

THE NEXT GENERATION OF BIOTECH

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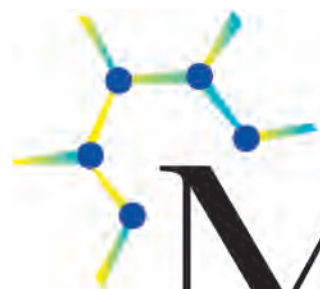


MISSION FOR A CURE

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Vol. 7, No. 2 | A publication of the Massachusetts Biotechnology Council | Spring 2014



MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

FORGING PARTNERSHIPS TO ADVANCE THE LIFE SCIENCES

ANNUAL MEETING

PAGES 4-5

FDA Commissioner Dr. Margaret Hamburg, left, joined MassBio President & CEO Robert K. Coughlin and Sen. Elizabeth Warren at the 2014 MassBio Annual Meeting.

PHOTO: SEAN BROWNE



COMMITTED TO THE CRO/CMO COMMUNITY



Attendees network and meet potential partners at the CRO/CMO Symposium.

BY MEAGHAN CASEY

Addressing the nearly 300 attendees gathered for the third annual Massachusetts CRO/CMO Symposium on May 5, MassBio President and CEO Robert K. Coughlin pledged to focus attention on advocating for policies that will enable the contract services sector to grow.

“CROs and CMOs play a critical role in drug discovery and development and we’re committed to this slice of the industry, in Massachusetts and beyond,” said Coughlin.

Responding to survey data which shows one in every three biopharma jobs in Massachusetts is at a CRO or CMO, MassBio’s executive team announced a new campaign to advocate for changes to Massachusetts’ tax incentive and financing programs to broaden access for contract services companies. Most CROs and CMOs cannot access incentive programs under the Life Sciences

Initiative or other state programs.

“We must reshape the state’s incentive programs in order to make the most of our opportunity,” said Coughlin. “In the end, it’s about reducing the cost and speeding the time to market for new drugs and therapies for patients in need.”

Earlier this year, the first census of the New England CRO/CMO community reported that contract-services companies employ nearly 17,000 people and occupy more than 5.6 million square feet of space.

“The key advantage to our New England CRO/CMO cluster is that it’s broad and deep, but in order to compete, we really have to lead,” said Peter Abair, MassBio’s Director of Economic Development and Global Affairs.

Impact 2020, MassBio’s industry strategic report,

See **CRO/CMO** Page 7

FUELING OUR INDUSTRY FOR THE FUTURE



ROBERT K. COUGHLIN

Thank you for your support as we continue to serve as the greatest advocate for the Massachusetts life sciences cluster, and for all the life-altering therapeutic and diagnostic solutions that our academic research centers, teaching hospitals and companies provide for patients. Last month, we released Impact 2020 – a culmination of nearly nine months of meetings, discussions, interviews and focus sessions. We

embrace and accept the recommendations of that report as a mandate to advocate for change, to convene industry leaders and to offer relevant programs that prepare our constituency to respond to the rapidly changing environment. Massachusetts may be the leader in life sciences, but we cannot rest on our laurels. The implications of decisions today—the clinical trials that start or don't start, the companies that are founded or not founded—will not be evident for 10 to 20 years. We must continue to champion innovation in order to bring value to the global healthcare system. Healthcare is all about patients, and value will ultimately be measured in patient outcomes.

I am grateful to those of you who attended our Annual Meeting, as well as the CRO/CMO Symposium. Both were highly successful in bringing you the information, connections and discussions you need in order to strengthen the supercluster here in Massachusetts. I encourage you all to attend the 2014 BIO International Convention in San Diego, as well as BioPharm America, right back here in Boston.

Remember: We create tomorrows. We improve the quality of life for patients. And we look forward to continuing to work with all of you.

Robert K. Coughlin is President and CEO of MassBio.



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RARE DISEASE DAY



Patient advocates, industry stakeholders and legislators gathered at the Mass. State House to mark the seventh annual Rare Disease Day.

