportunities across the state and to all residents of the State of Possible. Change won't happen overnight, but as we look five or even 10 years into the future, we must prepare the most diverse population possible to tackle the toughest unmet medical needs for patients. I'm calling on all MassBio members to create or commit summer 2019 internships at your company for this new program to ensure our cluster can continue its incredible growth.

David Lucchino is Chair of the MassBio Board and CEO of Frequency Therapeutics.

NEW MASSBIO MEMBERS

Adlai Nortye
AgenTus Therapeutics
Agility Labs by Triple Ring Technologies
Alexandria Launch Labs
Alloplex
Akili Laboratories
Akouos Inc.
Anchiano
Amide Technologies
Axial Biotherapeutics
Axol Biosciences
Backpack Health
Beat NB Cancer Foundation
BioAgilytix
Biolojic Design
Cambridge Trust
Celgene Corporation
Centogene US
Central Pharma
Cosec Consulting
Courageous Parents Network
CSC Leasing
Cures Within Reach for Cancer
CXL, Ophthalmics
DDF US Discovery
Directed Genomics LLC
DivcoWest
Entourage Therapeutics
Entrada Therapeutics
Excite Pharma Services
Festo USA
Flaskworks
Genevant
Genialis
GNEMSDC
Geneius Biotechnology
Halloran Consulting Group
Higgins Group
Honeycomb Biotechnologies
Hongxing Erke Investment Inc.
Imbue Partners
immunoSCAPE
ImmunSYS, Inc.
Insphero
Invest Northern Ireland
Inzen Tx
ISS, Inc.
iTeos Therapeutics
Kymera Therapeutics
Labshares Newton
National Brain Tumor Society
New Perspectives
Novotech
OMPI of America
Oncologie, Inc.
Orion Clinical Services
PHC Corp. of NA
Preomic GmbH
PureTech Health
Quay Pharaceuticals
Raremark
Renovia Inc
Repare Therapeutics
Sartorius Stedim
Skyhawk Therapeutics
Skyland Analytics
SLAS
Solarea Bio
Tilos Therapeutics
Toxic Reports
Valeritas Inc
Witt/Kieffer
Woodland Pharmaceuticals
Yield10 Bioscience
Young BioPharma
ZoBio

Jobs and investments on the rise in Massachusetts

Massachusetts continues to be home to a biotechnology supercluster that is second to none, as revealed by MassBio's 2018 Industry Snapshot report.

The report, released in August, shows that the Massachusetts biopharma industry is growing at an impressive rate, captured through investments, IPOs, job growth, lab space development and more.

Industry jobs in Massachusetts grew by 4.3 percent in 2017, and by 28 percent in the last 10 years. Across the state, there are nearly 70,000 biopharma employees, collectively earning $10.4 billion in wages. In terms of drug development, these employees are researching and developing products for patients with more than 400 different medical indications. Oncology continues to be the most frequently researched therapeutic area.

As jobs have multiplied, so has commercial lab space. More than 12 million square feet of lab space has been added to Massachusetts in the last 10 years, which is an increase of 71 percent.

Elizabeth Steele, MassBio's Vice President of Programs & Global Affairs, says workforce development will become even more critical in the years ahead.

"MassBioEd, our sister organization, is predicting nearly 12,000 new jobs by May 2023, and we are looking to our high schools and local universities to fill them," said Steele. "Competition for talent is already at an all-time-high, so we need new workers coming into the industry, and that starts with our schools."

