

MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

POLICYMAKERS CONFRONT VALUE AND THE OPIOID CRISIS AT ANNUAL BREAKFAST

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PHOTO: SEAN BROWNE



PAT QUINN

SUMMIT HELPS BUILD PATIENT-CENTERED BIOTECHS

BY MEAGHAN CASEY

In the summer of 2014, the ALS Ice Bucket Challenge took the world by storm, becoming a global social media phenomenon and raising more than \$220 million in the fight against ALS.

Pat Quinn, who had been diagnosed with the disease in March 2013 and launched the challenge with fellow patient and advocate Pete Frates, never imagined it would take on such a life of its own.

“It’s really amazing, the power of social media.

What resulted from it is the most amazing thing. It created hope among patients who really didn’t have any,” said Quinn, closing out MassBio’s Patient Advocacy Summit on Oct. 27. He was joined at the event by Andrew Frates, Pete’s brother.

“August 2014 was a crazy, crazy month for my family and I like to think of it as the first building block in the end to ALS,” said Andrew, who has become his brother’s caretaker.

Both Quinn and Frates were diagnosed with

ALS before their 30th birthdays, and the pair—hailing from Yonkers, N.Y. and Beverly, Mass. respectively—came together in the hope of spreading greater awareness of the disease. Their campaign elevated ALS to a topic that was discussed in the homes of millions of people. The funds raised are now being used to support ALS care services, public policy initiatives and research, including four collaborative research initiatives to build understanding of the

See **SUMMIT** Page 7

APPROACHING 2016 WITH A PATIENT-DRIVEN MISSION



ROBERT K. COUGHLIN

As we approach the opportunities and challenges ahead of us in 2016, we do so under the guidance of our new mission statement—to advance Massachusetts' leadership in the life sciences to grow the industry, add value to the healthcare system and improve patient lives.

The statement is meant to bring focus to the people at the heart of the work we all do: the patients. We work to bring that mission to life through programming like our second annual Patient Advocacy Summit, a sold-out event that brought industry leaders and patient advocates together to examine ways in which life sciences companies can more fully incorporate the patient voice into the work they do throughout the drug

development cycle.

Patient advocacy continued to be a theme at our recent Policy Leadership Breakfast, where we addressed issues touching the lives of residents across Massachusetts—with particular focus on both drug access and abuse. MassBio has and will continue to convene stakeholders to discuss alignment on drug cost and pricing topics, through conversations and programming at our Annual Meeting and events later in the year. We will continue to work with legislators such as Attorney General Maura Healey, who joined us at the breakfast, and Gov. Charlie Baker, who will be joining us in March for our Annual Meeting, to find solutions.

Also at the policy level—by convening the Biotech Caucus, participating with the Health Policy Commission and serving on the Managed Care Organization Task Force—we hope to ensure the work our member companies are doing is incentivized and rewarded in any evolving healthcare system. It is critical in Massachusetts and beyond that we preserve and foster innovation so patients can receive the cures they so desperately need.

Thank you for your support and I wish you the best as you continue to innovate. We will continue to champion for you.

Robert K. Coughlin is President & CEO of MassBio.



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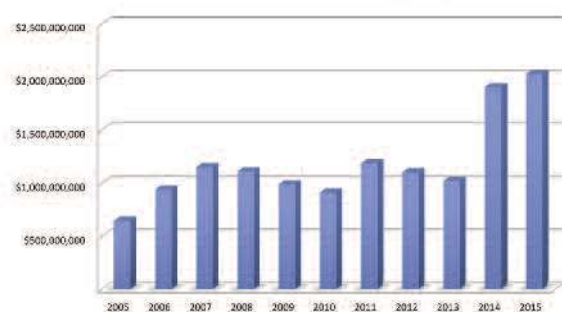
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VENTURE CAPITAL INVESTMENT

Investment in Massachusetts Biotech Companies, 2005-2015



Venture investment in Massachusetts rose to an all-time high of \$2 billion in 2015.



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SYMPOSIUM SHINES A SPOTLIGHT ON THE STRENGTH OF THE CRO AND CMO COMMUNITIES



Shikha Barman, CEO of Integral BioSystems, showcased her company's innovative drug delivery technology, OcuSurf.



