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MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

Cancer survivors Michele Rhee, Dan Schorr and Kelley Tuthill share their personal stories at the summit.
PHOTOS: SEAN BROWNE

#PATIENTDRIVEN®

MassBio’s third annual Patient Advocacy Summit brought industry leaders together with patient advocates and other stakeholders to examine ways in which life sciences companies can more fully incorporate the patient voice into the work they do. **Pages 4-5**

MA DRUG DEVELOPMENT PIPELINE, CLINICAL TRIAL CANDIDATES GROW

The number of drug candidates in clinical trials from Massachusetts-headquartered companies increased by 14 percent from 2014, and the total number of drug candidates increased to 1,645, a 10-percent increase, according to an annual industry report published by MassBio.

Massachusetts employees are currently researching and developing products for patients with 362 different medical indications. Oncology drugs make up 36 percent of that pipeline with systemic anti-infectives, central nervous system, and musculoskeletal therapeutic areas as other strong areas of research. The state’s drug development pipeline includes 13 candidates that are pending FDA approval.

The 2016 MassBio Industry Snapshot shows that in the last 10 years, Massachusetts biopharma manufacturing employment has grown by 34 percent to 10,616 jobs statewide. In the same time period, the U.S. lost 24,000 biopharma manufacturing jobs, a 7.9-percent decrease. Massachusetts biomanufacturing employment grew by 6.3 percent, outpacing other industry subsectors in 2015, including research and development.

“Massachusetts continues to shine in research and development, and this year we are also proud to see marked growth in biomanufacturing,” said Robert K. Coughlin, President & CEO of MassBio. “MassBio has been working with our partners in government and academia to cultivate an ecosystem that supports biomanufacturing, so we are pleased to see those efforts paying off.”

Massachusetts continues to lead the nation in biotechnology R&D jobs with 31,469 positions in 2015. Total employment in the biopharma industry in the state rose to 63,026 in 2015—approximately 2,570 jobs more than 2014, based on data from the U.S. Bureau of Labor Statistics’ Quarterly Census of Employment and Wages.

See **SNAPSHOT** Page 6

PUTTING PATIENTS, TALENT PIPELINE FIRST

ROBERT K. COUGHLIN

It's been a whirlwind of a start to the fall as we hosted our 22nd Annual Golf Classic, our third annual Patient Advocacy Summit and our first gender diversity summit, "Finding Our Next Generation Leaders."

Thank you to all who participated in these events. The golf tournament again helped us to raise critical funds for MassBioEd and its support of biotechnology education in Massachusetts public schools. The gender summit served as an important launching pad for our Gender Diversity Initiative. This industry is losing its female talent, particularly in the boardroom and at the executive levels, and it's time to change. It's time to build and sustain a more diverse pipeline at all levels. We are committed to this and excited to roll out workshops, training programs, mentoring programs, female CEO profile series and more in the next few months.

Our Patient Advocacy Summit reminded us why we are all in this industry. It was so inspirational to hear directly from the patients about what this industry is doing to help them—and what we can do to better help them. I'm pleased to report that #PATIENTDRIVEN® is officially trademarked by MassBio. Think of that phrase daily, and remember that we are driven by our patients to find cures and save lives.

In this edition, you'll read more about one patient who is still waiting for his cure. Duchenne muscular dystrophy has received much attention lately thanks to the approval of the drug EXONDYS 51, developed by one of our member companies, Sarepta Therapeutics. This was an exciting milestone, but it's not the end—especially for patients like Aiden. It's important to remember that with every step forward, there are still more steps to go, and patients are waiting for us.

What's promising, as you'll see in the snapshot of the industry, is that the number of drug candidates in clinical trials from Massachusetts-headquartered companies increased by 14 percent from 2014, and the total number of drug candidates increased by 10 percent. More candidates moving forward means more potential treatments for our patients in the near future.

Looking ahead, we will be hosting our CRO/CMO Symposium and MassBioEd will be hosting a reception honoring EMD Serono, Mike Bonney and Brockton High School science department head Jonathan Shapiro as champions of biotech education.

Robert K. Coughlin is President & CEO of MassBio.

MassBio news

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'FORE' A GOOD CAUSE

Peter Abair of MassBioEd (second from right) visits a golf foursome of special guests: former New England Patriots Ross Hochstein (left) and Joe Andruzzi (right), Jim Boushell of ProMedDx and Stephen Lyle of KEW Group (second and third from left).

MassBio hosted its 22nd Annual Golf Classic to benefit the MassBioEd Foundation on Sept. 9 at Pinehills Golf Club in Plymouth. Funds raised through this charity event support MassBioEd, which engages teachers, inspires students and guides the life sciences workforce.

MassBioEd's BioTeach program supports more than 200 public schools in Massachusetts. In June, MassBioEd announced grant funding awards for eight schools: Agawam High School, Hampshire Regional High School in Westhampton, Holyoke High School, Monson High School, North Attleborough High School, Shepherd Hill Regional High School in Dudley, Tewksbury Memorial High School and Watertown High School. MassBioEd will grant these schools a total of $80,000 to purchase lab equipment and consumables that support life sciences-related laboratory activities in the 2016-2017 school year. As a key part of the program, teachers chosen to receive grant funds will also receive intensive, lab-based training and one-on-one mentoring.