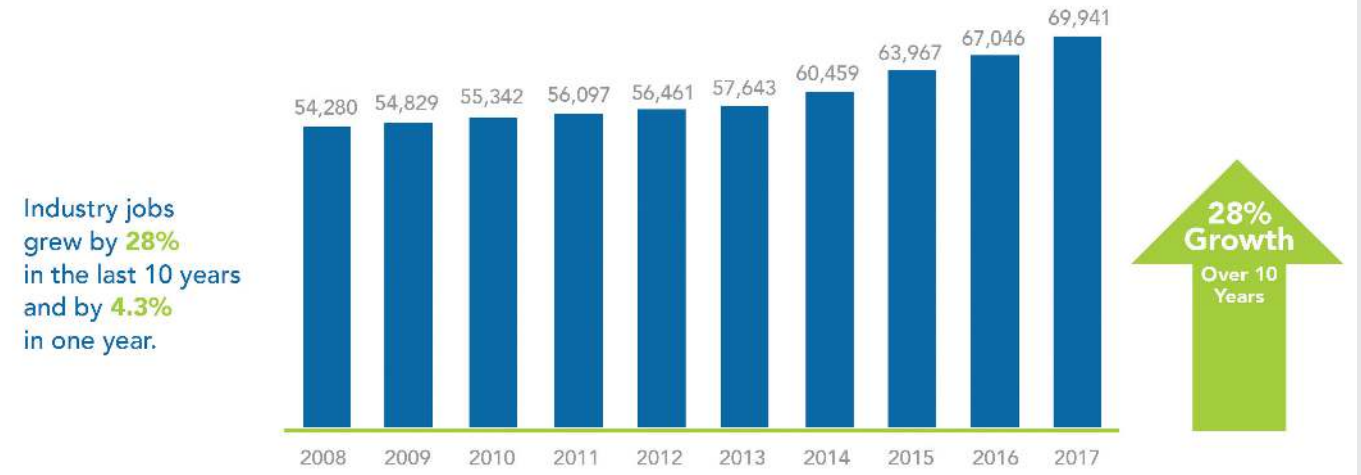
The industry's sustained growth in Massachusetts has captured the attention of public and private investors alike. The state continues to attract the greatest share of NIH funding per capita, with five of the top six NIH-funded independent hospitals, 57 percent of all NIH funding to independent hospitals, and $1.25 billion to Massachusetts centers of higher education and research institutes in 2017. Massachusetts biopharmas dominated both the IPO market and venture capital investment in 2017, accounting for 48 percent of all U.S. based biotech IPOs and 37 percent of all U.S. biopharma VC dollars. VC investors poured $3.1 billion into these companies — the highest ever.

"When looking at the first half of 2018, it looks like we're on track to beat 2017 records, with $2.7 billion already invested," said Steele.

Steele points out that innovation is driving the consistent investment.

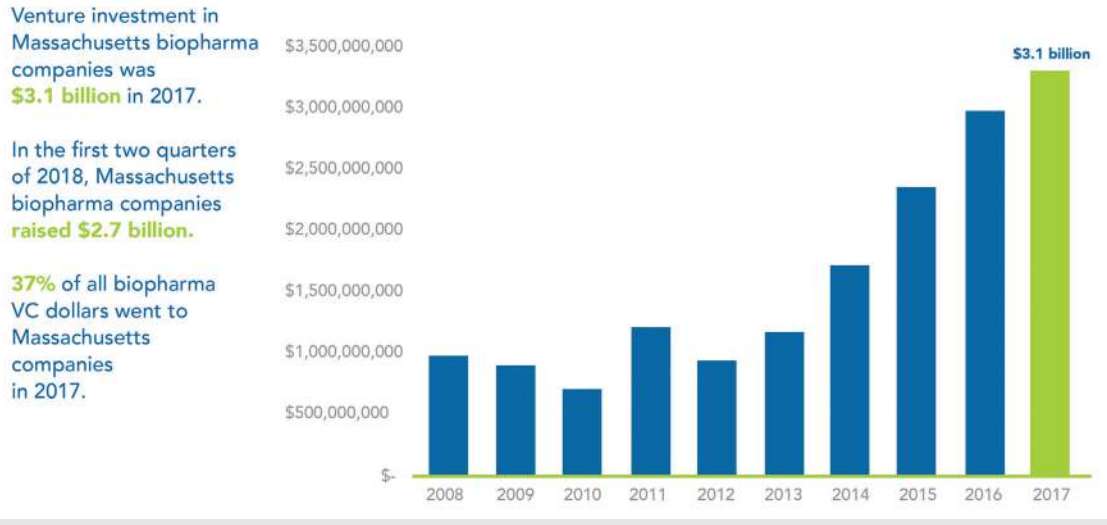
"The money is following the science, and the science behind these companies is strong," she said. "We're seeing a new wave of breakthrough therapies that treat the underlying cause of disease, not just the symptoms. People are even starting to use the word cure for some of them. Savvy investors want to be a part of this success."