MassBio President Robert K. Coughlin connected with Travis McCready, President of the Mass. Life Sciences Center.



Michael Martorelli, Director of Fairmount Partners, delivered the keynote presentation.

Strategic partnerships accelerate drug development and maintain momentum in Massachusetts

BY MEAGHAN CASEY

An increasing number of biopharma companies are turning to contract research and manufacturing partners for time and cost savings, as well as for access to scientific expertise. Worldwide, nearly a third of biopharma research dollars are spent on outsourcing services offered by CROs. That figure was approximately \$27 billion in 2015, and is projected to keep growing. More than \$17 million was spent in the CMO market last year.

At the MassBio CRO/CMO Symposium, held on Nov. 13 at the Renaissance Boston Waterfront Hotel, more than 300 attendees gathered to focus on leveraging strategic partnerships to accelerate drug development and maintain momentum in Massachusetts. The event is an annual one that shines a spotlight on the strength and success of the CRO and CMO communities in the Commonwealth.

"We know that more companies are contracting services out—or remaining virtual—in order to bring new products to market faster and more efficiently, and we know Massachusetts companies can provide the full spectrum of services," said MassBio President & CEO Robert K. Coughlin. "We need to

talk about how to ensure our competitiveness well into the future."

Michael Martorelli, Director of Fairmount Partners, gave a keynote presentation on piloting strategic options and stressed the importance of maintaining focus in terms of nature and range of services, geographic scope, types of prospective clients, desired rate of revenue growth and extent of profitability.

A series of case studies, interactive panel discussions and networking sessions followed, providing ample time to explore the various techniques and approaches being used by both virtual and established companies. Panelists discussed trends in outsourced biopharma and global and local options for outsourcing—stressing that locally grown companies can provide ease of access, operational efficiency and an unmatched level of technical expertise.

"A lot of what we do is in the discovery phase, and time matters," said Jim Jersey, President of Agilux. "It's important to be adaptive and think about the advantages of proximity."

"If you're looking for services that are capacity-oriented vs. capability-oriented, you have different needs," said Richland Tester, Senior Manager at Celgene. "If you just need the space, that's one thing, but if you're selecting a company for what they can do, you're probably going to look more locally. You might have to regularly visit the biologists in the lab to build a closer relationship."

"You want to invest in quality," said Jörg Holenz, Director of Discovery and Preclinical Sciences at AstraZeneca. "Find the niche CRO best suited to your project and leverage that brain power early on."

Also at this year's symposium, the amount of roundtable discussions doubled, with 18 hot topics for attendees to choose from.

In the closing keynote presentation, Willy Shih, professor at Harvard Business School, spoke about the risks and opportunities for the Massachusetts cluster.

"The choices you make today will influence the long-term health of this cluster," said Shih. "The easiest choices might not be the best ones. We've seen this in the electronics industry, the computer industry and the semiconductor industry."

Shih talked about his time at Kodak—which for a long time focused on film and outsourced manufacturing. Then the industry went digital, forcing the company to refocus its priorities back to the camera itself. Shih said the biotechnology industry also might need to refocus.

"There's a huge difference between being able to create a couple milligrams in a lab and a year's worth of supply," said Shih. "We really need to strengthen biomanufacturing in this country."

In an effort to improve and streamline access to local resources, MassBio launched the Massachusetts CRO/CMO Gateway in partnership with OnDeckBiotech. This online portal facilitates direct, real-time access to resources that accelerate development timelines and reduce costs for cluster members. For biotech and drug development companies, the portal combines resources to search for specific capabilities, detailed company profiles and tools to quickly execute confidentiality agreements.

Visit the MA CRO/CMO Gateway at www.MassBio.org.

SAVE THE DATE: MARCH 31- APRIL 1, 2016



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TACKLING THE COMMONWEALTH'S BIGGEST CHALLENGES



From left, Marylou Sudders, Dr. Dennis Dimitri, Michael Heffernan, Richard Pops and Spencer Williamson discussed the opioid crisis.

Attorney General Maura Healey opened the event.



MassBio President & CEO Robert K. Coughlin, center, joined representatives from Sunovion Pharmaceuticals at the Policy Breakfast.

