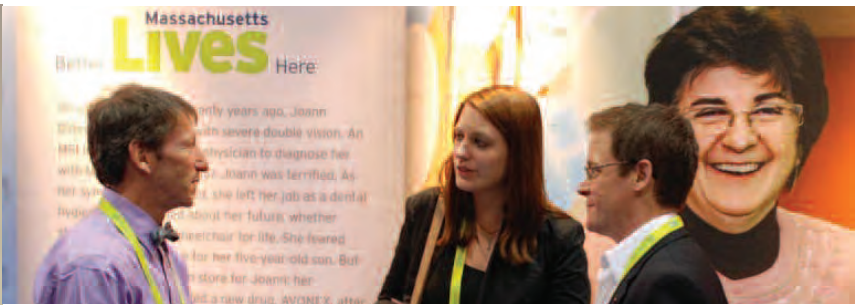




BIO RECAP Page 5



Vol. 6, No. 2 | A publication of the Massachusetts Biotechnology Council | Spring 2013



MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

RESEARCH LIVES HERE



FDA Commissioner Dr. Margaret Hamburg

Declaring it a golden age for biomedical research, FDA Commissioner Margaret Hamburg appealed to the nearly 400 biotechnology industry leaders gathered for MassBio's 2013 Annual Meeting for increased collaboration, noting that more progress needs to be made to effectively translate scientific discoveries into therapies, prevention or cures.

Pages 4-5

PHOTOS: SEAN BROWNE



John Crowley, Chairman & CEO, Amicus Therapeutics



PATIENT Page 8



Page 2



Q&A Page 3

COMMITTED TO INDUSTRY'S MISSION



ROBERT K. COUGHLIN

We at MassBio are committed to maintaining and growing an ecosystem where each of our member companies can achieve its full potential. We believe in this industry's mission of treating and curing disease, driving the economy and meeting unmet medical needs for patients everywhere.

Thank you to all who joined us in the past few months at some of our biggest events of the year – our Annual Meeting, the 2013 BIO International Convention in Chicago and the MA CRO/CMO Symposium. I appreciate your support and I hope you enjoyed the discussions on the most pressing industry issues and what we need to do to ensure Massachusetts remains the world leader in life sciences.

It seems like just yesterday we were publishing our landmark 2015 Strategic Report and we are already in the process of developing our 2020 Report. It will serve as a viable roadmap for advancing both collaboration and innovation in the Commonwealth.

We save lives, we create jobs, but now is no time for complacency.

This association could not exist without our members and your active support, and I urge you to remain mobilized and involved.

Robert K. Coughlin is President & CEO of MassBio.

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MassBio news

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The last day of February has been designated as Rare Disease Day in Massachusetts to call attention to the public health issues associated with rare diseases, which affect nearly 30 million Americans and countless others around the world. This year's event was held on Feb. 28 at the State House.

MASSACHUSETTS RECEIVED \$2.47 BILLION IN NIH FUNDING IN 2012. FOUR OF THE TOP 15 NIH-FUNDED PRINCIPAL INVESTIGATORS WERE BASED IN MASSACHUSETTS.

