It has been a banner year for MassCONNECT graduates, who have inked deals with biopharma company partners, won international business plan competitions and reached product milestones. These successes set the bar that much higher for the 2015 MassCONNECT cycles, for which applications are now being accepted.

MassCONNECT is MassBio’s mentoring program, matching budding entrepreneurs with industry experts for in-depth, industry-specific support and mentoring. It is the only program that dives deep into the life sciences industry, helping build new drug discovery, platform, diagnostic, medical device and healthcare IT companies.

Since the program’s creation in 2010, 50 entrepreneurs have graduated from MassCONNECT. Of these graduates, two have inked deals with big pharma or big biotech companies. Ten have raised funds from venture capitalists, angels and other non-dilutive sources of funding. The majority of graduates have made significant connections with big pharma and biotech through MassBio Pharma Days as well as connections with venture capital.

CEOs David Meeker of Genzyme, Steve Perrin of the ALS Therapy Development Institute, Michelle Dipp of OvaScience and Robert Forrester of Verastem participated in a panel discussion.
COMMITTED TO PUTTING PATIENTS FIRST

An important part of our mission at MassBio is to communicate the unmet needs of patients and enhance patient care through advocacy, education and programming efforts. Inside this edition, you will read about our first ever Patient Advocacy Summit, designed 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. Thank you to all the industry leaders who came together with patient advocates and other stakeholders for this successful event.

Also inside, you will be introduced to Blyth Lord, a patient advocate with a powerful voice in the rare disease community. Stories like Blyth’s make all of us realize just how precious every moment is in this industry, and inspire us to do everything we can to speed the development of new treatments and cures.

This fall, we were honored to host BioPharm America here in Boston for the fifth year and welcome our colleagues from around the world. The conference provided an ideal venue for industry stakeholders to make the connections they need to thrive and grow their businesses. Additionally, we hosted our 20th Annual Golf Classic, which raised significant funds to support the initiatives of the MassBioEd Foundation. Your participation and continued investment in the future will ensure that we continue to work together to bolster our industry’s position and fully articulate the impacts of legislative and regulatory proposals on the patients we serve.

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It was with great sadness that we said goodbye to Mayor Tom Menino in October. We will be forever grateful for all that he did for the City of Boston and for this industry during his long and successful tenure. He will be greatly missed.

As we look ahead to 2015, I encourage you to save the dates for our Policy Leadership Breakfast in January, Rare Disease Day in February, our Annual Meeting in March and of course BIO International Convention in June.

I would also like to congratulate newly elected Gov. Charlie Baker. We look forward to working with him to bolster our industry’s position and fully articulate the impacts of legislative and regulatory proposals on the patients we serve.

Robert K. Coughlin is President and CEO of MassBio.

NEW MEMBERS

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CATCHING PROJECT TIGER

At the end of September, Baxter, a leading provider of therapeutic treatments that save, sustain and improve the lives of people with rare conditions, chronic diseases or limited treatment options, announced that they will locate 400 jobs at a new Global Innovation and R&D Center in Cambridge. The new facility, eventually slated to reach 200,000 square feet, will begin operations later this year.

MassBio is thrilled to welcome Baxter to the Massachusetts supercluster, particularly since MassBio’s Director of Economic Development & Global Affairs Peter Abair worked with MassEcon, Deloitte and the Baxter site selection team as they made their decision.

MASSBIO WELCOMES NEWLY ELECTED OFFICIALS

On Nov. 4, the voters of Massachusetts elected Republican candidate Charlie Baker as Governor of the Commonwealth. When asked about our industry at the MassBio Gubernatorial Candidate Forum in October, Governor-Elect Baker said that life sciences is a critically important piece of the Massachusetts economy and that as governor, he would be an aggressive advocate for NIH funding and the competitive process as the right way to move innovation.

Governor-Elect Baker previously served as Secretary of Administration and Finance under Governors Weld and Cellucci, and is the former CEO of Harvard Pilgrim Health Care.

Governor-Elect Baker will be sworn-in to office on Thursday, January 8, 2015.

The winners in the race for the other constitutional offices are:
- Karyn Polito, Lieutenant Governor; Maura Healey, Attorney General; William Galvin, Secretary of the Commonwealth;
- Deb Goldberg, Treasurer; Suzanne Bump, Auditor.