Event speakers included (from left) Ilene Sussman, Executive Director of VHL Alliance, Pamela Gavin, Chief Operating Officer of the National Organization for Rare Disorders, Katie Brandt, caregiver & volunteer coordinator for the Association for Frontotemporal Degeneration, and Carmen Camacho, a patient with the Hermansky-Pudlak Syndrome Network Inc. Also pictured is event organizer Sarah MacDonald, VP of Development & Communications at MassBio.

5 GREAT REASONS TO BE...AT THE BETC



The Biomanufacturing Education and Training Center (BETC) is a pilot-scale training facility with state-of-the-art classrooms and labs at Worcester Polytechnic Institute. The BETC's practical, hands-on approach includes courses developed collaboratively by WPI and industry partners—and we customize training as needed to best serve biomanufacturers and their employees.

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UBI France
uniQure, Inc.
Vince & Associates Clinical Research
Voyager Therapeutics, Inc.
Wake Up Narcolepsy, Inc.
XB Insight, Inc.

Q What is the current climate for life sciences startups and how do you see that changing in the next five years?

A This is an incredibly exciting time for the life sciences industry, particularly in Massachusetts. Over the past decade areas of science that had historically been seen as very promising but largely futuristic, such as RNA therapies, gene therapies, and nanotechnology, are now poised to yield significant, even transformative, benefits for patients. In these fields much of the pioneering academic research and many of the leading companies have emerged from the Massachusetts life sciences cluster. With our accelerating understanding of disease biology, traditional small molecule and biotherapeutics drug modalities have created major breakthroughs. For example, the first disease modifying therapy for cystic fibrosis was discovered and commercialized here in Massachusetts. Success builds confidence and attracts both investment and talent. The enthusiasm in the public capital markets for the biotechnology sector has injected large amounts of capital into the local ecosystem. Also, we continue to see a migration of pharmaceutical company research centers to the Massachusetts area. All this bodes well for the prospects of startups. Launching a startup is never easy, particularly a life sciences startup, but I believe we are entering promising new era. The key to success will be staying sharply focused on the value chain right from the very beginning by developing products that dramatically improve the lives of patients. I am very optimistic about the opportunities of the next five years in both what we can accomplish for patients and as an industry.

Q What advice would you give to entrepreneurs in Massachusetts today?

A Any success I have had as an entrepreneur I attribute to the many generous and talented mentors and colleagues I have had the privilege to work with and who have supported me over time. The unprecedented geographic concentration of

Q & A

WITH GLENN BATCHELDER

*Founder and Board Member of Citivas Therapeutics and
Chairman of the MassBio Board of Directors*



the Massachusetts life sciences cluster has not only drawn together one of the most remarkable talent pools in the world of leading physicians, scientists, engineers, drug developers, venture capitalists, and entrepreneurs, but they are often only a short walk or drive away. My best advice to a new entrepreneur is go seek them out, get to know them, learn from them. Whether through MassBio, the MIT Enterprise Forum, Xconomy or the many other organizations that facilitate making connections, these pioneers and veterans are accessible and generally willing to help. I believe our ecosystem is at the core of what makes Massachusetts such a fertile ground for startups. This network is readily accessible for any tenacious, talented entrepreneur with a good idea.

Q What are the most important challenges and opportunities on the horizon for MassBio?

A I think the truism that the greatest risk associated with establishing a leadership position is complacency applies to the Massachusetts life sciences cluster. Our critical priorities need to be continuing to strengthen our unique ecosystem, proactively adapting to the rapidly evolving landscape and ensuring we do not become complacent. The cluster was strengthened through the Patrick Administration recognized early on the importance the life sciences to Massachusetts' economy and visibly bolstering the underpinnings with initiatives like the Massachusetts Life Sciences Center a focus. Going forward we need to ensure all the stakeholders remain focused on maintaining the vibrancy and growth of the life sciences industry. To support innovation we need to ensure we focus on translating research and development

into value for all constituencies: patients, doctors, healthcare centers, life science companies and payers.

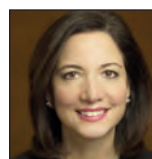
One important dimension in delivering value is continuing our progress in precision medicine, right medicines get to the right patients through more targeted therapies along with enabling diagnostics and data platforms. This is important both for improving the patients' experience as well as to ensure our innovation model remains viable and affordable for society. With leaders from all the constituencies present in Massachusetts and MassBio uniquely positioned to bring the various constituencies together and collaboratively find solutions, this is our opportunity and our responsibility. The recently published Impact 2020 report provides a roadmap and I am confident we can make significant progress together.