Learn more at www.MassBioEd.org.

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Verill Dana
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From left, behavioral economist Iris Bohnet delivers the keynote speech; Maria Nieradka of Biogen engages with panel members; panelists Susan O'Connor, Amri Johnson and Colleen Wilson discuss the life cycle of leadership development.

CLOSING THE GENDER GAP

MassBio takes steps to champion a diverse leadership pipeline

By Meaghan Casey

The life sciences industry is known for its groundbreaking innovation and forward-thinking science, yet when it comes to gender diversity, there is still a lot of work to do. According to a study by Liftstream, women make up only 20 percent of the leadership teams in this industry, and only 7 percent of CEOs are female. One in 10 of the industry's board members are female, and less than 4 percent of boards have chairwomen.

That's why MassBio launched its Gender Diversity Initiative at its first gender diversity summit, "Finding Our Next Generation Leaders," held at Biogen in Cambridge on Sept. 16. The initiative aims to make an impact in three areas: growing women's participation on corporate boards; expanding the number of women in the C-Suite; and shoring up and building the pipeline of diverse candidates.

"We know that a robust and diverse pipeline will be crucial to maintaining the Massachusetts life sciences industry's growth trajectory," said Sarah MacDonald, Executive Vice President at MassBio.

"I find it incredible that we only tap into a percentage of the workforce who can perform at the top level," said Abbie Celniker, former President and CEO of Eleven Biotherapeutics, Chair of the MassBio Board and co-chair of the MassBio Gender Diversity Initiative. "We need to take the risks that will move the right number of people forward, into the C-Suite."

"Personally, I've had a great journey, but I want a better environment for my two girls," said Jodie Morrison, President and CEO of Tokai Pharmaceuticals and co-chair of the MassBio Gender Diversity Initiative. "At the end of the day, it's not men versus women; it's about talent development. Massachusetts has been a leader in this industry and this is our time to be a leader in this area."

During the summit, Liftstream CEO Karl Simpson presented early findings of the survey data collected by MassBio and Liftstream, which will be used to set a baseline and track improvements.

"We want to determine at what point women are dropping out of the pipeline and use the data to drive behavior and solve the problem," said Simpson, noting that of the 275 survey takers, 45 percent of women feel they have less opportunity for professional growth than men and none feel they have more opportunity. "In start-ups, there's a feeling of larger opportunity, but that tapers off in larger companies."

The data also shows women weigh factors like flexbile working schedules and locations, as well as the gender makeup of a leadership team when considering a new job or promotion. They are also twice as likely as men to value the culture of a work environment.

"Organizations that have not addressed these factors are at a disadvantage for attracting and attaining female talent," Simpson said.

Keynote speaker Iris Bohnet, a behavioral economist and professor of public policy at Harvard's Kennedy School, delved into the issue of inherent biases. She told the story of Heidi Roizen, a successful Silicon Valley venture capitalist who became the subject of a case study at Columbia Business School. Half of the students in the class were presented the case study with Heidi's name on it and the other half received the same case study with her name changed to "Howard." The students—both men and women—not only liked "Howard" better than Heidi, but they reported they learned more from him.

"Heidi defies the norms of what a venture capitalist looks like or what a woman does," said Bohnet. "This is not about pointing fingers; we're all biased and see patterns in the world. We don't see men as nurses or women as engineers. But how can we liberate our minds and not put individuals in boxes?"

Bohnet encouraged the leaders in attendance to look more closely at the language in their job postings, to implement blind or structured evaluations, to eliminate self-evaluations and to look for a presence of diversified role models—even as reflected by the portraits hanging in offices.

During a discussion on recruiting and retaining a diverse talent pipeline and leadership pool, Amri Johnson, Head of Diversity and Inclusion at Novartis Institutes for BioMedical Research, talked about the company's mentoring program, now in its 10th year. "The program pairs senior people with emerging talent, so everyone becomes accountable for people thriving," he said.

In another panel on putting inclusion theory into practice, Samantha Singer, Chief Operating Officer at the Broad Institute, talked about Women@Broad, a group created to inspire, connect and empower the institute's female employees and champion strong, conscientious leaders.

"A group of women created this and it's grown to include training and an annual symposium," said Singer. She also mentioned the Broad's investment in caregiver support: "That was a big investment last year and we wanted to bend the curve, allowing women to stay in the workplace—first with young children, but we're expanding that to aging parents as well."

When discussing why companies should implement some of these more flexible changes, Singer and Aida Sabo, Vice President of Diversity at PAREXEL, summed it up clearly: "Diversity is a fact, and inclusion is a choice," said Sabo. "The best and the brightest want to be in a place where they are valued and respected."