MASSACHUSETTS BIOPHARMA INDUSTRY EMPLOYMENT

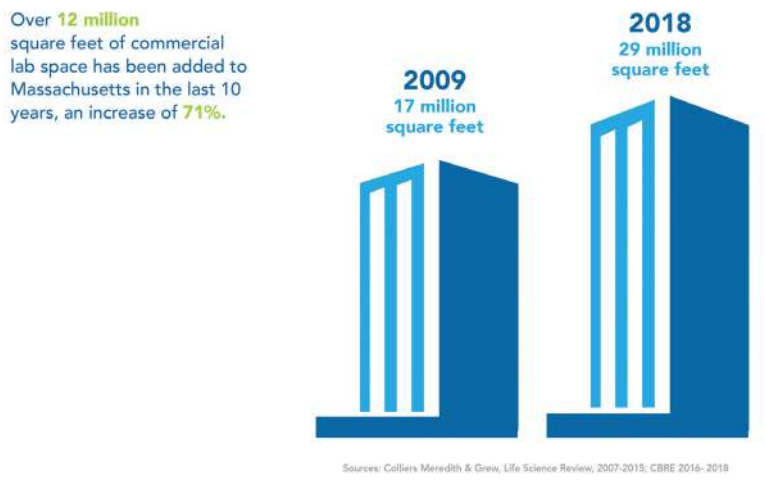


Source: Privately owned companies, U.S. Bureau of Labor Statistics, Quarterly Census of Employment and Wages (QCEW); MassBio

VENTURE CAPITAL INVESTMENT: BIOPHARMA



LAB INVENTORY GROWTH



Sources: Colliers Meredith & Grew, Life Science Review, 2007-2015; CBRE 2016- 2018

Patient advocacy at a crossroads

Summit addresses adding value and measuring impact of patient involvement

By Meaghan Casey

Seven hours into MassBio's Patient Advocacy Summit, attendees erupted in resounding applause in response to MassBio President & CEO Robert K. Coughlin's closing statement: "There's nothing that saves more money in healthcare than a cure."

Coughlin was building on a point that keynote speaker Dr. Phil Reilly had made earlier in the morning. Reilly, the author of "Orphan: The Quest to Save Children with Rare Genetic Disorders," outlined past, present and future advances in treating rare genetic disorders. He noted that life-saving treatments and cures far outweigh the initial cost to get new products to market.

"New therapies will save society and the healthcare system a lot of money," said Reilly. "Investment in rare diseases will help not only those patients, but also people with more common, related diseases."

Reilly, a doctor, lawyer and venture partner at Third Rock Ventures, drew from his experiences as a respected clinical geneticist with an extensive track record of launching and building companies in the rare disease space. He explored four disorders that were once considered hopeless — phenylketonuria (PKU), beta thalassemia, hemophilia A and Gaucher disease—and, through breakthroughs in screening, medicine and gene therapy, are now treatable. Marveling at the advances in genome sequencing and regenerative medicine, coupled with the cost of genome therapy dropping by a millionfold in the last 20 years, Reilly says there is new promise in understanding complex diseases such as autism and schizophrenia.

Erin Mistry, Managing Director of Global Pricing and Market Access at Syneos Health, delivered the second keynote presentation at the Summit, which was held on Nov. 2 at the Hyatt Regency in Cambridge. Mistry spoke about the changing landscape of value and access to treatments. She warned that although medical science will continue to accelerate, patients will meet roadblocks due to evolving pricing trends. She anticipates copay accumulator programs will be standard by 2020. Globally, she is seeing incentives to utilize generics and biosimilars, as well as a shift in cost from payers to patients, with payers emboldened to say no to new breakthroughs.

"Drug approval doesn't always equal market access," said Mistry. "Patients and physicians assess value by the clinical benefits to the patient, quality of life and affordability, while payers assess value by cost and the added benefit over existing drugs."

She stresses that communication of value must begin early, with all stakeholders weighing in on objectives and target audience.

Attendees share ideas during one of the breakout sessions. **PHOTOS: DAVID FOX**

> One of our key takeaways is that you wouldn't believe how much small things make a **HUGE DIFFERENCE.**
>
> – Mike Jagielski

"Patient advocacy groups can be the shapers and the partners that put the patient first," Mistry said.