WELCOME TO THE BOARD

MassBio has elected and installed the following new board members:



Jim Burns is Head of the Boston R&D Hub at Sanofi. He was Senior Vice President and Head of Drug and Biomaterial R&D at Genzyme prior to joining Sanofi. He earned a B.S. in biology from Purdue University and an M.S. and Ph.D. in bioengineering from the University of Illinois-Chicago where his thesis work focused on drug delivery. Following his graduate studies, he was a postdoctoral research fellow in the Materials Science and Engineering Dept. at the University of Florida. He is a member of the National Academy of Engineering.



Liz Lewis is Chief Counsel & Chief Compliance Officer Millennium: The Takeda Oncology Company/Takeda Pharmaceutical International Co. She has been instrumental in creating Millennium's legal and compliance functions that support the global oncology business and has played an integral role in the successful launch, commercialization and market expansion of VELCADE. Liz has more than 20 years of legal expertise advising manufacturers, payors and other ancillary providers in the health care life sciences space. Prior to Millennium, she served as a partner in the national health law practice of Epstein, Becker & Green. She was inducted into the Takeda Leadership Institute, serves as the chair for various steering committees, and is an active participant in industry trade

organizations, PhRMA and BIO, often being asked to provide legal and compliance leadership. Recently, the *Boston Business Journal* named Liz as one of the top 20 "2014 Women to Watch" in the life sciences industry.



Paris Panayiotopoulos is President and Managing Director of EMD Serono, Inc., one of the top U.S. biopharmaceutical companies focused exclusively on specialty care. He came to EMD Serono from the company's Japan subsidiary, Merck Serono, where he served as President and Managing Director. Other positions he held at Merck Serono include Head of Western Europe Operations, Director of Global Marketing, Global Commercial Team Leader and Head of the COO Office. Prior to that, he served in roles across multiple therapeutic areas and global brands at

Eli Lilly & Company, based in the United Kingdom.



Adlene Perkins has served as President and CEO of Infinity Pharmaceuticals since 2009 and as the company's board chair since 2012. She has played an integral role in leading the company, guiding its transition from a platform to a product-focused organization, building the team and forming strategic partnerships to advance the company's product pipeline and establish a strong financial foundation. She has more than 25 years of international business experience in the biopharmaceutical industry, focused on building and leading high-caliber, cross-functional teams, corporate strategy, licensing and business development, strategic finance and product life cycle management. Prior to Infinity, she held leadership

positions at TransForm Pharmaceuticals, Genetics Institute and Bain & Company.



Rick Russell is Executive Vice President and Chief Commercial Officer at Sunovion Pharmaceuticals, providing leadership for North American and U.K. commercial organizations. In his role, he drives a patient centric culture across sales, marketing, commercial operations and managed market group activities. He has more than 20 years of experience leading commercial operations and in developing and implementing sales and marketing activities for major pharmaceutical brands. His pharmaceutical experience spans several therapeutic areas ranging from central nervous system disorders, women's health, cardiovascular disease and neurodegenerative disorders.

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OPPORTUNITIES ABOUND

STAKEHOLDERS GATHER AT ANNUAL MEETING

TO DISCUSS THE FUTURE OF BIOTECHNOLOGY

By Meaghan Casey

The importance of forging partnerships and harnessing the power of policy makers to advance the life sciences was a focal point of the 2014 MassBio Annual Meeting, held April 3-4 at the Royal Sonesta Hotel in Cambridge.

“In my 30 years, I can’t recall a more exciting time,” said Biogen Idec CEO George Scangos. “What were theories a decade ago are becoming realities. We’ve never had so much opportunity to do so much good for so many people. But, we’re also in an era where the science is getting ahead of the regulatory process. Maintenance of this ecosystem is essential.”

Scangos was presented with the Henri A. Termeer Innovative Leadership Award, named for the retired Genzyme president. He is the sixth industry executive to receive the annual award, which recognizes a leader who actively supports community-based organizations, has a strong presence and commitment to growth in Massachusetts and contributes to science education to prepare the future workforce.