At the federal level, the Massachusetts Congressional Delegation will have a new Congressman serving in the 6th Congressional District. Democrat and Iraq War veteran Seth Moulton defeated longtime Congressman John Tierney in the Democratic primary and then went on to beat Republican candidate Richard Tisei in the general election. Senator Edward Markey, and Representatives Richard Neal, James McGovern, Niki Tsongas, Joseph Kennedy, Katherine Clark, Michael Capuano, Stephen Lynch and William Keating all retained their seats.


KEN MOCHE, former president and CEO at Chimerix, was a keynote speaker. "Operationally, for us, that means every decision we make is one that benefits society and promotes quality research, while avoiding unnecessary delays. "Patient-centric care is a giant step in the right direction," Moch said. "They require the development of a synergistic research culture that's focused on improving outcomes for patients." 


The day-long event, held at Ginseng Center, was the first MassBio Patient Advocacy Summit. Opening the summit, Nancy Roach, founder and chair of Fight Colorectal Cancer, stressed the need to do clinical trials that benefit society and promote quality research, while avoiding unnecessary delays. "Patient-centric care is a giant step in the right direction," Roach said. "They require the development of a synergistic research culture that's focused on improving outcomes for patients."

Ginseng President and CEO David Meeker moderated a panel that examined how companies can integrate patient voices into their operations. "We have to be thinking through the lens of the patient, every day," Meeker said. "Michelle Dipp, CEO and co-founder of Dendreon. "Operationally, for us, that means every decision, enrollment, press release, marketing material and event is reviewed with that in mind. It think you need someone in your company— a patient advocate—who has the same job function to remind everyone else to put the patient first."

"Improving patient care is at the core of what we aim to accomplish," said Robert Fendeur, President and CEO of Verastem. "Engaging with patients and patient advocates early in the development process is critical to fully understanding the unique needs that exist with currently available therapies. Part of our job is also to find out how to extend a patient’s quality of life—whether that's through a symptom or improving transportation to appointments—and that's what we can get out of those conversations. Every patient has his or her own personal story, and we can learn something from every one."

Another panel examined the gray areas in the interaction between companies and patients along the drug-development continuum. "We want to develop and market drugs. The more important, we want patients to have a good experience with those drugs," said Liz Lewis, Chief Counsel and Chief Compliance Officer at Millennium. The Miller Oncology Company. "Together, we want to come up with a product and administer it in a way that’s most beneficial to the patient. Industry supports advocacy groups but what are the rules to collaborating appropriately and ethically when there are federal restrictions on sharing information?"

John Richert, Vice President of Global Medical Affairs, MS Franchises, at Biogen Idec also talked about new concerns with liability. "Becoming a breach in patient-company relationships. "When we're talking about drugs in a pipeline, that could be taken as pejorative or sensational," he said. Sue Kahn, Executive Director of National Tay-Sachs and Allied D iseases Association, added if it's clear, continued relationship could be developed without risking the program: "Something could go out in a tweet that shouldn't and that private heart is broken," said Jerry Moscicki, Community Manager at the Lifly Clinical Open Innovation Team, raising concerns about social media.

In his keynote address, Ken Moch, former president and CEO of Chimerix, discussed just how powerful a tool social media can be. In March, Chimerix was bombarded with media demands to give the company's anti- viral drug to a 5-year-old Josh Hardy, who had developed a viral infection during immuno-suppression following a bone marrow transplant. Chimerix had discontinued its compassionate use program in order to gain approval for market use as soon as possible, and the drug was still in phase 3 trials. In four days a hashtag was trending in the top 1 on Twitter and the company’s Facebook page had 1.3 million views.

"It’s an ethical dilemma," said Moch. "Do you use the life in risk in front of you or do you continue on the path to approval to save as many lives as possible? It’s a difficult, painful position to be in."

"Chimerix announced it would offer an open-label, 28-person study beginning with Hardy. One month after the first dose, Hardy was clear of the virus. "But what if it hadn't worked out?" asked Moch. "What if the medical outcome wasn't as positive for Josh? Would that have been the end of a drug that could have worked for thousands of others? Compassionate use is not drug development. There are complexities and risks that go along with it."

The event further tackled the issue of getting to, and through, the FDA approval process. "I’ve lived in the rare disease world— on the development side— for 30 years, and there’s great disparity in that world," said Norman Burton, President, Vice President of Clinical Development at Shire. "There’s a negative outcome that doesn’t work but that’s a need for research that was talking about transportation and the flip side of bringing clinical trials to patients, rather than having them travel to designated sites.