The program's panelists led interesting discussions on how to integrate the patient into development and how to bring the patient's voice into clinical trials. Laura Greco, a stage IV lung cancer patient and activist, noted that just getting to a clinical trial site — particularly without travel and per diem expenses covered — is often an inhibitor.

"Why do I have to drive 3.5 hours?" Greco asked. "Can some of these tests be done remotely? And I'm lucky. Others have to fly in every six to eight weeks and spend several hundred or thousands of dollars. It needs to be less of a financial burden."

Rachael Brake, Global Program Lead at Takeda, explained there is often a tug-of-war between meeting patients' needs and adhering to a structured setting for results.

"We're in a regulated environment, so one of the challenges is customizing a trial for a patient," Brake said. "But low-hanging fruit, like reimbursement for parking, can sometime make a difference. If that means a person is one-percent more motivated to come in, it's a win."

Mike Jagielski, CEO of KCR, a contract research organization that has partnered with a service design company to conduct a study that listens to patients' voices, agreed with that sentiment. With the #HumanBehindEveryNumber project, KCR is attempting to fully map the patient's journey during the clinical trial, and to understand the emotions associated with each stage.

"One of our key takeaways is that you wouldn't believe how much small things make a huge difference," Jagielski said.

Nick Lunger, whose young son has fragile X syndrome, argued for the benefits of seeing a patient outside of the clinical setting, and not just for the sake of convenience.

"When it comes to patient advocacy, come to us; don't make us come to you," said Lunger. "If you want to learn about fragile X, you want to watch my son in his own environment. All the little details — watching him brush his teeth or walk into a room — that's fragile X. It's not bringing him out of his comfort zone. To understand, visit our world and see how it really is. Make it personal. Engagement comes from feeling like we're part of your journey, not just a data point along the way."

Jill Bell, Director of Global Outcomes Research at Takeda, took note.

"At Takeda, we're really interested in the patient experience when thinking about protocol development," said Bell. "We need to talk to patients and learn about their day-to-day. We want to capture their full experience and measure what's most important to them. They have to be driving our decisions."

The event also included ample networking opportunities and breakout sessions on ways in which organizations are measuring the impact of their patient engagement efforts; methods to incorporate the FDA's patient-focused drug development guidance documents; and creative ways in which companies have added value to their teams. During an engaging workshop session, attendees addressed the benefits and pain points based on where their patient advocacy function sits.

1. Moderator Scott Campbell, lung cancer patient Laura Greco, Kinser Cancelmo, an advocate for juvenile Huntington's disease, and Nick Lunger, a parent of a fragile X patient, discuss patient perspectives.
2. Dr. Ann-Marie Richard of Pfizer and Shana Yansen of Harvard Global Health Institute share a laugh.
3. Parisa Sanandaji of Intercept Pharmaceuticals discusses the role of patient advocacy.
4. Eliane Markoff, founder of Art in Giving, points out reasons to be patient-driven.
5. Dr. Phil Reilly of Third Rock Ventures speaks on integrating the patient into development.
6. Moderator Mike Jagielski, Jill Bell and Rachael Brake, both of Takeda, and patient advocate Nick Lunger bring the focus to clinical trials.
7. Maura Gavaghan of Translate Bio and Lindsey Smith of Corbus Pharmaceuticals brainstorm during a workshop.

Teeing off for MassBioEd

MassBio hosted its 24th Annual Golf Classic to benefit MassBioEd on Sept. 14 at The International in Bolton. Participants enjoyed the club's two award-winning, 18-hole courses — the Pines, designed by Robert Trent Jones, and the Oaks, designed by Tom Fazi. The winners were J. Calnan & Associates on the Oaks and CSC Leasing on the Pines.

The event raised approximately $123,000, which will be used to support MassBioEd in its efforts to engage teachers, inspire students and guide the life sciences workforce. Its flagship program, BioTeach, supports high school teachers as they work to launch or expand lab-centered, inquiry-based biotechnology and life sciences curricula in their classrooms. Its Take Out Training and Equipment (TOTE) program, newly launched last year, has been successful in providing teachers in smaller schools with classroom-ready activities and labs. Last year, 41 Massachusetts public schools received grants for needed equipment and supplies from MassBioEd, and 1,188 students participated in Career Exploration Days, Biotech Futures and Speakers in Schools events.