“Henri Termeer exemplifies courage and determination and this award is a constant reminder to never stop trying to do what’s right and what’s best,” said Scangos.

Also at the event’s award luncheon, the Gov. Deval L. Patrick administration—represented by Dr. Susan Windham-Bannister, President of the Massachusetts Life Sciences Center—was presented with the MassBio Leading Impact Award. The award honors individuals and organizations that have successfully worked to improve the competitiveness of Massachusetts as a destination for the life sciences industry.

U.S. Food and Drug Administration (FDA) Commissioner Dr. Margaret Hamburg—one of the event’s three keynote speakers—highlighted what the agency is doing to facilitate and expedite development and review of new drugs. She said more than three-quarters of products approved last year were approved in the U.S. first. This has been a result of programs such as breakthrough therapy designation, fast track designation, accelerated approval and priority review.

By reaching out to biotech and pharmaceutical companies early on and working with them throughout the development process, Hamburg said the time needed to develop new products has decreased by as much as five years for some companies.

“It underscores that engagement and collaboration can really make a difference,” said Hamburg. “Our goal is to enable a rapid and reliable review process and deliver on the promise of science. It matters to you, it matters to us and, most of all, it matters to the patients.”

In her introduction of Hamburg, Sen. Elizabeth Warren described her as “a person of great vision.” Warren said she and other law makers are urging Congress to double funding for the National Institutes of Health (NIH) and do more to cultivate innovation and government-supported research.

“We have the opportunity to support a new generation of scientists and build economic development

here in Massachusetts and across the country,” said Warren.

The keynote speakers in addition to Hamburg were Brad Margus, CEO of Genome Bridge, and Dr. Flemming Ornskov, CEO of Shire. Both brought the focus back to the patients.

Margus transitioned from running a shrimp processing company to becoming a self-taught genetics expert when two of his two sons were diagnosed in 1993 with ataxia-telangiectasia. Better known as “A-T,” it is an extremely rare genetic disease that combines a loss of muscle control with cancer and immune deficiency.

“My response was to learn the science, how it’s funded and how to raise funds,” Margus said.

He created a nonprofit patient advocacy group called the A-T Children’s Project, launched Perlegen Sciences Inc., co-founded Envoy Therapeutics Inc. and now heads Genome Bridge, a non-profit organization working with partners to build an unparalleled technology for aggregating genomic and clinical data to improve medicine. Though there is still no protein identified with A-T to target with a drug, he continues to watch treatments developed for other diseases.

“I want to explore every new technology,” said Margus. “I’ve had some of the most fertile and interesting discussions right here in Massachusetts. My hope is that we can learn from the genome and affect science.”

Ornskov, who started out in a neonatal unit and has extensive experience in the pharmaceutical sector, is optimistic about the future of the industry. “We’ve come a very long way since my days as a physician,” he said. “A lot of the ‘We don’t know’ has turned into ‘We have an option for your child.’ Whatever we do, we want to make the treatment of patients simpler.”

During the event, MassBio also unveiled its strategic report, Impact 2020. A panel of experts discussed the report and its recommendations, stressing a need for the industry to center on the patient, demonstrate the value of innovation, maximize access to capital, cultivate anchor companies and capitalize on information technology.

“We’re at this moment when the world is wrestling with the issue of how we’re going to afford to produce the drugs for our aging populations,” said Civitas Therapeutics Founder Glenn Batchelder, who was elected the new MassBio Chairman at the meeting. “How do our innovations and breakthroughs translate into viable treatments that are accessible to all?”

“The issues we’re seeing are global issues,” said Skip Irving, partner and managing director of Health Advances. “Healthcare costs and funding innovation are challenges we all face. To solve some of these issues moving forward, we need the leaders of our biggest companies engaged in dialogue.”

The Annual Meeting also featured breakout sessions on innovative ways to fund early-stage companies; clinical trial trends; mobile technology and 3D printing; converging relationships among biotech, pharma, investors and academia; the image problem of the industry; the second coming of molecular therapies; and new approaches to treatments for neurological disease, among others.



Biogen Idec CEO George Scangos looks on as MassBio President & CEO Robert K. Coughlin presents him with the Henri A. Termeer Innovative Leadership Award.