| | Principal Investigator | AWARDS | FUNDING | Affiliation | City |
|----|------------------------|--------|--------------|--|-------------------|
| 1 | KRISCHER, JEFFREY P | 4 | \$50,654,119 | University of South Florida | Tampa, FL |
| 2 | LANDER, ERIC S | 3 | \$46,908,294 | Broad Institute of Harvard and MIT | Cambridge, MA |
| 3 | WILSON, RICHARD K. | 3 | \$44,991,288 | Washington University School of Medicine | St. Louis, MO |
| 4 | COREY, LAWRENCE | 8 | \$44,773,218 | Hutchinson Cancer Center, University of Washington | Seattle, WA |
| 5 | KURITZKES, DANIEL R. | 3 | \$37,274,377 | Brigham & Women's Hospital | Boston, MA |
| 6 | JOHNSTON, S. CLAIBORNE | 6 | \$33,325,070 | University of California, San Francisco | San Francisco, CA |
| 7 | GIBBS, RICHARD A. | 5 | \$28,254,568 | Baylor College of Medicine | Houston, TX |
| 8 | ADAMSON, PETER C. | 3 | \$27,640,744 | Children's Hospital of Philadelphia | Philadelphia, PA |
| 9 | LACHIN, JOHN M. | 2 | \$26,786,559 | George Washington University | Washington, DC |
| 10 | SCHREIBER, STUART L. | 6 | \$24,238,609 | Broad Institute of Harvard and MIT | Cambridge, MA |
| 11 | NADLER, LEE MARSHALL | 3 | \$23,696,386 | Dana-Farber Cancer Institute | Boston, MA |
| 12 | HAYNES, BARTON F. | 1 | \$22,685,614 | Duke University medical Center | Durham, NC |
| 13 | CEFALLU, WILLIAM T. | 2 | \$21,402,305 | LSU School of Medicine | New Orleans, LA |
| 14 | EL-SADR, WAFAA M. | 5 | \$21,397,883 | Columbia University | New York, NY |
| 15 | SUNDARAM, UMA SUNDARAM | 2 | \$19,894,195 | West Virginia University | Morgantown, WV |

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For more information on membership, contact Jon Allard at 617-674-5100 or jon.allard@massbio.org.

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POLICY UPDATE

BY JOHN HEFFERNAN

In March, MassBio announced that our Chairman of the Board of Directors, Geoff MacKay, was named to the Health Policy Commission (HPC) Advisory Council. In his new role, Geoff Mackay will help guide the Health Policy Commission's implementation of the state's health care cost containment law. The Council will advise the HPC's overall operations and policies and provide feedback on a grant program to support new system delivery and payment reform methods. We look forward to our Chairman's new role as he continues to bring insight and an industry voice to the health care reform implementation process.

On March 12 and 13, MassBio participated in BIO Fly-In in Washington, D.C., where biotechnology executives from across the country met to take part in legislative briefings and advocacy. MassBio had a strong presence at the event, meeting with members of Congress and their staff to address important legislative issues facing our industry. One highlight from BIO Fly-In was MassBio's Spring Reception event, where members of the Massachusetts Congressional Delegation and staff met with biotech leaders from the Commonwealth to discuss the current state and future of our industry. The reception proved to be a successful event and we would like thank everyone who attended for participating in such an important opportunity for our industry.

The Massachusetts Biotech Caucus has also been very busy this year. To kick off the legislative session, Senator Karen Spilka and Representative Joseph Wagner, co-chairs of the Biotech Caucus, held a Caucus event at the State House on Influenza Vaccination and Public Health. In the wake of the influenza outbreak this past winter, the event provided a timely review of the current vaccination options and a chance to discuss innovative public health vaccination initiatives and steps that can be taken to improve public health in the Commonwealth.

In March, AstraZeneca welcomed members of the Biotech Caucus to their R&D Facility at Gatehouse Park in Waltham, their headquarters for global infectious disease and oncology research. In April, the Biotech Caucus continued its tour of the AstraZeneca facilities when it visited the Westborough manufacturing facility. Biotech Caucus events continue to be great learning opportunities for legislators and their staff, and MassBio looks forward to future Caucus events to come.

Q&A WITH SEN. ELIZABETH WARREN

Q Congratulations on your appointments to the Senate Health, Education, Labor and Pensions (HELP) Committee, the Banking, Housing, and Urban Affairs Committee, and the Senate Special Committee on Aging. As you delve deeper into your Committee work, would you share with us a few of your priorities for the coming months?

A My top priority is working to build a future—a future for ourselves, our children and our grandchildren. I think that's about making the right investments, about investing in education, infrastructure and research. I also believe we build a stronger field when we have a level playing field, when small businesses can compete against big businesses, and everyone who works hard and plays by the rules has a fair shot at success. I'm looking for every opportunity I can find to advance those goals.