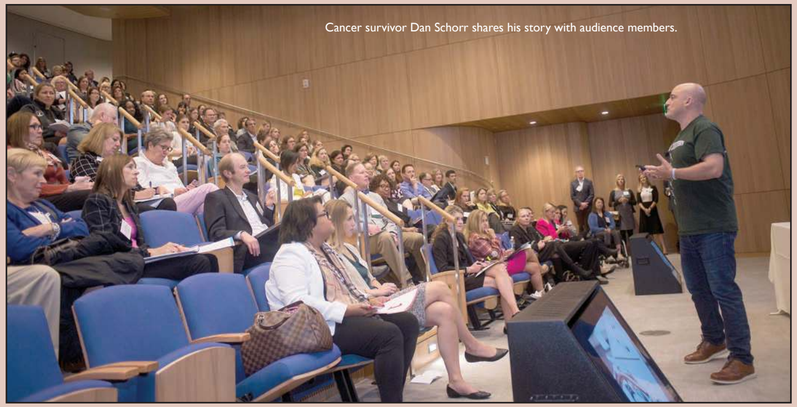
"We can make the talent argument and the economic arguments, but it's also just the right thing to do," said Singer.

Moving forward, MassBio—guided by an Advisory Committee of industry leaders—will launch a series of programs, partnerships and challenges focused on giving companies and stakeholders the tools necessary to build diverse organizations.

"This is going to be a sustained effort by MassBio," said Robert K. Coughlin, MassBio President & CEO. "We're Massachusetts. Have we changed social policy and health policy? Yes. We can do this."

ANNUAL SUMMIT AIMS TO BUILD A PATIENT-CENTERED ECOSYSTEM

From left, audience members engage the panelists; guests enjoy Beyond the Diagnosis, an art exhibit from the Rare Disease United Foundation; MassBio President & CEO Robert K. Coughlin joins Paul Giusti and Dr. Kenneth Anderson for a fireside chat; Michelle Dillione, Tracy Seckler and Kara Malcolm discuss the rules and tools of patient input; author and neuroscientist Lisa Genova delivers the keynote speech; Jan Nielsen, Kenneth Hobby, Gillian Mullins and Wendy Erler discuss taking therapies into the clinic.



Cancer survivor Dan Schorr shares his story with audience members.

By Meaghan Casey

Before Lisa Genova became the New York Times bestselling author of "Inside the O'Briens," "Love Anthony," "Left Neglected" and "Still Alice," she was first and foremost a scientist—a neuroscientist, to be exact.

"I used to spend my time in a lab with rats and DNA," said Genova, who earned her Ph.D. in neuroscience from Harvard University in 1998. When her grandmother was diagnosed with Alzheimer's disease, she took it upon herself to learn more.

"That was the seed for 'Still Alice,'" said Genova. "I did my homework, and that involved getting to know 27 people with the disease. I was in touch with them every day for the year and a half that I was writing the book. These are inspiring, heroic people. Meeting them, I was able to breathe three-dimensional life into the subject. What is it like to live as a human being with this disease?"

Throughout the process of writing, Genova began thinking of herself not only as a neuroscientist and a novelist, but as an advocate.

"I realized I had an opportunity and a responsibility to give these folks a face and a voice," Genova said. "We often think of Alzheimer's as a disease of the elderly living in nursing homes, and we tend to ignore it, but we're ignoring millions of people who are suffering. It was important to me to drag these issues out of the closet and into people's living rooms and movie theaters, where you can begin to imagine the full human experience." She would encourage anyone in the life sciences industry to take the similar approach in building relationships with patients—to see, hear and feel what it's like to live as one.

"Give this to yourself. I guarantee you, it will change your life and change the way you get up in the morning," she said. "I learned more about how to live from people with life-threatening illnesses. We sometimes forget that we're mortal and that we might not have tomorrow. To make the connection with your patients as human beings, it will plug you in and ignite you."

Genova served as the keynote speaker at MassBio's Patient Advocacy Summit, held on Sept. 28 at Novartis in Cambridge. The third annual event brought industry leaders together with patient advocates and other stakeholders to examine ways in which 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.

Panelists discussed patient advocacy beyond U.S. borders, the rules and tools of patient input, start-up best practices in patient advocacy and taking therapies into the clinic.

"When we got into this space it was about our son," said Annie Ganot, Head of Patient Advocacy at Solid Biosciences. "Now I feel like I'm the mother of 300,000 sons."

Ganot's husband, Ilan, left his job in finance to start the company after their son was diagnosed in 2013 with Duchenne muscular dystrophy. As parents, they understand the urgency to not only develop drugs more quickly, but to try to improve the lives of Duchenne patients in the meantime.

"These families are smart; they're savvy," said Ganot, who advocates for maintaining a strong connection to the patients and their caretakers. "They're living with the disease."

Tracy Seckler, another Duchenne mother and the director of Charley's Fund, spoke about the importance of sharing real-world evidence with the medical community.

"What I'm seeing at home on a daily basis is more valuable as data than what I'm seeing at the doctor's office when Charley is trying his hardest to get up off the floor as quickly as he can," said Seckler.

Fellow panelist Michelle Dillione, Senior Director of Commercial Counsel at Alkermes, concurred with Seckler's perspective.

"We need to be humble enough, as pharma companies, to acknowledge we don't have all the answers," said Dillione. "We need the insight of the patients."

"Where I see manufacturers missing the boat is by not hearing patient stories or understanding beyond commercialization," said Jan Nielsen, Division President of Sonexus Health, a Cardinal Health Specialty Solutions company. "There needs to be continued education with patient groups from beginning to end."

Gillian Mullins, Director of Alliance Development at Biogen, agreed. "I've seen drugs fail because the wrap-around services weren't set up appropriately," she said.