Attendees network at the reception hosted by Alexandria Real Estate.



Katrine Bosley and Glenn Batchelder discuss the recommendations of Impact 2020.



MassBio President & CEO Robert K. Coughlin and Deanna Petersen of Shire HGT, present Dr. Susan Windham-Bannister, representing Gov. Deval Patrick, with the MassBio Leading Impact Award.



Dr. Flemming Ornskov, CEO of Shire.



Brad Margus, CEO of Genome Bridge.



FDA Commissioner Dr. Margaret Hamburg.

ASSABET VALLEY HONORED FOR INNOVATION



MassBio President & CEO Robert K. Coughlin, Superintendent Patrick Collins, Joshua Boger, student Alex McMahon, Lisa Adler of Millennium: The Takeda Oncology Company, teacher Monica Hayes and MassBioEd Executive Director Lance Hartford.

Assabet Valley Regional Technical High School in Marlborough was presented with the Joshua Boger Innovative School of the Year at the 2014 MassBio Annual Meeting. The award honors a Massachusetts school for inspiring students to explore life sciences careers through innovative biotechnology education and exemplary science career programming.

Assabet was presented with one of MassBioEd's BioTeach awards in 2008 and has since enriched its science program by developing external partnerships, introducing student-driven research projects and encouraging student peer-teaching experiences. The school has also formed a STEM Alliance Team, comprised of biology, chemistry, math, engineering and biotechnology faculty members who are working to fully integrate biotechnology into both academic and vocational studies.

"We have come so far thanks to the materials and the support of MassBioEd, the equipment donations and the

mentoring," said Assabet biotechnology teacher Monica Hayes. "The world of biotechnology can be intimidating, but the resources and field trip opportunities we have been given are invaluable. They have changed the face of biotechnology at Assabet and inspired many students to consider biotech-related careers."

"The experiences have helped me realize a life in biotechnology is for me," said sophomore Alex McMahon, who visited ARIAD Pharmaceuticals as part of MassBioEd's Biotechnology Job Shadow Day and Worcester Polytechnic Institute through the foundation's Biotech Futures program. "I someday hope that a career in this industry will allow me to say that I have been able to contribute to the greater good of humanity and that the work I've done will leave a lasting impact on society. Currently, this is a dream. But I know that if I continue to pursue this dream by putting forth my hardest work, this dream will come true."

BACK TO SCHOOL

MASSBIOED ANNOUNCES PROFESSIONAL DEVELOPMENT COURSES FOR THE FALL

Clinical Trial Overview: The Players, Process & Language

Sept. 16 & 23, 5:30 p.m. - 9 p.m.

This course will provide an overview of clinical trials, particularly those involved, how trials work, and the basic terminology of clinical trials.

Biotech 101 for Non-Scientists

Sept. 18-19, 9 a.m. - 4:30 p.m.

or Dec. 4-5, 9 a.m. - 4:30 p.m.

Designed especially for non-scientists looking to learn more about the industry, this course includes hands-on activities and easy to follow lectures that will teach you the basics of the industry.

Managing Clinical Trials

Oct. 7 - Nov. 11, Tuesday evenings,

5:30 p.m. - 9 p.m.

This modular, 6-session workshop will introduce the major skills needed to manage outsourced clinical studies within a CRO/sponsor environment.

Biotechnology Project Management

Oct. 8 - Nov. 12, Wednesday evenings,
5:30 p.m. - 9 p.m.

Gain practical experience with project management tools and learn strategies for working in a team environment to analyze risk, allocate resources, track projects, resolve conflicts, make decisions, and manage projects.

Leading & Managing Effective Teams

Oct. 16-17, 9 a.m. - 4:30 pm

Learn how to effectively manage teams and time, and learn systematic approaches to making better decisions.

Influence Without Authority

Friday, Nov. 7, 9 a.m. - 4 p.m.

Improve your ability to manage "up," get agreement from peers and work effectively in project and cross-functional teams.

All programs will be held at the MassBio offices, 300 Technology Square, Cambridge. To view a full course listing and to register, visit www.MassBioEd.org and click on Corporate Professional Development.