Q The budget sequester and dramatic cuts to domestic spending present serious threats to our nation's ability to support cutting-edge research, bring new innovative therapies to market, and ensure patients have access to the treatments they need. How do we get Washington to understand that such cuts can have serious and long-term consequences for our nation's health and economic vitality?

A Research is our future. Research is the foundation for innovation and for economic growth. The breakthroughs of today – in science, technology, and engineering – lead to the success stories of tomorrow: discoveries that cure disease; innovations that reduce the cost of power; prosthetic limbs for wounded warriors.

I'm deeply proud to go to Washington to represent Massachusetts because we are leading the world in the research that produces new products and new industries - and creates the demand for new jobs at every stage. We are showing how investments in basic research and infrastructure create jobs and grow our economy.

We can reduce our deficit and balance our budget in a balanced, sensible way by closing corporate tax loopholes, cutting agricultural subsidies, and finding strategic savings in our defense budget. Instead, we threaten our own future with mindless, across-the-board budget cuts. I will continue to fight for our priorities, to fight for the investments that will help us build a future here in the Commonwealth and across the country.

Q We often hear about the contentious political climate in Washington these days, and many observers lament the difficulty our political leaders have in making progress on important national issues. In your first several months as our Senator, what have you seen that presents cause for hope – for the life sciences and biotech communities and, more generally, for the American people?

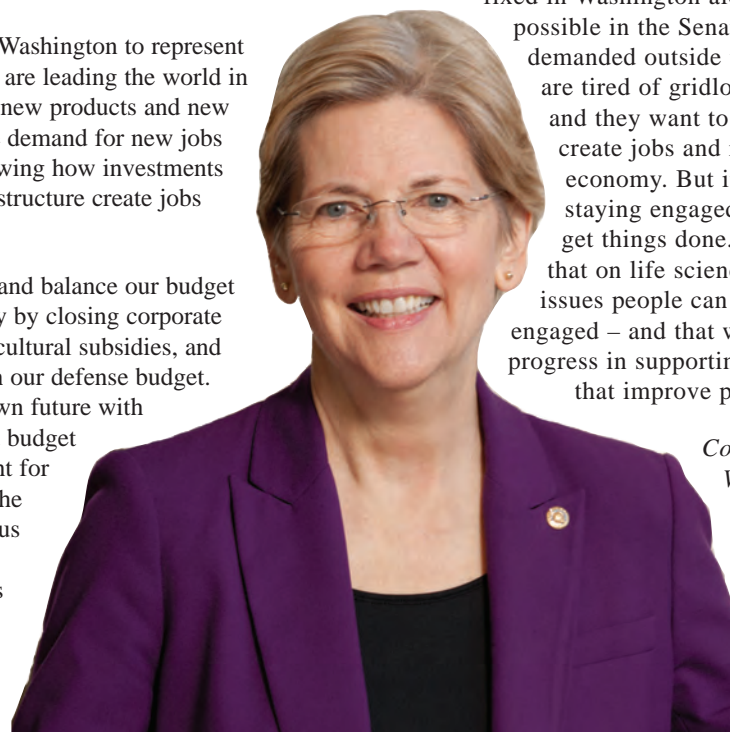
A I went to Washington to try to get something done for the people of Massachusetts. While there is a minority of the minority in the Senate focused on playing hostage politics and blocking legislation, I believe there are opportunities to make real progress on important issues.

During my first few months as Senator, I have cosponsored bipartisan legislation to jumpstart reform of Fannie Mae and Freddie Mac, met with Republican Senators who are interested in working together to end Too Big to Fail, and passed an amendment with Senator Lisa Murkowski (R-AK) to support critical disaster assistance for fisherman. It's not as much bipartisanship as I want, but it's a good start.

Support for the life sciences and biotech communities – and particularly for scientific and medical research – is widespread. People all around this country understand that innovations and breakthroughs in these areas will improve the lives of families and promote economic growth.

