

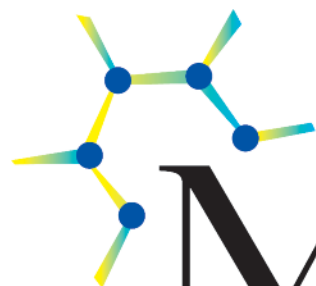


MASSACHUSETTS MARKS RARE DISEASE DAY Page 7



HOPE FOR FA PATIENTS Page 8

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MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

1985 **30** YEARS OF INNOVATION 2015

**HEALTHCARE REFORM ON THE TABLE
AT MASSBIO POLICY BREAKFAST**
PAGES 4-5

David Balekdjian, Glenn Batchelder, Eric Gascho, Kenneth Kaitin and David Seltz led a panel discussion. PHOTOS: SEAN BROWNE

House Speaker Robert DeLeo was honored at this year's MassBio Policy Leadership Breakfast.

MASSBIO ANNUAL MEETING WILL ADDRESS VALUE, PRECISION MEDICINE

The agenda is now available for the MassBio Annual Meeting, being held March 26-27 at the Royal Sonesta Hotel in Cambridge.

Patient and seasoned patient advocate Kathy Giusti, Founder of and Executive Chairman of the Multiple Myeloma Research Foundation (MMRF) and the Multiple Myeloma Research Consortium (MMRC), will open the meeting. MIT's Andrew Lo, one of TIME magazine's 100 most influential people and financial engineering expert, will close the meeting. In between: a dozen sessions, networking



KATHY GIUSTI



ANDREW LO

receptions and the annual awards luncheon.

This year, MassBio will honor Mark Levin, co-founder of Third Rock Ventures and an industry leader with 40 years of experience, with the Innovative Leadership Award.

The event features two conference-wide sessions, one focused on defining value and one on the future of precision medicine, and a number of breakout sessions in better business and trends in science tracks.

See **ANNUAL MEETING** Page 7

CELEBRATING 30 YEARS OF INNOVATIVE SUCCESS



ROBERT K. COUGHLIN

As MassBio celebrates 30 years this year, it's a perfect time to reflect on just how much this industry has grown since 1985. We know the success of biotechnology in Massachusetts is a direct result of innovation and talent of the scientists, venture capitalists and entrepreneurs researching, developing and manufacturing groundbreaking therapies and technologies that improve the lives of patients every day. Looking ahead to 2020, it is our job to ensure that innovation thrives and our biotechnology supercluster remains second to none.

This year, as always, MassBio will continue to advocate for the legislative initiatives and regulations that protect the incredible ideas that are born in the academic institutions and medical centers right here in

the Commonwealth. We will persist in supporting bipartisan efforts to increase NIH funding for basic research. We will also continue to bring you the events that you have come to expect and look forward to—events such as our Annual Meeting in March, and our Golf Classic, CRO/CMO Symposium and Patient Advocacy Summit this fall. I hope you can join us for some of these events, as well as the BIO International Convention in Philadelphia this June.

Thank you to all who attended our Policy Leadership Breakfast on Feb. 4. I appreciate everyone battling the snow-covered sidewalks and blocked-off sections of the Patriots parade route to be there. Our expert panel held a lively discussion on how to create a sustainable future for life sciences innovation so that we can continue to

develop and deliver treatments to patients around the world. I was also pleased to present the Honorable Robert DeLeo, Speaker of the House, with the award for the 2014 MassBio Legislator of the Year. I look forward to working with new members of the Mass. legislature, especially Gov. Charlie Baker, in the year ahead.

At the end of February, we celebrated Rare Disease Day and heard from a number of patients who rely on the innovation of this industry the most. Inside, you will also read about Jean Walsh, who has battled a rare disease for three decades. It is inspiring to hear her message of hope.

Thank you for your continued support. We look forward to what the next 30 years brings.

Robert K. Coughlin is President & CEO of MassBio.



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2014 MASSACHUSETTS IPOs

There were 17 IPOs for Massachusetts companies in 2014—almost double the amount of the previous record year of 2013.