In addition to the panel discussions, the event featured four different case studies. In one, Christine McSherry, who founded the Jett Foundation when her son was diagnosed with Duchenne muscular dystrophy, joined Diane Berry, vice president of Global Public Health and Government Affairs at Sarepta Therapeutics, to talk about the Duchenne Alliance. Nearly 40 different foundations united to form the Duchenne Alliance, which has raised more than $9.5 million towards advancing the top biomarker research and clinical trials.

"I realized a lot of other parents were doing the same thing, and had the same goal— eventually our kids to live longer—but we were working in silos," said Moch. "Now we can co-identify, co-fund projects." McSherry has been advocating for FDA approval of Sempax's therapy, empagliflozin, and hopes for its own, Jett, turned 19 in October, will start the trial shortly.

"Using the umbrella organization, we’re able to collectively build a threm that’s loud enough to affect change," said McSherry. "It’s a chance to get this process that’s in the hands of the patients and families themselves," said Berry.

"I can’t think of a better voice to convey the urgency than the patient advocate," Moch said. "Something could go out on a tweet that shouldn’t and that private heart is broken," said Jerry Moscicki, Community Manager at the Lifly Clinical Open Innovation Team, raising concerns about social media. "I realized a lot of other parents were doing the same thing, and had the same goal— eventually our kids to live longer—but we were working in silos," said Moch. "Now we can co-identify, co-fund projects." McSherry has been advocating for FDA approval of Sempax's therapy, empagliflozin, and hopes for its own, Jett, turned 19 in October, will start the trial shortly.

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PREPARING THE STATE’S NEXT GENERATION OF BIOTECHNOLOGY LEADERS

Students benefit from spending a day exploring a local life sciences company

MassBioEd’s Career Exploration Days provide high school students with an opportunity to experience a tangible connection between the STEM curriculum they see in their classrooms and the groundbreaking scientific innovation that drives the Massachusetts life sciences supercluster by spending the day at a life sciences company. Part of MassBioEd’s BioTeach program, Career Exploration Days aim to increase students’ interest in pursuing science, technology, engineering and math (STEM) related careers.

Career Exploration Days are instrumental in exposing students to the numerous and diverse career opportunities that exist in the life sciences – on the scientific side and business side – and in encouraging students to pursue a college education that includes a science focus. Hosting a Career Exploration Day provides about 30 students the chance to explore your work environment and corporate culture, meet your employees, and learn how your company is changing the lives of patients.

AbbVie, Bristol-Myers Squibb, Cubist, Foundation Medicine, Genzyme, ImmunoGen, Leica Biosystems & Beckman Coulter Genomics, Massachusetts General Hospital, Pfizer Cambridge, and Pfizer Andover have hosted or will be hosting Career Exploration Days this academic year. MassBioEd is seeking additional host companies so that more students from across the Commonwealth can learn more about careers in the life sciences and be inspired to pursue them.

Please email Larissa.Fawknor@massbio.org if you are interested in learning more or hosting a Career Exploration Day in the 2014-2015 school year.

THANK YOU, MAYOR MENINO

MassBio is very saddened to hear of the loss of Mayor Thomas M. Menino. Mayor Menino was a steadfast supporter of the life sciences, in Boston and in Massachusetts, and he will be missed.

“There’s no doubt that Mayor Menino is one of the reasons Massachusetts has become the best place in the world for healthcare and life sciences,” said Robert K. Coughlin, President & CEO of MassBio. “His vision and commitment to innovation, most obvious in the now-booming Innovation District, wasn’t only an economic or planning initiative. It was also meant to harness the resources and talent in Boston for the benefit of sick people, the patient population, around the world. That’s what he cared about, people. We are truly grateful for his vision and leadership, and our thoughts are with his family at this time.”

‘FORE’ A GOOD CAUSE


Event sponsors Walter Shaw from Janitronics Building Services (third from left) and Tony Greico from Airgas (far right), and MassBio’s Robert K. Coughlin (second from right) announced the Nicklaus Course winning foursome from Stericycle Inc. including (from far left) Terry Helmetag, John Jepsen, Paul Jepsen, and Rick Salomonsen.
**Q & A**

**WITH DR. DAVID MEEKER**

President and Chief Executive Officer of Genzyme

**Q** Can you tell us a little bit about Genzyme’s philosophy and approach to working with patients? How has it changed over the years?