The 2016 Policy Leadership Breakfast addressed the growing opioid addiction and healthcare cost concerns

BY MEAGHAN CASEY

MassBio’s 2016 Policy Leadership Breakfast tackled two of the most pressing issues facing the Commonwealth—a growing opioid addiction epidemic and a healthcare system that needs to find a balance among access, quality and cost. Attorney General Maura Healey opened the event, held on Jan. 27 at the Omni Parker House, with a reminder that, on average, heroin and prescription drug abuse is claiming four lives in the Commonwealth every day. “If we’re going to solve this crisis, we need to come together,” said Healey. “We’re looking to make treatments more available, intervene and educate people as early as possible and get Narcan in the hands of more first responders. We need strong action and we need it now. I know it’s a difficult issue but there’s no greater collection of minds than those in this room.” Healey also addressed the challenge of treating diseases such as hepatitis C, which is rising in conjunction with injection drug abuse, when the approved drugs come with a list price upwards of \$100,000. “There’s an infectious disease that’s rising, and there’s a cure,” said Healey. “How do we get it into the hands of more patients? Manufacturers and investors should rightly reap their financial reward, yet patients need access.” She has since sent a letter to Gilead urging for discounted pricing on hepatitis C drugs Harvoni and Sovaldi for the Commonwealth’s public payers. Healey has made it clear she intends to use her authority to shape the conversation and public policy, and hopes to work directly with the MassBio community to do so.

“The work you’re doing is changing lives and building a healthier, more promising future,” said Healey. “But this business is fragile. The reward is great, but so are the risks and setbacks. You need support for workforce development and the [Massachusetts] Life Sciences Center. My office is here to work with you.”

“The work you’re doing is changing lives and building a healthier, more promising future.”

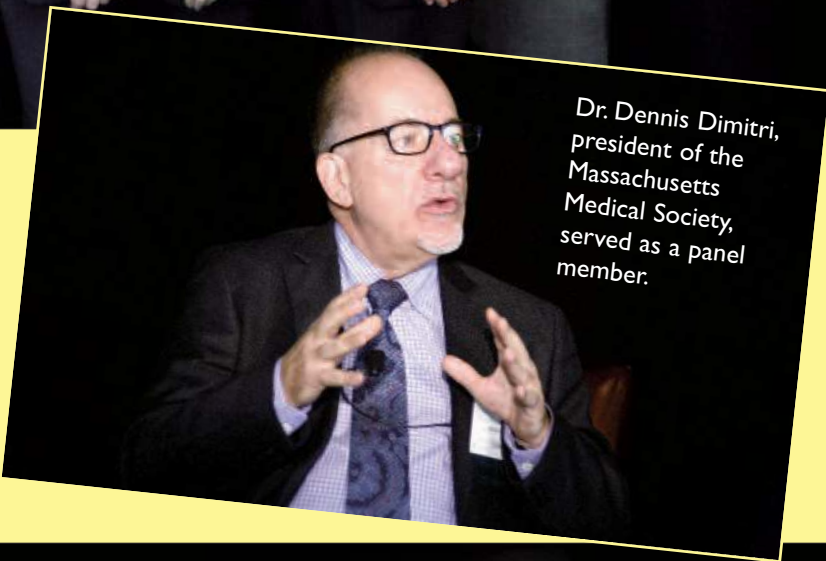
— Attorney General Maura Healey

During the panel discussion on innovation’s role in the opioid crisis, Marylou Sudders, Secretary of Health and Human Services, shared her dream for a long-term solution—the development of a non-addictive painkiller, which MassCONNECT graduate startup Blue Therapeutics is currently working on. Until that comes to fruition, companies throughout the Commonwealth, such as Alkermes, Collegium Pharmaceutical and kaléo are doing their part to create safeguards to prevent and treatments to reverse overdoses, or to find ways to block opioid pleasure receptors. Michael Heffernan, Founder, President and CEO of