Akebia THERAPEUTICS	\$100 M*	Histogenics	\$65 M
ALDEYRA THERAPEUTICS	\$12 M	MINERVA NEUROSCIENCES, INC.	\$32.7 M
CERULEAN Pharma Inc.	\$59.5 M	Ocular Therapeutics	\$65 M
CoNCERT Pharmaceuticals Inc.	\$84 M	PROTEON Therapeutics	\$61 M
Dicerna pharmaceuticals	\$90 M	Radius	\$52 M
ELEVEN BIOTHERAPEUTICS	\$90 M	SAGE THERAPEUTICS	\$90 M
flexion therapeutics	\$65 M	tōkai	\$98 M
genocea BIOSCIENCES	\$66 M	T2 Biosystems	\$57 M
		zafgen	\$96 M

*Figures refer to "offer amounts" (investment secured) at time of IPO
Source: Nasdaq.com, BioSpace

MARKING A MILESTONE

In 1985, Microsoft released the first version of Windows, a joint American-French expedition located the wreck of the RMS Titanic, and pop stars united to release 'We Are the World' to raise money to fight hunger in Africa.

Here in Massachusetts, our industry forefathers were coming together to launch the first-ever biotech trade association, the Massachusetts Biotechnology Council. Built from a small group Purchasing Consortium (which today does \$55 million on contract sales) to an organization offering networking, professional development, advocacy & policy support, economic

development and industry research, workforce development programs and special events, it has been a heck of a ride.

In celebration of our 30th anniversary, we at MassBio are collecting stories, pictures, mementos and anecdotes from the organization's early days. We'll incorporate all of this crowdsourced history into programs and events throughout 2015, starting with

our Annual Meeting March 26-27.

Do you have stories to share?
Contact Elizabeth Steele at elizabeth.steele@massbio.org or 617-674-5100.



NEW MASSBIO MEMBERS

Agilis Biotherapeutics
Allied Minds
Archer DX
BioGate Technologies
Biomedical Research Models
Clementia Pharma
CRELUX
Duke Clinical Research Institute
Emerald Green Building Services
Flex Pharma
Global Regulatory Partners
In Vitro ADMET Laboratories
Industrial Protection Products
Intellia Pharma
Jamestown Properties
Kendall Square Association
Mount Ida College
National Foundation for Cancer Research
Nuventra Pharma Sciences
Planet Nutshell
PureHoney Technologies
Restorgenex
Robins Kaplan LLS
Scilligence
Seattle Children's Research Institute
SeLux Diagnostics
Southern Research Institute
Surface Oncology
TriMetis Life Sciences
Wilson Therapeutics
Zafgen



CONNECT WITH US





Q & A WITH KEVIN LEE

**Chief Scientific Officer
Pfizer Rare Disease Research Unit**

Q Can you tell us about Pfizer's approach to rare disease drug discovery and development?

A Pfizer has a decade of experience and a portfolio of 22 approved medicines for a range of rare diseases that have made an impact on the lives of patients and caregivers. Today, our investigational programs in rare disease focus on hematology, neuromuscular and rare pulmonology, with experimental candidates for sickle cell disease, Duchenne muscular dystrophy and hemophilia.

Key to our success is collaboration with patient advocacy groups, academic investigators and other industry partners.

A few examples of Pfizer's collaborations:

- Cystic Fibrosis Foundation, with whom we have a multiyear agreement to help speed the discovery and development of potential therapies that target the underlying cause of cystic fibrosis.
- The Rare Disease Consortium (RDC), a collaboration between Pfizer and GMEC Management Company Ltd., a joint venture of six major UK universities, designed to help accelerate the next generation of potential medicines.
- The Genetic Medicine Institute (GMI) in London, a partnership to develop potential gene therapies under the leadership of Michael Linden, Ph.D., Professor at King's College London and Director of the University College London (UCL) Gene Therapy Consortium, who is joining Pfizer on a two-year secondment.
- Pfizer's strategic collaboration with Spark Therapeutics to progress the clinical program for SPK-FIX for the potential

treatment of Hemophilia B, a genetic blood clotting disorder.