Throughout the event, patients and survivors such as Michele Rhee, now working in patient advocacy at Takeda Oncology, shared their own personal stories.

"Having cancer saved my life," said Rhee, who was diagnosed with a rare heart tumor less than a year after going into remission from cancer. While performing scans, doctors caught pieces of the tumor that had broken off and lodged in her lungs.

"I had hated cancer," she said. "It stole my time and my youth, but without it, I would have gone undiagnosed. That disease would have killed me before cancer ever did."

Dan Schorr, also a cancer survivor, faced his situation with a positive attitude and sense of humor. In 2014, he was diagnosed with an aggressive form of lymphoma on the same day that his wife announced she was pregnant with their first child. He started a blog—Humor with Tumor—to help himself and others get through the battle. Four months after going into remission, he welcomed his daughter into the world.

Kelley Tuthill, who, after 18 years as a WCVB newscaster, now serves as Vice President of Public Relations and Communications at Regis College, spoke about being diagnosed with breast cancer in 2006 at age 36. Her tumor, which was HER2-positive, was aggressive. It was successfully treated with a targeted therapy known as Herceptin, which was approved by the FDA in 1998—just eight years before Tuthill would need it.

"Timing, when it comes to cancer, is everything," said Tuthill. "I didn't realize how lucky I was at first to have access to Herceptin. Thanks to it, my life is fuller and happier 10 years later."

The summit also featured case studies—one of which looked at how four mothers came together to found the Food Allergy Science Initiative (FASI), raise $10 million and partner with the Broad Institute to officially launch in June. Justine Levin-Allerhand, Chief Development and External Relations Officer at the Broad Institute, joined Lesley Solomon, parent and co-founder of FASI, to talk about the need for the initiative and to outline the next steps of the scientific plan.

"Right now, there's no preventive treatment or cure," said Levin-Allerhand. "People are told to avoid the food and carry an EpiPen. That can't be the solution."

Over the next five years, they hope to define the cellular landscape of the gut, uncover mechanisms of allergen sensing, determine immune responses to allergens, evaluate microbiota and establish clinical trials that translate into therapy.

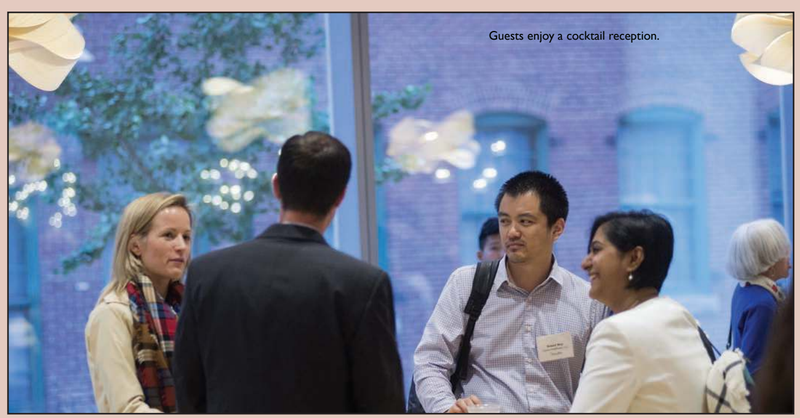
"While we're focused on food allergies, I think we're going to learn a lot about the immune system through this," said Solomon.

A second case study focused on Moving Mountains for Multiple Myeloma—a collaboration between CURE Media Group, Takeda Oncology and the Multiple Myeloma Research Foundation to raise awareness and funds for myeloma research. Since January, myeloma patients, caregivers, doctors, nurses, researchers and loved ones have been taking on challenging mountains like Mount Kilimanjaro, the Grand Canyon and Peru's Machu Picchu to demonstrate that the advancements being made in recent years are helping patients live longer with a higher quality of life than ever before.

"It's about raising money and global awareness, but it's also about hope and inspiration," said Marty Murphy, Director of Patient Education at CURE magazine. "We have the privilege of being with these patients and hearing their amazing stories that we're not going to get sitting around a boardroom table."

At the conclusion of the event, MassBio President & CEO Robert K. Coughlin joined Paul Giusti, President & CEO of the Multiple Myeloma Research Foundation, and Dr. Kenneth Anderson, Program Director of the Jerome Lipper Multiple Myeloma Center & LeBow Institute for Myeloma Therapeutics at Dana-Farber Cancer Institute, for a conversation about patients and policy. They discussed the progress for myeloma: 18 new treatments in the last 12 years and the launch of the CoMMpass study (Relating Clinical Outcomes in MM to Personal Assessment of Genetic Profile), which is following 1,000 patients for eight years.

"It's the gold standard in terms of data," said Giusti. "The future is all about having that information available to translate. This is how we're going to start solving it. This is how we're going to let the researchers do their work. There's a lot of reason for hope, but clearly our job isn't done yet. Less than 5 percent of cancer patients are in clinical trials. There's still so much to learn."



Guests enjoy a cocktail reception.

Attendees remain engaged throughout the day-long event.