Collegium, spoke about his company’s technology platform, DETERx, which provides extended-release drug delivery, while safeguarding against common methods of abuse and tampering including crushing, chewing, heating and injecting. “Like seatbelts or airbags in a car, it’s not the solution, but it’s part of the solution,” said Heffernan. “We all need to collaborate to form a fully integrated approach to fight addiction.” Closing the event, Blue Cross Blue Shield of Massachusetts CEO Andrew Dreyfus and Sarah Emond, Chief Operating Officer of the Institute for Clinical and Economic Review, joined MassBio President & CEO Robert K. Coughlin to discuss the pressures the healthcare system has faced and what to expect in the years ahead. “Personalized medicine is upon us and we’ll have to find a way to fund it,” said Dreyfus. “There will have to be a certain level of caution and a focus on those treatments that we know will be effective.” Emond agreed her organization’s value framework will have to be adjusted to measure one-time treatments such as gene therapy. Coughlin called for a Payer-Provider-Industry Task Force to develop solutions and to bring those solutions forward to policy makers. “We have a lot of work to do,” said Coughlin. “We want to cure diseases with gene therapy and invent non-addictive opioids. No one said it would be easy, but all of us, collectively, can solve these problems. We have to educate and engage every stakeholder to find a path forward together.”



Richard Pops and Spencer Williamson shared their companies' perspectives on the opioid crisis.

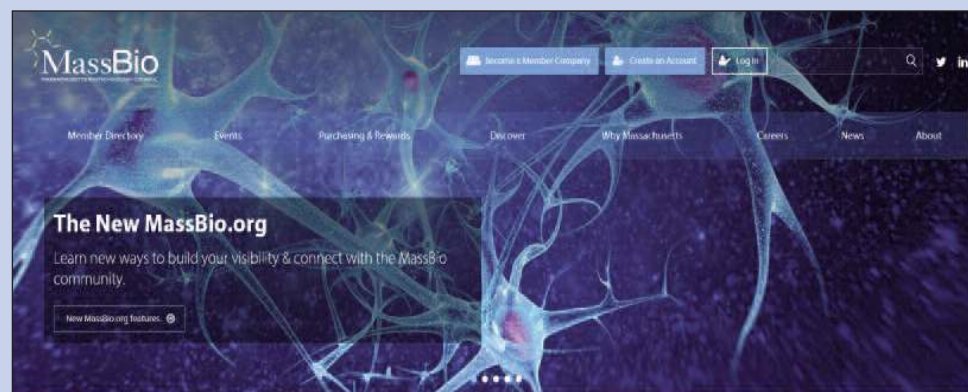


Dr. Dennis Dimitri, president of the Massachusetts Medical Society, served as a panel member.



Sarah Emond and Andrew Dreyfus joined MassBio President & CEO Robert K. Coughlin for a fireside chat.

WBUR Healthcare Reporter Martha Bebinger moderated a panel.



MEET THE NEW MASSBIO.ORG

At the beginning of February, MassBio launched the new MassBio.org, designed to give the life sciences community new resources, new visibility opportunities and new ways to connect. The sleek, responsive design not only looks better, it's built better. Key features, like MassBio's member directory and events listing, are easier to access than ever before.

Keeping the Patient at the Center

MassBio's new website will help MassBio share stories from the people at the heart of the life sciences industry: the patients. Read patient stories and learn more about MassBio member companies that are improving—and saving—lives around the world.

Improved Resources

In the new Discover section of MassBio.org, visitors can explore the wealth of resources MassBio offers including a crowdsourced Knowledge Base for MassBio members, resources for entrepreneurs, funding

opportunities and MassBio's policy work. Members can also access great savings through the MassBio Purchasing Consortium and MassBio Employee Rewards program.

The new website also includes a new MassBio Career Center with enhanced capabilities for companies that are hiring and for job-seekers themselves.

Tell Your Story on MassBio.org

Every MassBio member company has its own page in the MassBio directory for increased visibility within MassBio.org and through public searches. Members can share their latest news, events and Knowledge Base resources and write for our blog. All of this content will connect to and enhance the company's landing page.

Discover the new MassBio.org today! Contact communications@massbio.org with any questions, or if you have a patient story or blog post idea that you would like to share.

EMPLOYEE REWARDS UPDATE UNLOCK THE SAVINGS

All employees of MassBio member companies can access discounts and deals on products, services, and experiences in the Greater Boston area and beyond through the Employee Rewards program. Here are companies that have recently joined the program:

Bar Boulud: Located at Mandarin Oriental, Boston, Bar Boulud is a French-inspired bistro and wine bar from internationally acclaimed chef Daniel Boulud. MassBio members receive a 15% discount on any food item purchased during dinner service from 5:30 p.m. until 10:30 p.m. on Sunday through Wednesday.