If your company has 100 or fewer employees you may be eligible to receive up to 50% reimbursement for the cost of training under the state's Workforce Training Fund Express Grant Program. Contact julie.deschenes@massbio.org to learn more.

NEW SAVINGS IN PURCHASING CONSORTIUM

Through the Purchasing Consortium, MassBio is continuously working with companies to provide the best resources for MassBio members. This spring, the Purchasing Consortium has expanded to bring discounted auto insurance, promotional products and HPLC Qualification to MassBio members.



Columbia Insurance Agency, Inc.

Columbia Insurance has joined the Purchasing Consortium as MassBio's Primary Supplier for Auto Insurance. Columbia offers MassBio members a 6% discount on auto insurance through Plymouth Rock Assurance and discounted homeowners insurance. Columbia Insurance will come to your work place to meet with employees and they will make trips to the RMV so employees do not have to take time off of work to take care of their insurance needs.

Learn more and take advantage of these new programs at www.MassBio.org.



W.B. Mason, MassBio's Primary Supplier for Office Supplies, has launched a hot list and steep discounts on promotional products exclusively for MassBio members. To make this program as accessible as possible, W.B. Mason created a special promotional products website for MassBio members that will connect with your existing W.B. Mason account: MassBio.logoshop.com



Alert Scientific, MassBio's Primary Supplier for Lab Equipment Maintenance, now offers HPLC qualification at significant discounts. Alert Scientific can repair, install, qualify and perform Preventive Maintenance on a vast array of HPLC systems. They offer accurate repair, installation and qualification of your auto sampler, column heater, pumps, and detectors VWD, DAD, RID.

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MASSBIO ISSUES IMPACT 2020 REPORT OUTLINING THE FUTURE OF BIOTECH

On April 3, MassBio released Impact 2020, a strategic report on the biotechnology and life sciences cluster in Massachusetts. The extensive report issues a call to action for Massachusetts stakeholders to take steps to safeguard the Commonwealth's definitive, but fragile, leadership position in life sciences and innovation.

Developed by consulting firm Health Advances over nine months of meetings, discussions, interviews, Twitter chats and in-person focus sessions, Impact 2020 recommends:

- Driving the conversation on defining value and reward for innovation in the era of outcomes-focused medicine;
- Seizing a leadership opportunity at the intersection of IT and life sciences;
- Evolving funding models for early stage companies and innovative ideas;
- Improving workforce development to ensure a workforce trained for the jobs of the future and support downstream expansion; and
- Highlighting patient stories to showcase the impact of innovation taking place in Massachusetts

"While we are riding a renaissance of life sciences funding, scientific advances and new product approvals in 2014, Impact 2020 identified real threats to the long-term viability of the model," said Robert K. Coughlin, President & CEO of MassBio. "We must take action to ensure our ability, regionally and as an industry, to continue delivering breakthroughs that benefit patients worldwide."

Impact 2020 examines the balance between value and evidence-based medicine in the era of healthcare reform and the uncertainty it creates for investors and entrepreneurs who seek reward for innovation and risk-taking.

"We all need a thoughtful approach to determining how new, life-changing therapies are valued and paid for," said Geoff



From left, Terry McGuire, Skip Irving, Katrine Bosley, Glenn Batchelder, and Robert Weisman discuss Impact 2020 at MassBio's 2014 Annual Meeting.

MacKay, outgoing Chair of the MassBio Board of Directors and President & CEO of Organogenesis Inc. "That dialogue needs to include private payers, Medicare, providers and the companies developing new treatments, to ensure that the needs of patients remain our core focus."

The report also analyzes new challenges in creating capital to fund new companies and drive new potential medicines, diagnostics and devices. As the cost and risk of bringing new products to market has increased, traditional investors have developed models that spread that risk. New players, including disease foundations, wealthy individuals, and even crowdsourcing organizations are filling a void left by the decrease in traditional sources of capital.

"With its unprecedented concentration of uniquely skilled talent, leading hospitals, world-renowned academic institutions, pharmaceutical research centers, and deep financial resources, Massachusetts has long been the ideal place to start a new biotech company," said Glenn Batchelder, MassBio Chair and Founder of Civitas. "To preserve this, MassBio and its partners must continue to develop supportive programs that reduce the startup hurdles for tenacious, talented entrepreneurs."