**A** Our core mission and business model is based on addressing unmet needs on behalf of patients living with rare and debilitating diseases. Our story as a company is Brian’s story of facing Gaucher Disease as a child. When we first met Brian and his family, he was a young child facing a diagnosis for a progressive, debilitating disease for which there was no treatment. More than two decades later, Brian is a married father of four, not defined by his disease and living a full life. Or Dexter’s story, having to stop playing with his friends in the yard because of the burning sensation in his hands from Fabry disease. Dexter is now a teenager receiving treatment, and continues to serve as a source of motivation for the team at Genzyme that manufactures his therapy.

Our patient advocacy team has been in place for almost 15 years. Their sole mission is to support patient advocates and communities to be more effective while helping to make a meaningful difference in patients’ lives. That really summarizes our approach — it’s been how we conduct our business from the earliest of days and is just as critical now as it was then. The external landscape has become more complex, with increased scrutiny and regulations, but the importance of mutually beneficial, transparent relationships with patient advocates has not changed in our minds.

**Q** The majority of MassBio member companies are small and early-stage. As they consider where to put their limited resources, what’s the business case for patient advocacy?

**A** It’s never “too early” to invest in patient advocacy. In general, many companies still struggle to understand what having an advocacy focus looks like in practice. It is less about the dollars and more about the time investment of people building relationships with advocates and soliciting feedback. We don’t question whether we would invest in relationships with physicians or payors so why should we think any differently about investing in patient advocacy? Patients are the ultimate customer, the ones who know their disease better than anyone, and the relationships with advocacy groups have critical impact all along the drug development pathway.

**Q** How do you see the role of patients in drug discovery, development and commercialization cycles shifting in the future?

**A** The influence patients have has dramatically changed in the last 5-10 years, and there’s no reason to think this will not continue to increase. I believe it will become commonplace to have patients with a seat at the table throughout the process of developing treatments, engaging in clinical development, and play a greater role in access for therapies — and I believe is SHOULD be commonplace.

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**MASSCONNECT GRADUATES CELEBRATE SUCCESS**

**MASSCONNECT:** from Page 1

capitalists, angel investors, crowdfunding platforms, national institutions, and other startup programs.

In May, Resilience Pharmaceuticals, a 2014 MassCONNECT graduate developing therapies for post-traumatic stress disorder (PTSD), won the $150,000 OneStart Grand Prize organized by SR One and the Oxbridge Biotech Roundtable. Retsina Meyer, Resilience Founder and Interim CSO, says her MassCONNECT experience has contributed to the company’s success, by helping the company hone their go-to-market strategy and connecting her to advisors, mentors and teammates.

“We look at our first deck and our last, and our learnings from MassCONNECT are on every page,” she said.

After graduating MassCONNECT in early 2014, the team behind Aldatu Biosciences won the Harvard Dean’s Health & Life Sciences Challenge Bertarelli Foundation Grand Prize ($40K) and were selected to compete in MassChallenge 2014. Aldatu is developing diagnostic tools to improve HIV disease monitoring. “Without a doubt the guidance and feedback our mentors provided through the MassCONNECT program facilitated these successes. The evolution of our plans and pitch over the course of the program is undeniable, and it is these matured materials that we used to achieve these honors. We are exceedingly grateful for the opportunity to join MassCONNECT,” said Aldatu’s David Raiser.

MassCONNECT attracts the best ideas and entrepreneurs by sourcing mentees through partnerships with leading academic institutions and medical centers, as well as other accelerator and business plan programs. Nancy Wetherbee, the head of Industry Translation at Tufts Medical Center, says MassCONNECT is “one of the essential tools in the translational toolbox.”

“MassCONNECT’s mentoring program for our clinician entrepreneurs is the top mentoring program in a crowded sea of programs vying for innovation,” she said. “What makes MassCONNECT unique is the unparalleled investment of time from top talent whose unique insight unlocks commercial value for impactful results. All of Tufts Medical Center’s teams have emerged stronger both financially and strategically as a result of our MassCONNECT experience.”

Applications are now being accepted for 2015 MassCONNECT cycles. Sponsorship of the MassCONNECT program, which introduces sponsors to emerging entrepreneurs, under the radar technologies and the constant refresh of innovation that occurs in the top life science cluster, is also available. For more information, contact John Hallinan or Rakshita Dhar at 617-674-5100, john.hallinan@massbio.org or rakshita.dhar@massbio.org.