Beekman: Inspired by the Beekman 1802 Farm in Sharon Springs, NY, Beekman is one of the fastest growing lifestyle brands in the country. MassBio members receive a 20% discount on all products.

Boston College Continuing Education Program: BC Continuing Education offers a range of courses and a full certificate program that gives professionals an

understanding of the clinical research landscape. MassBio members receive 10% off Continuing Education clinical research courses.

The Compass Tavern: Located in Worcester, the Compass Tavern offers a delicious menu featuring New England dishes made from scratch. MassBio members receive 20% off of all appetizers and lunches Monday through Friday until 4 p.m.

The Lenox: Boasting a hallmark Back Bay location, the Lenox is a family-owned, boutique hotel that expertly melds its century-old story with modern, luxurious accommodations. MassBio members can now access discounted room rates.

Wooberry: Worcester's first yogurt shop and winner of the Best of Worcester for four years straight, Wooberry is offering MassBio members 10% off of its gourmet ice cream, frozen yogurt, and smoothies.

Learn more and start saving at www.MassBio.org.



Q Why is MassBioEd undertaking a new Job Trends initiative?

MassBio's Impact 2020 report identified a challenge for companies as they grow and thrive in Massachusetts—data on and access to the right workforce. MassBio's job listings site reached an all-time high in 2015, averaging well over 2,000 listings per day. Anecdotally, industry employers are reporting difficulty in hiring for a variety of positions. Such reports raise concerns that our traditionally strong pipeline of high quality workers is under strain. It is critical for us to better understand the nature of this challenge in growing and attracting the highly-talented workers needed by the industry. That's where the MassBioEd Foundation's Job Trends initiative comes in.

Q What is the state of entry-level jobs for the life sciences industry in Massachusetts?

In January, we published the first Briefs of our ongoing Digest of Biotechnology Job Trends.

The first series of Briefs focused on entry-level job trends which make up 23% of all core job listings in the Massachusetts industry. The Briefs revealed baseline skills that cut across all degree levels, while also identifying the specialized skill requirements of leading job categories at each degree level. Of note, while Associate's degree level jobs increased as a percentage of total core industry jobs since 2010, only about 300 jobs, or 15% of all entry level job listings, are available to those who hold an Associate's degree or less.

Through this research, we discovered that we must uncover and articulate employers' expectations for entry level candidates and how academic programs can better align with industry needs.

Q What do you expect to see out of this initiative in the future? How can companies help?

Our goal is a complete understanding of industry job trends and skill requirements job seekers need. Armed with this knowledge, we can guide industry in their development of job listings and training programs, and engage higher education so that degree programs align with industry needs. It is uncharted territory and MassBioEd looks forward to producing much needed guidance for academia and industry.

Companies can help by designating appropriate staff to serve on our Skills Advisory Group. It is this Group that drives the direction of our research. Ideally, members of the Skills Advisory Group will possess a broad range of knowledge about the skills required of prospective job candidates. However, this does not exclude experts who have deep knowledge in more narrow areas or human resources generalists with broad perspectives on candidates in the job market. We hope to have a representation across the sector, from novel drug development companies to contract research to biopharma manufacturing at both small and large companies.

Save the date for MassBioEd's Conference on Job Trends on May 24, 2016.

Visit www.MassBioEd.org or contact Peter.Abair@MassBio.org to learn more and get involved.



Michele Rhee of bluebird bio and Kathleen O'Sullivan-Fortin of ALD Connect were presented with the Caring Collaborations Award.



Richard Peters of Sanofi Genzyme and Jim DeTore of bluebird bio discussed the business case for patient advocacy.



Journalist Ron Suskind spoke about his role as caregiver to his son.

SUMMIT HELPS BUILD PATIENT-CENTERED BIOTECHS

SUMMIT: from Page 1

disease, target new therapies, expedite clinical trials, and make DNA and RNA sequencing data available to the entire ALS research community.

"I think a big lesson is that advocacy starts with the patient," said Quinn. "If you're not willing to fight, who's going to? I knew early on I would be proactive. You have to put yourself out there to make things happen."

Quinn's lesson and the theme of building a patient-centered biotech was consistently repeated throughout the second annual summit, which 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.