MassBio will be working with its members to engage key areas within the Massachusetts cluster to foster growth and innovation in the region. Member companies, whether large or small, established or early-stage, will play an integral role in undertaking the initiatives outlined in the report.

Specifically, MassBio will look to large and established pharmaceutical companies for collaboration on carrying out local initiatives to ensure funding, space and support for startup companies, as well as leadership around infrastructure, policy and workforce issues. Early-stage companies are encouraged to participate in MassCONNECT and to participate in other MassBio programs.

Impact 2020 includes additional analysis and recommendations around the region's opportunities at the intersection of IT and healthcare; the need to expand workforce development programs that reduce 'door to floor' time; and Massachusetts' biomanufacturing capabilities, among other topics.

Learn more at www.MassBio.org or download the full report at www.MassImpact2020.com.

COMMITTED TO CRO/CMO SUCCESS

CRO/CMO: from Page 1

outlines a new business model for drug discovery and development where companies focus on efficiency and contract out research and manufacturing functions. Data shows this trend in transitioning from in-house R&D to strategic CRO/CMO partners will continue to grow, and the report urges industry and legislative leaders to support policies that encourage this growth in Massachusetts.

"With the current funding climate making it much more challenging to build fully-integrated companies, we will see a continued surge in virtual companies and contract services to drive forward new treatments and cures," said MassBio Chief Business Officer John Hallinan.

The CRO/CMO Symposium, designed to shine a spotlight on the strength and success of the Commonwealth's CRO/CMO community, was held at the Burlington Marriott this year. Attendees discussed and evaluated the best alternatives that have evolved in shortening time to market. A series of case studies, interactive panel discussions, roundtable conversations on hot topics and networking sessions provided ample time to explore the various techniques and approaches being used



Jeff Saunders of Ember Therapeutics and Margaret Porter Scott of Epizyme participate in a panel on integrating internal and external resources.

by both virtual and established companies.

"It's an efficient and cost-effective way for people developing products for the life sciences to learn about the services that are available locally, and to meet companies with the expertise to help them advance their projects," said Brad Carver, CEO of SynDevRx.

Throughout the day, many of the symposium participants stressed the importance of communication.

"You have to view your relationships with providers not in a transactional way, but as partnerships," said James Tomos, Director of Procurement and Outsourcing for Pfizer. "We look at how we can yield more from our

conversations so that every dialogue is contributing scientifically and enhancing the work we do. All organizations look at their internal organization, but we need to nurture and enhance external relationships."

"You often see a resistance to outsourcing," said Margaret Porter Scott, Senior Director Lead Discovery at Epizyme. "I think we've been successful by bringing in the human element and building scientist-to-scientist relationships. We emphasize to our employees that they have ownership of the data even if they didn't create it with their own hands."

"We work in an industry that's about people and interaction, and it's hugely important that

as society goes more electronic, we still recognize the need to pick up the phone or schedule that face-to-face meeting and communicate directly," said Mark Goldberg, President and Chief Operating Officer of PAREXEL International Corp.

In a case study titled "It Takes a Village," Dr. Michael Singer, Chief Scientific and Medical Officer of Topokine Therapeutics, Inc., and Judith Newmark, Program Manager at Toxikon Corporation, discussed how they have been able to successfully work together.

"Our model is to be very involved with CROs, very hands-on and very responsive," said Singer.

"We like the collaborative spirit," said Newmark. "The more interaction from the sponsor side of things, the better the collaboration will be and the more successful the study will be. We understand the need to be flexible, but we also understand that everyone involved needs to be informed and know where and how to move with the information we're giving them."

Symposium Coordinator Shelley Amster, Principal and Founder of ShelleyCO, LLC, echoed the panel members' remarks.

"Whether you're running Big Pharma or a one- or two-person operation, you're running a symphony," said Amster. "Every member has to be in sync. It doesn't matter how strong your science is without great organization and communication skills."



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**PATIENT
PROFILE**

WAKE-UP CALL



Thomas Gow presents a check of \$30,000 on behalf of Wake Up Narcolepsy to Mali Einen of the Stanford University Center for Narcolepsy.