Brian Denehy attempts to hit the 100-yard field goal. The contest was sponsored by GE.

Gilbert Zawaira putts on the Oaks Course.

The Oaks Course winners were Mark Herzog, Greg Lewis, Dan Annacone and Brian Mikolaycik.

Molly Canfield chips to the green.

There is power in numbers!

Check out the savings actually achieved by MassBio members:

By aggregating the purchasing power of the member companies within MassBio, the MassBio Purchasing Consortium allows members to have a strong presence in the marketplace so they can bring more to their bottom lines. These case studies show savings actually achieved by MassBio members. MassBio continuously evaluates the needs of member companies and the existing contracts to ensure the best value.

Employees: 318
Medium Biotech

Items Purchased:

- *Packaged Gases* • *Travel* • *Lab Supplies*
- *Lab Equipment Maintenance*
- *Biomedical Waste Management* • *Shipping Services*
- *Hazardous Waste Management*
- *Office Supplies*

Consortium Spend	$3,915,468
Est. Savings Off List Price	$1,403,244
+ Year-End Rebate	$ 27,100
- MassBio Annual Dues	$ 14,299
Bottom Line Savings	**$1,416,045**

Employees: 120,000
Large Biotech

Items Purchased:

- *Lab Equipment Maintenance*
- *Bulk Lab Gases*
- *Hazardous Waste Management*

Consortium Spend	$2,469,544
Est. Savings Off List Price	$ 789,968
+ Year-End Rebate	$ 21,768
- MassBio Annual Dues	$ 30,800
Bottom Line Savings	**$ 780,936**

MassBio positions itself as a thought leader around drug pricing

VALUE OF HEALTH: from Page 1

for episodes of pulmonary exacerbations can cost more than $100,000 and a liver or lung transplant can cost upwards of $800,000, if a patient is lucky enough to find a donor.

"Current therapies, as helpful as they are, are really just band-aids," said Coughlin, pointing out that 95 percent of rare diseases do not have any FDA-approved treatments. "Our healthcare system is broken. It's 'sick' care, not healthcare."

Coughlin is encouraged by the promise of new treatments, such as the one that is in the pipeline for his son's specific genetic mutation, but worries that payers may hinder medical progress.

"There's a new wave of breakthrough drugs that hold so much promise," he said. "These therapies are cures, and if not cures, they can at least change the course of the disease. But even before these treatments are available, some are already claiming they will be too expensive."

He stands behind MassBio's commitment to advocate for the adoption of innovative solutions to pay for these high-value therapies.

"It's frustrating when people boil it down to dollars and cents when it should come down to life or death," said Coughlin. "My son will no longer have an expiration date. As a parent, you can't put a price tag on that."

He also reminds us that even if a drug is considered an expensive treatment when it is launched, it will most likely go generic, resulting in a dramatic price drop. And it will prevent high-priced transplants and other "band-aids" along the way.

"The CF one has been a breakthrough for my son," said Coughlin. "It will save his life, but it will also save the system money. The way to save costs is by making sick people healthy."

Coughlin says it is the industry's responsibility to reinvent the healthcare system so that it can keep up with the science.

"Why have something like gene therapy if we can't use it?" asked Coughlin. "This needs to change now. We're working closely with the payers to fix the problem, but we need everyone."

CRO/CMO Symposium focuses on connecting buyers and sellers, accelerating drug development

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navigate those extra risks we're taking on as we're accelerating?"

Slatkavitz would encourage companies to set up expectations up front, defining quality and timelines. "In this business we're in, whether it's breakthrough, fast-track or standard, variably you're going to have a problem," said Slatkavitz. "It's to be expected. The key is how you deal with those problems. Make sure the trust is in place to work together and resolve it."

The event closed with a panel titled "Voice of the Customer." Panelists discussed circumstances when customers should push back on outside expertise, and when should they follow their advice. Akash Jain, Director of Program Leadership at Spero Therapeutics; Padma Narayan, Director of Formulation Development and Tech Ops at SAGE Therapeutics; Daniel Couto, Chief Technical Officer at Vedanta; and Trish Hunter, Senior Vice President, Pharmaceutical and Preclinical Sciences at Vertex offered their thoughts on what makes a successful partnership.