MASSACHUSETTS DRUG DEVELOPMENT PIPELINE, CLINICAL TRIAL CANDIDATES GROW

SNAPSHOT: from Page 1

As a result of the growth in combination products and the continued blurring of the lines between drugs, diagnostics and devices, MassBio has also started to track the performance of the medical device segment of the life sciences industry. Data show that medical device employment is down by 6.9 percent over the last 10 years, which puts Massachusetts in the middle of the pack of states with the largest number of medical device jobs.

The report also highlights that venture investment in Massachusetts rose to a record $2.1 billion in 2015, with Massachusetts receiving 28 percent of all VC dollars in biotech in the U.S.

This year, MassBio analyzed the locations of lead venture capital funding and found that Massachusetts-based VCs invested over $596 million into Massachusetts biotechs, representing 25 percent of the total investment. Massachusetts-based VCs provided 79 percent of the total seed capital and 49 percent of Series A funding for Massachusetts biotechs in 2015. This shows that the local venture community is playing a disproportionate—and critical—role in funding seed and early stage ventures.

Last year, MassBio took a look at slowed growth in the area of seed-stage funding for Massachusetts companies. In 2015, and so far in 2016, there has been a turnaround in seed-stage investing, which is a positive indicator of the industry's long-term growth potential.

This year's snapshot also includes the MassBioEd Foundation's job demand forecast which predicts a total increase in employment from May 2015 to May 2018 to be 4,325 new jobs, a 6.7-percent increase. MassBioEd conducts quarterly job trends research that can be accessed at www.MassBioEd.org.

Additional highlights from the report include:

• Massachusetts accounts for 13 percent of the U.S.-based drug development pipeline. Massachusetts-headquartered companies account for 5.9 percent of the global pipeline.

• There were 7 Massachusetts biotech IPOs in the first half of 2016.

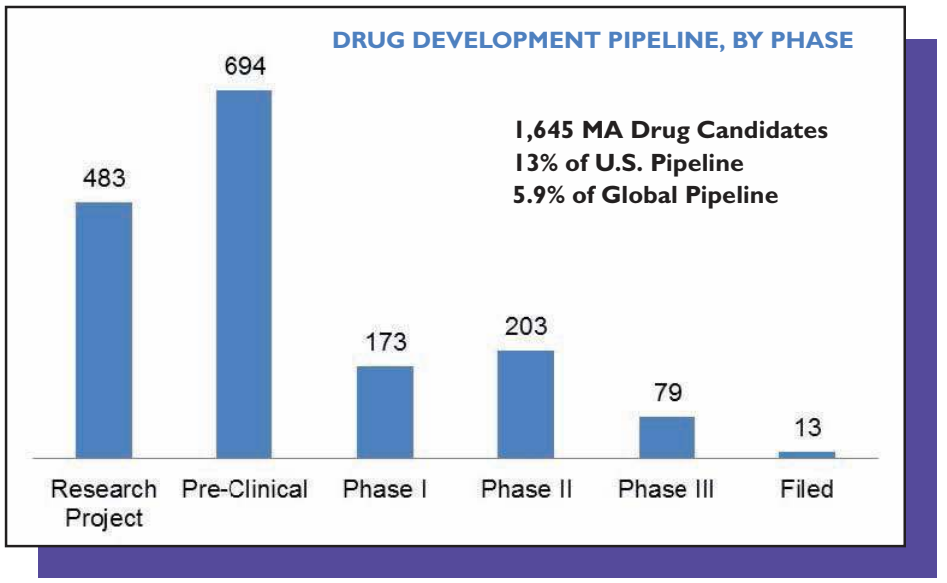
• The top 4 NIH-funded independent hospitals (and 7 of the top 15) in the U.S. in 2015 are in Boston.

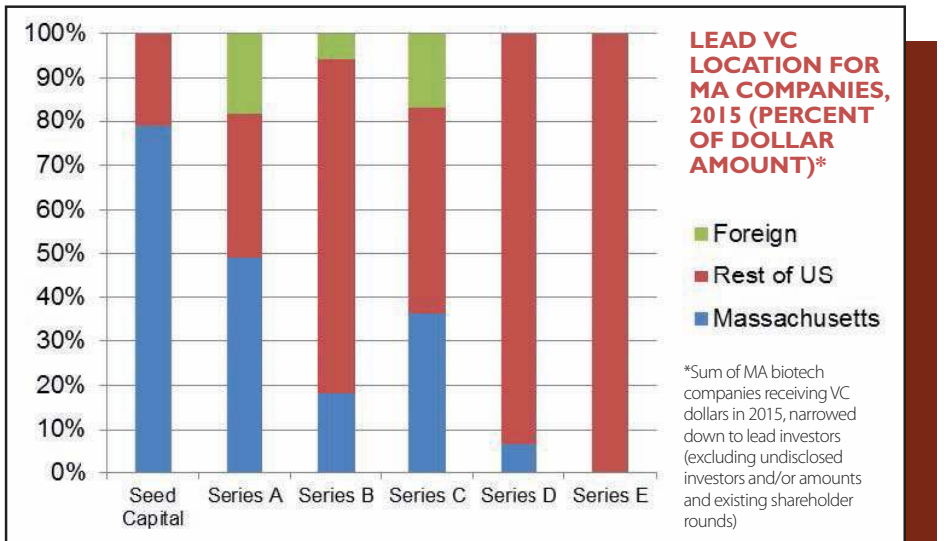
• On an NIH-funding per capita basis, Massachusetts continues to far exceed other leading NIH-recipient states. Only California receives more total NIH funding.