But make no mistake – these problems won't be fixed in Washington alone. Change is possible in the Senate when change is demanded outside the Senate. People are tired of gridlock in Congress, and they want to see us working to create jobs and rebuild our economy. But it will take people staying engaged in the process to get things done. I am optimistic that on life sciences and biotech issues people can and will stay engaged – and that we can make progress in supporting breakthroughs that improve people's lives.

Contact Sen. Elizabeth Warren at 617-565-3170 or visit www.warren.senate.gov.



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“GOLDEN AGE” FOR RESEARCH

NEARLY 400 INDUSTRY LEADERS GATHER FOR 2013 ANNUAL MEETING

By Meaghan Casey

Declaring it a golden age for biomedical research, FDA Commissioner Margaret Hamburg appealed to the nearly 400 biotechnology industry leaders gathered for MassBio’s 2013 Annual Meeting for an increase in collaboration, noting that more progress needs to be made to effectively translate scientific discoveries into therapies, prevention or cures.

“As a nation, we must take a serious look at everything from intellectual property and patent policy to economic policy, including such things as access to capital, incentives, and tax policy, how we invest in science, our reimbursement policies and, of course, regulatory policy and regulatory reform,” said Hamburg, who served as a keynote speaker at the two-day event held at the Royal Sonesta Hotel in March. “As a result of our dialogues with industry – including MassBio – we have heard loud and clear that you want increased clarity, certainty and predictability about the FDA’s standards and expectations. I think I can say that we are striving to do our part. Success will require regulatory flexibility, advancing regulatory science and true collaboration among health professionals, industry, government, academia, and our global health partners.”

Hamburg shared examples of Massachusetts companies who have successfully navigated approval pathways, including the new expedited approval pathway. Of the 47 novel drugs and biologics the FDA approved in 2012 – the most in more than a decade – one out of three received fast track designation, 41 percent received priority review, and 10 percent were approved under the accelerated approval program. Those developed in Massachusetts included: Iclusig, which treats two rare forms of leukemia and was approved in just 2.6 months; the orphan drug Juxtapid, approved based on a single clinical trial of 29 patients; Linzess, a drug for irritable bowel syndrome; and Kalydeco, the cystic fibrosis drug, which was approved in 3.5 months.

“The FDA was able to review and approve many of these new therapies quickly by communicating with drug companies early in the development process, allowing flexible clinical trial design, and using priority review and other drug approval mechanisms to expedite approval,” said Hamburg. “As a result, the majority of these important products were made available to patients

in the U.S. earlier than anywhere else in the world.”

John Crowley, Chairman & CEO of Amicus Therapeutics, also served as a keynote speaker at the annual meeting, sharing his experiences as a biotech executive, father and patient advocate.

“So much of what we do is about selling hope – not just hope for everyone who is invested today, but hope for so many people who don’t even know that they need it, that they’re going to be sick and that their lives are going to profoundly change,” said Crowley.

Crowley and his wife Aileen’s lives changed abruptly in 1998 when they were told that their daughter Megan (then 15 months old) and Patrick (just a few days old) had a rare genetic disorder, Pompe disease. Crowley left his job at Bristol-Myers Squibb and invested himself and his life savings in Novazyme Pharmaceuticals, a start-up company that was conducting research on a new experimental treatment for the disease. In one year, Novazyme went from an endowment of \$37,000 to \$27 million and was quickly acquired by Genzyme. Crowley was named head of Genzyme’s global Pompe program. He left the company in 2002 and in January 2003, Megan and Patrick received the enzyme replacement therapy through an experimental trial, which Crowley credits with saving his children’s lives. Megan is now 16 and Patrick is 15.

“Much of what we do is about extending and enhancing life – making that hourglass a lot bigger,” said Crowley. “As humans, all we really have and all that we’re pursuing is time. It’s essential to keep taking risk, if in the next 20 years we want to deliver the dream of all these early-stage ideas.”

The annual meeting also included discussions on healthcare reimbursement, what’s next in oncology research, the future of biosimilars, rare disease therapies and research resources sharing.