"Help involve your vendor partner with prioritization of activities," said Narayan. "Especially when you're in research and development, there are a lot of serendipitous things that can occur, so it's important to have a common understanding, good project management and prioritization of items that are key for the business to achieve milestones. You also have to involve your partners in understanding why those milestones are important so they can have an infrastructure that works with you."

Moderator Colin Minchom, Trish Hunter of Vertex, Daniel Couto of Vendanta and Padma Narayan or SAGE Therapeutics discuss the voice of the customer.

Left, attendees engage the panelists with further questions.

Akash Jain of Spero Therapeutics also speaks on the voice of the customer.

PHOTOS: DAVID FOX

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THE WIN-WIN OF OUTSOURCING

CRO/CMO Symposium focuses on connecting buyers and sellers

By Meaghan Casey

As outsourcing research and manufacturing continues to be the answer to shortening time to market, it's no wonder MassBio's seventh annual CRO/CMO Symposium drew more than 300 attendees this fall.

Through a series of panel discussions and breakout sessions, attendees evaluated the strategies that have evolved in outsourcing and strategic partnerships in pre-clinical, clinical and manufacturing, and commercialization. The Symposium, taking place on Sept. 6 at the Hynes Convention Center, was held in conjunction with Biotech Week Boston — a unique festival of events that included scientific and business conferences, training courses, structured partnering, keynotes, CEO meetings and more.

Neal McCarthy, Managing Director of Fairmount Partners, and Syed T. Husain, Chief Commercial Officer of Alcami, opened the event with a focus on why CROs and CMOs are merging and how they are mitigating the impact on the customer. McCarthy gave the example of how Waltham-based Thermo Fisher Scientific last year acquired Amsterdam-based Patheon, a leading contract development and manufacturing organization, while Lonza, a Swiss biotech company, acquired Capsugel, a Morristown, N.J. company that manufactures and sells drug capsules. Both expect revenue synergies out of the mergers.

"They both say 'it allows us to give our customers a continuum of solutions,'" said McCarthy. "What it means is I can sell more of my stuff to his customers and vice versa."

Thermo Fisher has estimated $90 million in savings a year, while Lonza announced it would eliminate $45 million in costs. Combining cost savings with increased sales, both acquisitions have been accretive to the companies' earnings.

Husain directed the conversation to the importance of integration once an acquisition takes place.

"The reason why that is important is because — and I'll go back to why the MNA was done, mainly to add scale, technology or more offerings to the marketplace — the only way a customer can feel that, embrace that and actually gain positive attributes from that is if the newly formed company is connected and integrated," said Husain. "In order for the client experience to be successful and enhanced, they shouldn't have to deal with any of the background noise of the newly formed MNA… In that first big splash, companies need to tell the market how they will be positioning themselves."

PHOTO: DAVID FOX

Moderator Sarah Kitchell of McDermott Will & Emery, Neal McCarthy of Fairmount Partners and Syed Husain of Alcami discuss the impact of CRO/CMO mergers.

The Symposium's roundtable discussions touched on a variety of topics, from overseas considerations to the challenges of outsourcing clinical trials. Participants weighed the benefits and risks of outsourcing internationally, offered best practices on how to vet a foreign CRO or CMO, and addressed critical considerations such as shipping delays, dealing with customs, and how to ensure a drug has the necessary protections in transit. They also discussed how new forms of drugs such as gene therapy, cell therapies and gene editing show incredible promise for treating or even curing certain diseases, but come with a unique set of challenges when it comes to manufacturing.

Kevin Slatkavitz, President and Founder of ThinkQuality, led a discussion on risk management and chemistry, manufacturing and controls (CMC) outsourcing. With more sponsor companies now receiving FDA fast-track, orphan drug and/or breakthrough therapy designations, there are questions on whether or not they are prepared to meet significant CMC development milestones at warp-speed.

> **"The only way a customer can feel that... is if the newly formed company is CONNECTED and INTEGRATED."**
> **– Syed Husain**

"I'm a firm believer it's never too early to challenge yourself," said Slatkavitz. "How well do we understand our products? How well do we understand our risks? When something is fast-tracked, how do we

See **CRO/CMO** Page 7