• Since 2007, 9 million square feet of commercial lab space has been added to inventory in Massachusetts.

• Massachusetts-headquartered companies have developed therapies that focus on patient populations of more than 250 million in the U.S. and more than 1.8 billion around the world.

This year's Snapshot was produced in partnership with EvaluatePharma®, the premier source for commercial analysis of the pharma and biotech sector. Snapshot statistics are compiled annually by MassBio from sources including EvaluatePharma, National Institutes of Health, the U.S. Bureau of Labor Statistics, the Quarterly Census of Employment & Wages and others.





MASSACHUSETTS TOPS IN BIOTECH SEED FUNDING

By Jonathan Gardner
Deputy News Editor, EP Vantage

The migration of venture capital from early to late stage funding rounds has been well documented, but a surprising finding from the MassBio 2016 Industry Snapshot has been how Massachusetts-based financiers have supplied much of the sector's seed funding.

Seed capital is by its nature a small part of the venture financing scene, but EP Vantage has shown how backing for the youngest of biotechs has stagnated while their more mature brethren have flourished. In 2012, $190 million went to seed capital rounds, an amount that slid to $123 million in 2015, representing just 1 percent of all VC funding; a rush to series B rounds has also been noted, with the $3.1 billion in investment accounting for nearly one-third of fundraising.

The MassBio report reveals that 79 percent of that 2015 seed funding came from Massachusetts-based VCs, and for the growing early-stage companies those same Massachusetts VCs accounted for 49 percent of series As. This is a sign that they are bucking the trend and placing big bets on innovation.

Cambridge-based Flagship Ventures accounted for a substantial amount of the 2015 seed funding when it invested $60 million in two early stage companies. Those were Evelo Therapeutics, recipient of $35 million to advance an immunotherapy platform, and Rubius Therapeutics, which will have $25 million to advance a technology that aims to re-engineer red blood cells to express biotherapeutic proteins.

These two companies emerged from Flagship's VentureLabs incubator, which has nurtured such companies as Moderna, Eleven Biotherapeutics and Seres Therapeutics.

It will take some doing for seed funding in 2016 to match 2015's total. So far, $31 million worth of investment has been announced. But one or two rounds could put 2016 on a more even footing.

Learn more at www.evaluate.com.

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	Total Investment ($m)				
Financing Round	**2015**	**2014**	**2013**	**2012**	**2011**
Seed Capital	123	135	135	190	140
Series A	1,858	1,466	1,341	1,185	1,270
Series B	3,088	2,430	1,034	1,092	1,047
Series C	1,567	948	900	794	510
Series D	1,192	722	447	673	362
Series E	546	233	489	195	404
Series F	148	67	205	140	49
Series G	49	128	62	210	127
Series H	-	208	-	-	-
Series I	2	93	-	-	-
Series Undisclosed	1,150	632	330	272	431
Total	**9,723**	**7,063**	**4,945**	**4,751**	**4,341**

POLICY RECAP: 2015-2016 SESSION

The Massachusetts Legislature discussed and voted on some issues critical to the life sciences industry in the 2015-2016 session, which drew to a close on July 31. MassBio, working alongside and on behalf of its member organizations, engaged legislators throughout the session, educating and advocating for policies that support innovation and the continued growth of the industry. Below is a recap of what turned out to be a successful session.

"TRANSPARENCY" LEGISLATION

This legislation would have required manufacturers to disclose information related to the price of drugs, including marketing and research costs, and was before the Committee on Healthcare Financing for consideration this session. It would have also allowed the Massachusetts Health Policy Commission (HPC) to cap drug prices. This legislation was sent to study.

CO-PAY ASSISTANCE - REPEAL OF THE SUNSET PROVISION EXTENDED UNTIL 2019

The language in Section 129 of the FY 2016 Budget, which was signed by the Governor, extends the sunset date for co-pay assistance programs and discount programs to 2019 and will allow companies to provide patients access to the innovative therapies that improve their lives.

COST TRENDS ACCURACY

The Health Policy Commission's (HPC) 2015 Cost Trends Report, based on data analysis from the Center for Health Information Analysis (CHIA), suggested that there was a considerable increase in overall healthcare costs between 2013 and 2014, including pharmaceutical costs. Upon further analysis, it was determined that the data CHIA shared with HPC was flawed due to the lack of accounting for vital information bearing on true drug spend, including the impact of rebates and discounts on pharmaceuticals. The language in Section 11 of the FY 2016 Budget, which was signed by the Governor, would allow HPC and CHIA to remedy the situation and provide more accurate pharmaceutical cost findings that are more appropriate for driving positive policy initiatives related to healthcare cost containment.

NON-COMPETE

The Massachusetts House of Representatives and the Massachusetts Senate passed different versions of non-compete legislation this session. The House version of the bill limited non-compete agreements to a year and provided for "garden leave" provisions where employees would be paid half their salaries or "other mutually-agreed upon consideration." In the Senate bill, non-compete agreements would last only three months and would include full salary to the departing worker. These bills were sent to a House/Senate Conference Committee, however the Committee was unable to reach a consensus on this legislation. This legislation died in conference.