“These conversations go to the very heart of what we do and how we as an industry will operate into the future,” said MassBio President & CEO Robert K. Coughlin. “We must understand our role in a shifting economic and regulatory environment and attempt to answer these big, complicated questions. Our only way forward is to address them together.”

Josef von Rickenbach, Founder, CEO and Chairman of PAREXEL

International, received the Henri A. Termeer Innovative Leadership Award for his commitment and contributions to the biotechnology industry at the closing awards luncheon. Von Rickenbach co-founded PAREXEL as a regulatory consulting company in 1982 and soon grew the organization into a pioneer in clinical research outsourcing. To make clinical development more effective for clients, he conceived a multidisciplinary and systems-oriented approach to outsourced clinical development, bringing efficiencies, scalability, and standardization to the process. Thirty years later, PAREXEL is a \$1.4 billion global company and one of the top biopharmaceutical service organizations in the world.

Gloucester High School was named this year’s Joshua Boger Innovative School of the Year by the Massachusetts Biotechnology Education Foundation (MassBioEd) for inspiring students to explore life sciences careers through progressive biotechnology education and exemplary science career programming. In 2009, Gloucester High was selected as a BioTeach school, and in four years, students and faculty have actively participated in both teacher professional development and biotechnology lab experiences. The school has also creatively adapted BioTeach labs and equipment to establish new biotech summer camp programs for high school students.

“Gloucester High School has done a remarkable job fostering STEM education and encouraging students to pursue careers in biotechnology as they look toward their futures,” said Lance Hartford, Executive Director of MassBioEd.

“This recognition is an important step forward for Gloucester High School, as we work towards our goal of becoming a leader in STEM-related high school education,” said Principal Erik Anderson.

To recognize its commitment to growing the life sciences supercluster in Massachusetts, Massachusetts Biomedical Initiatives (MBI) of Worcester was honored with MassBio’s Leading Impact Award. MBI is the second organization to receive this award. Last year’s winner was Pfizer’s Center for Therapeutic Innovation.



MassBio President and CEO Robert K. Coughlin welcomes visitors to Lifecycle Massachusetts.

MASSACHUSETTS TAKES THE STAGE IN CHICAGO

MassBio was thrilled to coordinate the Massachusetts presence at the BIO International Convention in Chicago this year. We brought back Lifecycle Massachusetts, a Pavilion design that debuted in Boston last year, and focused on the patients who drive the research and development taking place across the Commonwealth.

We had a great few days—meeting with colleagues from around the country and the world, talking about the depth and breadth of the life sciences supercluster in Massachusetts. Some highlights:

- ♦ The announcement of a new International Collaborative Industry Program (ICIP) with Victoria, Australia, Wallonia, Belgium, Quebec, Canada and Alsace, France. The Massachusetts Life Sciences Center seeks collaborative projects in late stage R&D that are comprised of a Mass. company and an international partner. Such collaborations are eligible for grants of \$100,000 to \$500,000. For more information on the program, visit www.masslifesciences.com.
- ♦ Our annual Celebrate the Patient panel discussion, where

we hear from patients on how the industry impacts their lives. Thanks to Deb Beaudette, a Pompe patient, and Ron Banks, who lives with MS, for sharing their stories, and reminding us all why we do what we do.

- ♦ The Mass Mixer reception, hosted by Thermo Fisher, was another jam-packed networking sessions made even better with scallops wrapped in bacon from the SouthCoast Partnership and Sam Adams beer from the Boston Beer Company.
- ♦ Industry panels including a discussion on the activities of the Neuroscience Consortium announced at BIO 2012, a showcase of the incubator capacity in Massachusetts and a conversation on how incubators contribute to the ecosystem, and a panel on the future of biomanufacturing in Massachusetts and beyond.

We thank everyone who stopped by to join in a program or to say hello. We will begin planning for BIO 2014 in San Diego very soon, so please let us know if you have feedback or would like to be involved.



Josef von Rickenbach, CEO of PAREXEL International received the Henri A. Termeer Innovative Leadership Award.



MassBio Board Chairman Geoff MacKay, President and CEO of Organogenesis Inc., delivered the State of MassBio address at the meeting.