FAMILY RAISES AWARENESS FOR NARCOLEPSY

BY MEAGHAN CASEY

When Monica and David Gow's son, Thomas, was diagnosed with narcolepsy with cataplexy, they were determined to bring greater awareness to the disease and help in the search for a cure.

They teamed up with Kevin Cosgrove to establish Wake Up Narcolepsy, Inc., which has quickly become a national leader for narcolepsy research and awareness. Cosgrove, a

medical sales representative for Aurora Laboratories, was diagnosed with narcolepsy in 2006. In addition to his role as the organization's co-founder, he has become a role model to Thomas, who was diagnosed in 2008 at age 11.

"My husband and I observe in amazement our son's quiet determination while he struggles to manage his life with narcolepsy," said Monica. "He continues to trudge forward, never complaining. We're lucky to have met Kevin and created the network that we have today. That part—getting to know other people with the disease—has been really good for him, and for us."

Narcolepsy is a chronic brain disorder that involves poor control of sleep-wake cycles. People with narcolepsy experience periods of extreme daytime sleepiness and sudden, irresistible bouts of sleep that can strike at any time and can last a few seconds to several minutes. They may unwillingly fall asleep while at work or at school, when having a conversation, eating a meal or performing a number of other daily activities. Other symptoms include vivid dream-like images or hallucinations.

"Three months before (Thomas) was diagnosed, he started falling asleep all the time—while he was reading a book or at the dinner table," said Monica. "He ended up collapsing during a pediatric visit and they sent us straight to the emergency room."

After initially having his symptoms dismissed and being misdiagnosed twice, a multiple sleep latency test (MSLT) revealed that Thomas had narcolepsy. Monica and David both have the genetic marker for the disorder, but never had

symptoms themselves, nor do their two younger children, ages 12 and 14. Thomas, now 16, was also diagnosed with cataplexy—a sudden loss of muscle tone, which makes a person go limp or unable to move.

"Narcolepsy is too often misunderstood and misdiagnosed, which is why we started on our mission," said Monica.

Every year, Wake Up Narcolepsy hosts various awareness-driven events and makes substantial contributions to cutting-edge narcolepsy research across the country. Last year, the organization donated almost \$90,000 to two of the world's leading scientific investigators studying narcolepsy: Dr. Thomas Scammell of Beth Israel Deaconess Medical Center and Dr. Emmanuel Mignot, Director of the Center for Sleep Sciences and the Center for Narcolepsy at Stanford University's School of Medicine.

"Over the last few years, our research has really benefited from Wake Up Narcolepsy's support," said Dr. Scammell, who is also an associate professor of neurology at Harvard Medical School. "It's allowed us to pursue high-risk experiments that we have not been able to fund through the NIH."

A majority of the organization's funds have been raised during the Boston Marathon over the past five years. In 2010, 2011 and 2012, Monica led Wake Up Narcolepsy's team of runners, raising remarkable awareness and research funds. David has led the team the last two years. In 2013, he was among the top 20 fundraisers of all Marathon runners, bringing in over \$38,000 to benefit Wake Up Narcolepsy's work. This year, he and 13 other runners raised more than \$85,000 for the organization.

"Hockey, not long-distance running is my first sport, so training for and running a marathon is a stretch for me," said David. "But I'd do anything for my children. Every day I watch Thomas struggle to overcome his narcolepsy. Running a marathon pales in comparison to the challenges he faces living with this invisible disease. If my efforts help kids like Thomas lead more normal lives, it's all worth it."

Through the organization, the Gows have also been active advocates, locally and on Capitol Hill. They are currently piloting a program in Massachusetts called Narcolepsy Goes to School, which is helping to teachers to recognize symptoms of the disease. Through social media, the idea has been expanding to other states.

Thomas, meanwhile, has been benefitting from treatments that are controlling his symptoms. One acts as a stimulant, while another controls the cataplexy, some of his daytime sleepiness and fragmented nighttime sleep.

"It's been life-changing," said Monica. "He still has to nap at school and be diligent about his sleep schedules, but he's been able to live an active life and keep doing the things he loves, like playing hockey and golf."

To learn more, visit www.wakeupnarcolepsy.org.