For more details, visit www.MassBio.org.

AN ANSWER FOR AIDEN?

PATIENT: from Page 8

organization's Collaborative Trajectory Analysis Project (cTAP), which is optimizing the use of data from clinical trials, patient registries and other sources. Because Duchenne progresses at very different rates in different patients, it is much more difficult to evaluate trial data. By working together, applying novel tools and sharing data, cTAP plans to create a new paradigm that will improve understanding of this disease, advance research and give patients access to a wider range of promising treatments.

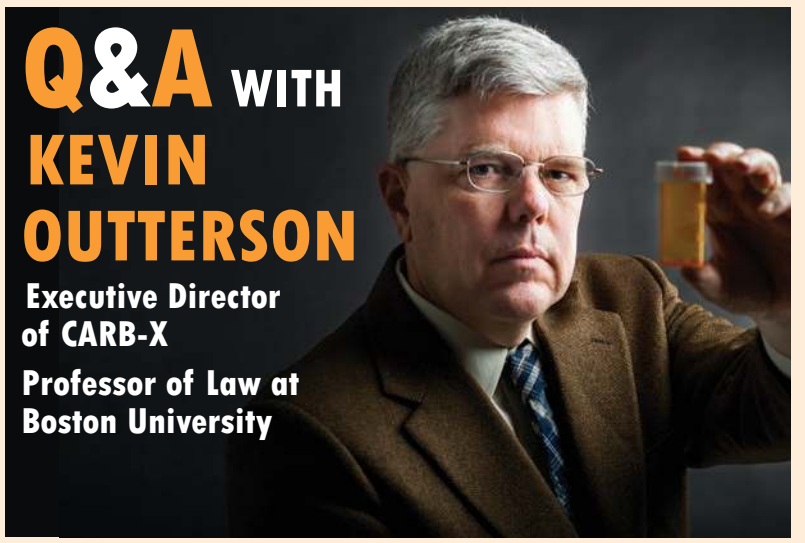
"Since its inception, CureDuchenne has done a remarkable job finding and funding early-stage projects in Duchenne," said Knowles. "The focus of Cure Duchenne Ventures is to de-risk promising research from academic labs and help translate the basic science into therapies that can attract capital from larger investors."

CureDuchenne is also launching a Virtual Durable Medical Equipment Expo, an interactive education and networking website to provide Duchenne caregivers and family members with up-to-date, unbiased information, as well as reviews on a wide range of mobility equipment to help patients remain mobile and active longer.

"There are many aspects to the disorder and CureDuchenne understands that," said Moore. "They have made a commitment to treatment and to bring options to all of our boys who suffer. Their entire team is dedicated to their vision to find a cure."

Aiden and Jillian Moore are thankful for the support of CureDuchenne, founded by Paul and Debra Miller, at right, with their son, Hawken.

Q&A WITH KEVIN OUTTERSON



Executive Director of CARB-X

Professor of Law at Boston University

Q What is CARB-X?

CARB-X is a new global public-private partnership for pre-clinical antibacterial research, with research funds for the first five years exceeding $350 million. The entity gets its name from the U.S. government's Combating Antibiotic Resistant Bacteria (CARB) initiative, and will directly address several key goals in the 2015 U.S. CARB National Action Plan. CARB-X aims to 'accelerate' a diverse portfolio of more than 20 high-quality antibacterial products towards entry into human testing. Key funders include the U.S. government (BARDA and NIAID), the Wellcome Trust of London, and the AMR Centre of Alderley Park, Cheshire in the United Kingdom.

We also partner with the two most innovative life science hubs in the world – Boston and San Francisco – where MassBio and California Life Sciences Institute run renowned programs to mentor and support life science start ups. CARB-X is also starting an innovative cross-disciplinary effort at the Broad Institute where we are founding a new Collaborative Hub for Early Antibiotic Discovery. Boston University was able to organize these great partners into CARB-X and serves as the leader for the project, with support from RTI, a non-profit research support firm headquartered in the Research Triangle Park in North Carolina.

Q Why do we need CARB-X for new antibiotics?

We urgently need a diverse and vibrant pipeline of new antibacterial products to prevent and treat drug-resistant bacterial infections. The number of pharmaceutical companies developing new antimicrobial therapies has decreased over the past few decades. As a result, there has been a significant innovation gap in antimicrobial product development. Companies increasingly cannot justify that the development of new antimicrobials is sufficiently profitable to warrant the substantial R&D investment required.

We're trying to build a fire station before the buildings catch on fire. We're looking for game-changing products that will make dramatic improvements in human health—not incremental change. We're going to spend this money on the areas of greatest health need, focusing on things that major pharmaceutical companies have abandoned.

Q How will CARB-X address these problems?

Drug-resistant infections pose a serious threat to global health, and international cooperation and coordination is key to addressing the problem. Therefore, by collaboratively selecting and investing in development of antibiotics, CARB-X can reduce the business risk inherent in drug development and, in so doing, incentivize companies to invest in the advanced development to see promising drugs candidates through to regulatory approval.

Q Who is eligible to apply for CARB-X funding?