Gloucester High students Morey Ronan and Jason Erwin accepted the Joshua Boger Innovative School of the Year Award from MassBio President and CEO Robert K. Coughlin, far left, and MassBioEd Executive Director Lance Hartford. Also pictured: Gloucester High teacher Eric Leigh and Josef von Rickenbach.

LIFECYCLE MASSACHUSETTS PAVILION SPONSORS



MASSBIOED LAUNCHES NEW WEBSITE WWW.MASSBIOED.ORG



The brand new MassBioEd.org will act as the go-to resource for Massachusetts educators, students, postdocs, and life sciences industry professionals, providing career information, resources for high school science teachers, and avenues for biotech professionals to volunteer with students interested in the life sciences.

“MassBioEd is a proud leader in STEM education, but over time we realized our website could no longer serve the diverse and ever-evolving needs of the STEM community” said Lance Hartford, Executive Director of the Foundation. “We are pleased to unveil our new site which provides top-notch STEM and biotech resources and complements our programming and events.”

MassBioEd.org, relaunched on March 20, offers a comprehensive, user-friendly experience for students and STEM educators as well as a variety of resources for postdocs and industry professionals.

For postdocs, MassBioEd offers the chance to explore career paths and opportunities in the life sciences industry. The postdoc section of the new website details programs such as:

- ◆ Industry Exploration Program: career exploration, site visits, and job shadowing opportunities outside of academia;
- ◆ Futures in Life Sciences Program: a joint initiative of MassBio, MassBioEd, and Propel Careers to bring together industry experts across a wide range of disciplines for panel discussions and networking; and
- ◆ Additional networking events.

For industry professionals, MassBioEd offers extensive professional development opportunities through the Biotech Learning Center. MassBioEd.org’s Corporate Professional Development section features current course listings in a range of topics such as Project Management, Presentation Skills, and Biotech 101 for Non-Scientists. MassBio members receive a \$150 discount and MassBioEd can bring tailored courses to company offices.

Through new MassBioEd.org features, MassBioEd also offers the opportunity for industry professionals to give back by connecting with and inspiring future scientists. The Corporate Involvement section of the new website allows current professionals to get involved by volunteering to be a speaker at a local school, hosting students for Job Shadow Day or site visits, and supporting a variety of other MassBioEd events and programs.

Alongside the new website, MassBioEd has launched a strong presence on social media. MassBioEd’s Twitter and Facebook pages serve as resources for STEM education stakeholders across the country.

MassBioEd invites life sciences industry members to explore the new website and encourages STEM stakeholders to “like” MassBioEd on Facebook and “follow” @MassBioEd on Twitter.

NEW FORUMS ADDRESS HOT TOPICS, TRENDS

At MassBio, we strive for excellence in meeting the varied needs of our diverse and vibrant community by providing the best, timeliest, most forward-looking and forward-thinking programming possible.

Check out our new Forums, programs planned by MassBio members for MassBio members to address hot topics, industry trends and emerging technologies and ideas.

All Forum events are free of charge to MassBio members and include time for networking among the membership. For members who can’t make it to Cambridge, Forum events are streamed live via our website, and available afterward for on-demand replay.

Each Forum is planned by our Forum Working Groups in partnership with the Forum Advisory Board.

The Roster of Forum Working Groups:

- ◆ Biostatistics / Data Management / Clinical Trials
- ◆ Business Development & Finance
- ◆ MA CRO/CMO
- ◆ Drug Discovery
- ◆ Entrepreneur’s University
- ◆ Formulation and Drug Delivery
- ◆ Human Resources
- ◆ Legal & Regulatory
- ◆ Marketing & Communications
- ◆ Safety, Environmental, Facility Operations



New Forum Series Kicks Off in June

Adventures in Bioteching: Stories of Success (and Failure): A six-part series of programs on the new frontier of drug discovery and development in a time of changing business models, limited resources and regulatory uncertainty. Register online at massbio.org.