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**PCT PARTNERSHIPS IN CLINICAL TRIALS**

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PERSONAL TRAGEDY CALLS PARENTS TO ACTION

BY MEAGHAN CASEY

Fifteen years ago, when cousins Cameron and Hayden Lord were diagnosed with infantile Tay-Sachs disease within three weeks of each other, the prognosis was devastating: no treatment, no cure.

Cameron was 6-months-old. Hayden was 18-months-old.

“The diagnosis of infantile Tay-Sachs was a death sentence,” said Cameron’s mother, Blyth Lord. “Little was happening in research and we knew there was absolutely no hope in the babies’ lifetimes.”

A rare genetic disorder caused by the absence of beta-hexosaminidase (Hex-A), Tay-Sachs disease progressively destroys nerve cells in the brain and spinal cord. As the disease progresses, affected children experience seizures, vision and hearing loss, intellectual disability and paralysis.

Because Tay-Sachs is an autosomal recessive disorder, both parents must be carriers for a child to be affected. Lord carried the Ashkenazi Jewish mutation. Her husband, Charlie, and his identical twin, Tim, had a mutation that had never been seen before, and Tim’s wife, Alison, carried what is increasingly recognized as an Irish mutation.

“The likelihood of these two brothers marrying carriers and having affected children was a statistical near-impossibility, but it happened: two brothers, two wives and two babies affected with a rare disease who would both die before their third birthdays,” said Lord.

Hayden died in December 2000 and Cameron died five months later, four days after her second birthday. In the months that followed, the four parents and six grandparents decided to start a foundation in memory of the children. Their goal was to decrease the incidence of lysosomal storage diseases, like Tay-Sachs, and leukodystrophies through medical research, and to increase the quality of emotional, spiritual and medical care for families facing these diseases.

“It’s common, as part of the bereavement process, to want to do something to establish a child’s legacy,” said Lord. “We were not different. As we moved through our grief, we wanted to do something in their name. We also believed something more had to come of their lives — and the extreme experience we had as a family. In some ways, it felt like a calling.”

Launched in 2001, the Cameron and Hayden Lord Foundation funds medical research, pediatric palliative care research and programs, and bereavement support for children and their families.

“Figuring out how to approach funding medical research was the hardest piece for us,” said Lord, who serves as executive director of the foundation. She is also the founder and executive director of the Courageous Parents Network, an organization that provides parents with the skills, tools and virtual support they need to cope during their child’s illness.

“None of us had a science or research background and we struggled those first few years to release well-articulated RFPs that might make a difference and to then review and select promising proposals,” she continued. “Plus our annual grant for research, while impressive for a family foundation in its infancy, wasn’t big enough to pack much of a punch on its own. We needed a better way.”

The way presented itself in 2002 when the National Tay-Sachs and Allied Diseases Association (NTSAD) founded its Research Initiative to provide mezzanine funding to small-seed research projects. The Lord Foundation hitched its annual funding to the Research Initiative, and has renewed that partnership every year.

“The strategic benefits of pooling our annual medical research grants with NTSAD are many, the most important one being that it enables the foundation to make highly informed investments in research that can have bigger possible impact,” said Lord.

The promise of this strategy was affirmed several years later, when the Research Initiative helped launch the Tay-Sachs Gene Therapy Consortium — an international collaborative of scientists experienced in gene therapy and disease research. The consortium went on to receive $3.5 million from the NIH for translational research towards delivering the missing gene to the brain and has since shown excellent results in animal models and is driving toward Phase 1 human clinical trials.

Although there is still no treatment for Tay-Sachs or Sandhoff or Canavan’s disease, NTSAD is readying itself for that day by aligning itself with pharmaceutical companies such as Genzyme, Good Start, Shire, Agios and Audentes. The association recently formed a Corporate Advisory Council to bring in the biotech and pharmaceutical expertise necessary to help it identify these options, advance the research agenda, and position itself as an attractive partner to industry.

“It’s incredibly exciting to see the progress, and to know that our foundation has played a significant role in making it possible,” said Lord. “It’s surreal to be on the threshold of a treatment for a disease when it doesn’t feel like very long ago we were receiving the hopeless diagnosis for our children.”

Though it is bittersweet for those families, like the Lords, who did not or will not benefit in time, Lord is quick to point out the silver lining.

“In the Tay-Sachs community, parents really understand that it’s about the future, and that’s kind of beautiful,” she said. “Every new child becomes a kindred spirit and you would do anything to help. I’m grateful to live in an age where family stories and personal tragedy can find allies in industry and make the world a better place for those that follow.”