The bulk of the money will go to small companies developing innovative products all over the world. We will fund the best science, wherever found. The goal is to invest money so that the products society needs will be ready in a decade. This is a social investment.

Learn more at www.CARB-X.org

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PATIENT PROFILE

AN ANSWER FOR AIDEN?

Finding hope where there's not yet a cure

By Meaghan Casey

Sixteen months ago, Waltham resident Jillian Moore received the devastating news that her son, Aiden, would lose his physical capabilities, one by one. Every muscle in his body would gradually deteriorate, potentially leaving him unable to walk, dance, hug, laugh or even smile.

"I felt like everything stopped," said Moore. "Every hope and dream for my son's future was shattered. His smile lights up every room and brings joy and purpose to my life on a daily basis. I can't imagine the world without it."

Aiden was diagnosed with Duchenne muscular dystrophy, a fatal genetic disorder characterized by progressive muscle degeneration and weakness. Children with Duchenne—primarily boys—cannot produce dystrophin, a protein necessary for muscle strength and function. As a result, skeletal muscle deteriorates first and most patients become wheelchair-bound by age 12. In the later stages, heart and respiratory muscles begin to fail, often by the time patients are in their late teens or 20s.

More than 300,000 boys are living with the disease worldwide. There is no cure. But there is hope—and even treatment now for some.

On Sept. 19, the FDA approved the first drug to treat patients with Duchenne. EXONDYS 51TM (eteplirsen), developed by Cambridge-based Sarepta Therapeutics, is specifically indicated for patients who have a confirmed mutation of the dystrophin gene amenable to exon 51 skipping—a subpopulation representing about 13 percent of the boys with Duchenne. Administered as a once-weekly intravenous infusion, EXONDYS 51 was approved under the FDA's accelerated approval program.

"It's just amazing," said Moore. "Aiden's mutation is different, but there was so much weighing on this. It opens the door to a lot of other possibilities and encourages other companies not to lose interest in the Duchenne space."

CureDuchenne, founded in 2003 to save this generation of Duchenne boys, provided early funding to Sarepta for the development of EXONDYS 51 and helped it to move into human trials. The organization also brought together key opinion leaders, physicians, parents and regulators to provide the patient voice about the need to approve eteplirsen.

"The first FDA-approved treatment for Duchenne is a landmark in our fight against this disease," said Debra Miller, Founder and CEO of CureDuchenne. "EXONDYS 51 is a huge step forward in turning Duchenne muscular dystrophy from a fatal disease into a more manageable condition. Boys on eteplirsen have experienced improvements in quality of life that are amazing for a progressive disease that has remained without an approved drug for so long."

Miller and her husband, Paul, founded CureDuchenne after their son, Hawken, was diagnosed with Duchenne. They assembled a seasoned staff and an expert Scientific Advisory Board and applied their professional backgrounds to create a strategically focused business model, making investments in or giving grants to companies and researchers doing promising work. CureDuchenne has raised more than $20 million in the fight against Duchenne and has leveraged more than $1.3 billion in follow-on investments from other foundations, venture capital, biotech, and pharmaceutical companies to fund research leading to a cure.

"For me, action is therapy," said Miller. "We cannot rest until there are therapies—and, ultimately, cures—available to everyone with Duchenne."

Hawken, now 19, is a sophomore at the University of Southern California, studying journalism. Like Aiden, he has a different mutation and will not benefit from EXONDYS 51. There are, however, new gene-based therapies that have recently emerged with noted advances in using conventional gene replacement strategies, as well as RNA-based technology and other pharmacological approaches that hold promise for the treatment of the dystrophic muscle. In fact, CureDuchenne has funded nine research projects that have advanced to human clinical trials.

"I feel better knowing there's more hope than years past," said Moore, who tries to stay positive every day. "This has taught me to be more patient and loving and to enjoy every moment. Aiden knows he can't run as fast as his friends, but he's always smiling or laughing. He's so resilient and so happy. I see him being happy and he makes me happy. He's a little light in the room."

Aiden, now 5 years old, was 3 when he was diagnosed. He has since adopted a regimen of prednisone, vitamins and other supplements to keep his muscles healthy.

"His legs get tired, especially walking up and down stairs or getting out of bed," said Moore. "He can still run and jump, which is awesome. As a parent, you get excited by these milestones, but then one day it starts to decline. You just have to take one day at a time."

Moore has become very involved with CureDuchenne, participating in events that help parents and caregivers better understand and implement Duchenne care guidelines. Additionally, she teamed up with the organization to host a fundraising event in Cambridge, Dealing for Duchenne, on Oct. 20—Aiden's birthday.

"Our families make a huge difference," said Miller. "Jillian knows she can make a difference and we're committed to Aiden—who gives the best hugs in the world—and all of our other boys. These boys are why we do what we do. They're our heroes."

CureDuchenne, which is based in Newport Beach, Calif., just opened a Cambridge office last year. It is led by Dr. Jak Knowles, who came on board as the Managing Director of CureDuchenne Ventures, an initiative to identify and fund a robust pipeline of therapies to treat Duchenne. He also serves as the organization's Vice President of Medical and Scientific Affairs. Knowles is leading the effort to source new Duchenne research projects and is playing an integral role in the

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Duchenne patient Aiden Moore is all smiles despite the challenging road ahead of him.