By working together in innovative and strategic ways, we are positioned to hopefully make a meaningful difference in the lives of individuals with rare diseases and their families.

Q What are the biggest challenges researchers and organizations face when it comes to developing new treatments for rare diseases?

A The field of rare diseases has unique challenges from those associated with more prevalent diseases.

Although there are an estimated 7,000 rare diseases in the world today, only about 400 have any approved treatments.

Developing effective potential therapies against these severe disorders has been a challenge due to several factors such as:

- Low frequency of each disease, fragmented knowledge and disparate patient populations
- Smaller, more heterogeneous patient population making clinical trials challenging due to patients scattered across geographies
- Limited number of specialists in each country who are able to diagnose and treat a particular rare disease, making it difficult to know how many patients suffer from a disease and for patients to actually get diagnosed at the onset of their symptoms

Q Where do you see rare disease drug discovery and development headed in the future?

A As exciting as our pipeline is today, our researchers in Cambridge, Mass. and the UK are pursuing the next wave of innovative potential therapies with multiple scientific and technology platforms. The dawn of gene therapy is providing new opportunities to explore transformational approaches that may enable us to provide potentially curative options for some people who currently have few treatment options and a limited quality of life.

The challenge for developing new treatments for rare diseases is too great for any one of us to go at it alone: collaboration is critical. Physicians, academic researchers, advocacy organizations and industry must all work together, and patients must be at the center of this approach.



**SAVE THE DATE: 2015 CRO/CMO SYMPOSIUM
FRIDAY, NOVEMBER 13, 2015
AT THE RENAISSANCE BOSTON WATERFRONT HOTEL**

Registration will open on May 1, 2015. Sponsorship and exhibitor opportunities are available now.
Contact Elizabeth Steele at elizabeth.steele@massbio.org or 617-674-5100.

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MassBio 2015 POLICY LEADERSHIP BREAKFAST

HEALTHCARE REFORM ON THE TABLE AT MASSBIO POLICY LEADERSHIP BREAKFAST



Ted Buckley of Shire posed a question to the panelists.

By MEAGHAN CASEY

On the same day the City of Boston celebrated a Super Bowl victory with a rolling parade, hundreds gathered downtown at the Omni Parker House to discuss how Massachusetts could remain dominant in another arena—the life sciences.

Kicking off the 2015 MassBio Policy Leadership Breakfast, held on Feb. 4, MassBio President & CEO Robert K. Coughlin outlined the following achievements in 2014: the Massachusetts biotech industry set a record for IPOs, the state’s biotech companies raised \$1.2 billion in venture capital and job growth in the medical devices industry outpaced growth in every other state. Despite the accomplishments, Coughlin stressed that companies, institutions and policy makers must take an active role in developing a sustainable model for life sciences innovation.

“As the world-leading life sciences cluster, Massachusetts has the most to gain—or lose—as the healthcare ecosystem evolves,” said Coughlin. “Our competition is getting steeper, internationally, and we cannot just sit back and take stock of our successes. We need to be sitting down with the leaders on Beacon Hill and Capitol Hill to shape smart public policy.”

Tackling the discussion of challenges facing innovators in the era of healthcare reform were panel members David Balekdjian, CEO of The Bruckner Group; Glenn Batchelder, co-founder of Civitas Therapeutics and chairman of the MassBio board; Eric Gascho, assistant vice president of government affairs for the National Health Council; Kenneth Kaitin, Tufts University School of Medicine professor and director of the Center for the Study of Drug Development; and David Seltz, executive director of the Massachusetts Health Policy Commission.

“The true defender of innovation is healthcare value,” said Balekdjian. “Companies need to demonstrate real, measurable value to stakeholders. Most companies have a better story to tell than the one they’re telling payers.”

Kaitin agreed that with payer policies increasingly defining access to therapeutics, companies must develop strategies for defining value early on.

“There needs to be more input from payers during the development process,” Kaitin said. “We have new scientific understanding of diseases, which gives us an opportunity to attack them. But there’s a cruel irony in developing a life-saving treatment that is unaffordable. There may need to be a compromise between what patients are willing to pay and what manufacturers are willing to do to make a product affordable.”