June 13, 2013

- ◆ Dreams to Reality: Case Studies, Successes

June 27, 2013

- ◆ Financing the Dream: Avenues for Raising Capital

September 2013

- ◆ Executing the Dream I: Strategy & Models for Company Growth
- ◆ Executing the Dream II: Science Tactics for Success

October 2013

- ◆ Selling the Dream: Partners, Payors and Patients
- ◆ Lessons Learned: Nightmares?

All Forums are open free of charge to MassBio members. The Adventures in Bioteching Series is open to nonmembers for a fee of \$25 per session. For more information, or to submit a topic of interest for a Forum, contact Susan Silberman at forums@massbio.org or 617-674-5100.

MA CRO/CMO GATEWAY TAKES OFF

Thirty-five MassBio member CROs and CMOs are now featured on the new MA CRO/CMO Gateway, an online portal created by MassBio and OnDeckBiotech to provide access to our unparalleled community of contract service providers.

The Gateway is seeing visits from between 50-100 unique visitors each day, presumably people seeking contract services, said Cliff Culver, CEO of OnDeckBiotech. It is at the core of a MassBio initiative to better connect life sciences companies with the broad spectrum of contract service providers in Massachusetts, and to ensure efficient access to resources that accelerate development timelines and reduce costs.

A 2011 analysis by Tufts Center for the Study of Drug Development (Tufts CSDD) found a nearly four-fold increase in the number of contract research organizations (CROs) in the U.S. alone during the past decade. The market for contract services in the Greater Boston area alone has reached between \$3.6 billion and \$4.3 billion. There are 126 companies in Massachusetts providing contract



MA CRO/CMO Symposium participants had the opportunity to preview the CRO/CMO Gateway and fill out company profiles at the event.

non-clinical research, and 68 offering clinical research. Another 111 companies are providing CMC services for clients around the world.

The Gateway was launched at the second annual MA CRO/CMO Symposium: Accelerating Drug Development which brought together 400 attendees from the biopharmaceutical industry and the Massachusetts CRO/CMO cluster. The program included a fireside chat on financing trends in the industry, and panel discussions on Recent Approaches in Sourcing, Discovery Through IND-Enabling Studies, IND to Commercial Manufacturing, Early Formulation Development & CMC, and Trends Towards & Best Practices for Use of Virtual Resources. The bustling exhibit hall featured 43 local CRO or CMO organizations.

Planning for the 2014 MA CRO/CMO Symposium is underway. For more information, contact Elizabeth Steele at elizabeth.steele@massbio.org or 617-674-5100.

To explore the Gateway, visit massbio.ondckbiotech.com.

MASSBIO INNOVATION SERVICES THRIVING

Just three years after the release of the 2015 Strategic Report, which identified the need to better align the research community with opportunities for commercialization, MassBio can report making significant headway. By introducing the MassCONNECT mentoring program and the popular Pharma Days, companies can capitalize on the state's position at the forefront the life sciences industry and leverage the power of the MassBio network to enhance collaboration and accelerate innovation.

Building on MassCONNECT Successes

MassBio Innovation Services' flagship program, MassCONNECT, is a unique mentoring program that connects entrepreneurs and founders with seasoned life sciences professionals to catalyze and commercialize innovation.

Since its inception in May 2010, MassCONNECT has graduated 40 companies. Despite an extremely challenging economic landscape, eight of those companies have already received funding following completion of the program.

"I see our experience with MassCONNECT as being a turning point in MindChild's development process," said

Adam Wolfberg, MD, chief medical officer of MindChild Medical, a MassCONNECT graduate from 2010.

"MindChild went from being a very early-stage, slightly naïve group of inventors and – I think because of the MassCONNECT program – turned into a startup company that is now very well-funded and well on its way to success."

Most recently, MassCONNECT graduate Johannes Fruehauf, PhD, used connections made through the MassCONNECT network to secure partners for LabCentral, a new shared lab space designed to facilitate the creation of startup biotech companies in Kendall Square. Through MassCONNECT, Johannes met Charles Wilson, Vice President of Strategic Alliances at the Novartis Institutes for BioMedical Research (NIBR), which later signed on as a founding partner of LabCentral.