Conversation circled around the importance

of the patient dialogue, particularly as medicine is becoming more personalized.

"With rare diseases, the patients and their families are really the ones who know the disease better than anyone. Patient advocacy is part of the fabric of what we do. I would cut everything else before I would cut that. Having someone remind us of our purpose is critical," said Richard Peters, Head of Rare Disease at Sanofi Genzyme. He joined bluebird bio Chief Financial Officer Jim DeTore to discuss their companies' efforts to build, maintain and measure patient advocacy programs.

"As rare patients, we realize we can't sit back and wait for an individual doctor or company to come up with a cure," said Kathleen O'Sullivan-Fortin, board member of ALD Connect—an international, non-profit group of ALD patients, patient advocates and researchers, who collaboratively educate, advocate, and conduct clinical research among those affected by X-linked adrenoleukodystrophy (ALD).

O'Sullivan-Fortin, who has a 10-year-old son with ALD, joined Michele Rhee of bluebird bio to talk about collaborating to achieve impressive outcomes including ALD being added to the federal Recommended Uniform Screening Panel. The pair was presented with MassBio's Caring Collaborations Award.

"It's amazing what you can accomplish when you don't care who gets credit," said O'Sullivan-Fortin. "We're trying to build a big, strong net to catch the cure. As a mom, it doesn't matter who finds a solution first. I hope everyone is wildly successful."

Another parent, Ron Suskind, a Pulitzer Prize-winning journalist and best-selling author, shared his experiences as caregiver to his son, Owen, who was diagnosed with regressive autism at the age of three. Recognizing Owen's affinity for Disney movies, Suskind began channeling characters to open up communication with his son.

"Look through the eyes of the patient, it's

amazing what you'll learn," said Suskind in an emotionally-charged keynote address.

Capping off the day's case studies, Victoria Dibiaso of Sanofi and Dr. Steven Edelman of Taking Control of Your Diabetes (TCOYD) talked about their work together to design a clinical trial that fits the individual needs of each patient.

"We wanted a two-way conversation to incorporate patient insights, reinvent research models and make smarter, faster decisions," said Dibiaso. "Based on feedback, we've been able to simplify our prototype and reduce development timelines. If we have the ability to make someone's life better, we have an obligation to do it fast and efficiently."

The day-long event, held at Google's Cambridge campus, also included panel discussions on how technology and patient data are changing drug discovery and development and how companies can set clear expectations for the patient in the case of expanded access.

LAABS, AURA BIOSCIENCES PROVIDE HOPE FOR FUTURE

PATIENT: from Page 8

Aura is in the process of submitting an Investigational New Drug (IND) application to the FDA and is advancing toward clinical testing. De los Pinos hopes to enroll 24 patients in the trial and has 10 hospitals throughout the U.S. lined up as sites. Dr. Evangelos Gragoudas, Director of Retina Service at the Massachusetts Eye and Ear Infirmary of Harvard Medical School, and Dr. Carol Shields at the Wills Eye Hospital in Philadelphia, are serving on the Clinical Advisory Board and helping to guide the clinical development. Dr. Ivana Kim, Co-Director of the Ocular Melanoma Center at Mass.

Eye and Ear, will also participate as one of the investigators in the trial.

"It has the potential to be transformative," said Kim. "The main benefit would be eliminating some of the side effects of radiation we're seeing now, such as damage to the retina or optic nerve, that lead to vision loss. I would also love to see it developed into a diagnostic tool, to test borderline lesions that could be treated early. That would be a big step forward."

As a survivor, Laabs is encouraged by the progress being made and is hopeful he, too, can play a role. Following his experience with ocular melanoma, he founded the Rare Cancer Research Foundation (RCRF) to advance

efforts to develop new and better therapies to treat rare cancer patients.

Laabs, who built a successful career in global renewable energy development and solar product distribution, sold his clean energy finance company, Climate Bridge—which had become one of the largest players in the global carbon markets—to embark on this, a different kind of socially innovative project.

"In remission, I was left with questions of what to do next—really where I could make the greatest positive impact as possible over the course of my lifetime," said Laabs. "I spent a lot of time thinking, 'should I work directly in ocular

melanoma?' But all these cancers have common needs, and I realized that I don't want anyone dying of cancer, no matter what type. So I came to the conclusion that the best use of my time and talent would be to connect with groups doing really important work and help them to do that work more efficiently."

Working with more than 200 foundations and medical research organizations, RCRF administers the research infrastructure and enables researchers to focus on novel, incremental innovation. The foundation offers solutions for patient registries, tissue samples, cell lines, animal models, and genome sequencing, available to researchers through a single, virtual, integrated

platform. The hope is that it will radically accelerate the time-to-market for new, life-saving therapies. At the same time, researchers can reallocate time, money and attention to novel drug development efforts and bringing new therapies to market.

"We're a supporting player, supplying the tools and the building blocks," said Laabs. "The most important thing is improving patient quality of life and longevity. To see better patient outcomes, it's necessary to bring together different groups to solve problems that not one individual group could solve on its own. It's that collaboration that I would want to see in my own treatment."



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**PATIENT
PROFILE**

A PATIENT'S DESIRE TO HELP

Laabs' foundation and Aura Biosciences advance efforts to develop new and better therapies to treat rare cancer patients

BY MEAGHAN CASEY

Four years ago, Memphis native Mark Laabs' future was bright. He was 28, working in China as chief operating officer of a company dedicated to fighting climate change, and living out his calling to follow his "personal North Star."

A single moment completely shifted where that North Star was leading him.

While in a meeting in Beijing, Laabs lost vision in his right eye. Nearly 48 hours and five consultations later, he was sitting with the dean of ophthalmology at a major medical center, being told it was ocular melanoma.

A rare cancer, ocular melanoma is diagnosed in about 2,500 adults every year in the U.S. Although produced from the same cells, called melanocytes, ocular melanoma is different from skin cancer. It develops in the uvea, or uveal tract, of the eye. In approximately half of those diagnosed, the tumor can aggressively grow and spread to other parts of the body, becoming fatal.

As Laabs quickly discovered, there have been no targeted therapies available and the cancer is usually treated with an invasive radioactive plaque placed against the exterior of the eye near the tumor. Known as brachytherapy or plaque therapy, this treatment can require multiple surgeries and can lead to cataracts, retinopathy and loss of vision. The alternative to radiation is surgery to remove the eye.

"I was essentially a biohazard for eight days while the radioactive plaque was on my eye and at the end of that process, the tumor was dead," said Laabs, who had flown back to the U.S. for treatment in Philadelphia. The treatment left him in remission, but he was unable to regain most of the vision in his right eye.

Aura Biosciences, a Cambridge-based biotech company developing highly tumor-targeted breakthrough therapies for rare cancers, is hoping to improve the options for future ocular melanoma patients. The new class of therapies would potentially target and destroy cancer cells selectively, while leaving surrounding tissue unharmed—an approach Aura calls



Elisabet de los Pinos is CEO of Aura Biosciences.

molecular surgery. It would effectively transform the treatment of ocular melanoma into a routine outpatient procedure.

"By enabling physicians to treat cancer more selectively, effectively and safely than they can do today, we aim to eliminate the need for risky procedures that carry significant morbidity and often do little to improve a patient's overall survival," said Aura's Founder and CEO Elisabet de los Pinos. "When you're focusing on a rare disease and thinking about the limited and highly invasive alternatives out there, it creates an incredible sense of purpose. Everything is aligning at the moment and, if successful, the end product will be extremely meaningful for patients."

In May, Aura was granted Orphan Disease Designation by the FDA for its lead product candidate, AU-011. The company's pre-clinical research, 'Evaluating the in vivo efficacy of a first-in-class drug for the treatment of primary uveal melanoma,' was delivered that same month by McGill University Health Centre researchers at the 2015 Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting.

"Aura's novel approach has the potential to dramatically improve the outcomes, and hope, for patients with

ocular melanoma," said Grant Allen, Co-Founder and Chairman of the Ocular Melanoma Foundation. "The Orphan Drug Designation for AU-011 is a huge step forward, enabling Aura to potentially bring this drug to patients in an expedited manner."

AU-011 consists of a viral-like particle drug conjugate that binds selectively to cancer cells in the eye and upon activation with an ophthalmic laser, the small molecule selectively destroys the membrane of cancer cells, killing them without damaging the adjacent retina.

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Ocular melanoma survivor Mark Laabs founded the Rare Cancer Research Foundation.