Scaling up Pharma Days

In 2013, MassBio is will host more of the popular Pharma Days programs than ever before, building on successes reported from past events. Pharma Days match pharma companies with biotech companies through invitation-only

in-person meetings and events customized by MassBio. Pharma Days depart from traditional "speed-dating" formats and focus on tailored, optimal connections.

Pharma Days are designed to make partnering as smooth and productive as possible. For pharma companies, Pharma Days offer visibility and the chance to connect with biotechs in the number one life sciences cluster. For biotech companies, Pharma Days provide access to senior-level executives from pharma and critical information on pharma companies' plans and strategies.

With new CBO John Hallinan at the helm and positioned in the heart of the thriving Massachusetts life sciences ecosystem, the MassBio Pharma Days program is on its way to becoming the industry-leading partnering program. To date, eight of the top 10 pharma companies have held Pharma Days. With six Pharma Days confirmed for 2013 and many more in process, MassBio aims to have held Pharma Days with all of the top 10 pharma companies by the end of the year.

To learn more about MassCONNECT or Pharma Days visit www.massbio.org/innovation or contact Rakhshita Dhar at 617-674-5100 or rakhshita.dhar@massbio.org.



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**PATIENT
PROFILE**



CELEBRATING LIFE AND THE PATIENT

BY MEAGHAN CASEY

Diagnosed with Pompe disease as a young mother of three active children, Deb Beaudette was originally told she would never live long enough to meet her grandchildren. That was 20 years ago. Today, she has a granddaughter who is nearly 2 years old and is treasuring every moment she has.

“The disease doesn’t define who you are or what you can or cannot do,” said Beaudette. “You define who you are. You are in control of your quality of life. You can choose to feel sorry for yourself and sit quietly in a corner, or can choose to use the help and options around you and get up and enjoy everything before you can’t get up anymore.”

Affecting about one in every 40,000 people in the U.S., Pompe disease is a rare, inherited disorder that disables the heart and skeletal muscles. Beaudette began noticing symptoms such as fatigue and the inability to move her legs. She described the feeling as “having heavy weights on her feet.” As her symptoms progressed, her primary care doctor tested her muscle enzymes, which were in the

1,000s, when they should have been in the 60s. From there she was sent to UMass Memorial Medical Center in Worcester and began the nine-month process towards diagnosis.

Through a specialist’s recommended regimen of diet and metabolic exercise, she was able to maintain her mobility and care for her children.

“I told him death wasn’t an option for me,” said Beaudette. “He saved my life.”

In 2010, when Genzyme began its trial for a medication that replaces a missing or deficient enzyme in people with late-onset Pompe disease, Beaudette was eager to participate.

“I thought, why not be a guinea pig and give my life some meaning,” she said.

The clinical trial was a randomized double-blind study and Beaudette had a 50-percent of being a test subject. Fortunately for her, she received the drug, not the placebo. By the third month, she reports that she was climbing hills without needing to rest, her lung capacity improved tremendously and the feeling of weights in her feet was

gone.

Because of Genzyme’s trial – of which a total of 81 of 90 patients completed – Beaudette also discovered a network of other Pompe patients, who have made an effort to remain connected.

“Genzyme gave me a community,” said Beaudette, who continues to remain vocal as a patient rights advocate.

That sense of support has fueled Beaudette in her journey. She still maintains an active life, with the help of a walker, and credits the combination of water therapy and Genzyme’s enzyme replacement therapy, which she has continued.

“Don’t settle; don’t accept that you’re disabled,” she said. “If you want to learn something or do something, do it. You have to keep moving.”

Beaudette served as a panelist at the 2013 BIO International Convention. She joined multiple sclerosis patient Ron Banks and moderator Kathleen Coolidge, Associate Director, Patient Advocacy – Rare Diseases for Genzyme in the Massachusetts Pavilion for a welcome session entitled “Celebrate the Patient.”