Panel members briefly discussed the 21st Century Cures initiative, which is expected to be presented as a bill later this year and could be a game-changer, if passed. With a goal of accelerating the pace of cures, backers are seeking faster approvals for drugs that receive breakthrough therapy designation from the FDA and more integration of patient perspectives into the regulatory process. “We need to better engage with patients and find out what matters most to

them,” said Gascho.

Kaitin broached the idea of value-based pricing, where payers and companies agree to link payment to value achieved (i.e. if the medication works), rather than volume. Batchelder and Gascho continued in that line of thought, discussing Foundation Medicine, a company leading a transformation in cancer care, where each patient’s treatment is informed by a deep understanding of the molecular changes that contribute to their disease.

“It’s about getting the right drug to the right patient—and in this case targeting the specific tumor—and using the best tools in the most effective way,” said Gascho. “That way someone isn’t paying for a product or therapy that isn’t working. They’re only paying for ones that do.”

Batchelder also discussed how the Affordable Care Act has helped to make the system more efficient and cost effective.

“Investors need to have confidence that if we succeed in developing a therapy that meaningfully improves patients’ lives or reduces the overall cost to the healthcare system that the doctor can prescribe it, the patient will receive it and the healthcare system will pay for it,” Batchelder said. “The biosimilars legislation created a robust and reliable framework to balance the best science and drug cost saving. That builds investors’ confidence to make the risky, long-term bets.”

At the event, Coughlin also presented House Speaker Robert DeLeo with the 2014 MassBio Policy Leadership Award. Since becoming Speaker, DeLeo has spearheaded two economic development packages that focus on improving the Massachusetts economy. He has been lauded for his effective and creative approach to job creation and his commitment to improving the safety of Massachusetts’ residents and communities. Previously, he brought legislation that cut healthcare costs for cities and towns throughout the state.

“Bolstered by our incredible research facilities, talent, workforce training, and financing incentives, Massachusetts has set the foundation for continued success in the biotech sector, but we must still do more,” said DeLeo.

DeLeo spoke about the success of the \$1 billion Life Sciences Initiative and the promise of organizations such as Massachusetts Computing Attainment Network (MassCAN), dedicated to developing talent capable of innovating in a digital world.

“As we strive to remain on the cutting edge of scientific achievement, we need to always be thinking about how we can retain our students, companies and employees,” said DeLeo. “We rely on them to contribute to the economic and social fabric of our state.”

DeLeo also spoke about the groundbreaking legislation to combat substance abuse that former Gov. Deval Patrick signed into law in August. Federal leaders in drug policy are turning to the law as a model.

“Massachusetts can be the leader in this field and we’ll be looking to MassBio for guidance,” said DeLeo.



Panelists discuss challenges facing innovators in this era of healthcare reform.



Speaker Robert DeLeo greets industry leaders.



Speaker DeLeo was presented with the MassBio Policy Leadership Award by MassBio President & CEO Robert K. Coughlin, left, and Board Chairman Glenn Batchelder.



Martha Bebinger of WBUR moderated the panel, which included from left, David Seltz, Eric Gascho and Kenneth Kaitin. Also on the panel were David Balekdjian and Glenn Batchelder.

INDUSTRY ENDORSES 17 PROGRAMS AT COMMUNITY COLLEGES

The Massachusetts Life Sciences Education Consortium (MLSEC) has officially endorsed 17 biotechnology degree or certificate programs at 10 community colleges and other educational institutions. The MLSEC is an initiative convened by MassBio and the MassBioEd Foundation to facilitate partnerships between the life sciences industry and higher education in order to more effectively match graduating students with the jobs companies are seeking to fill.

The MLSEC celebrated the successes of these programs at an endorsement event at Shire in Lexington on December 2, 2014.

Guest speakers included David Cedrone, Associate Commissioner for Economic and Workforce Development and STEM and Executive Director of the STEM Advisory Council at the Massachusetts Department of Higher Education; Matt Sigelman, Chief Executive Officer of Burning Glass Technologies; Bill Ciambone, Senior Vice President of Technical Operations at Shire; and Alex Wilson, Manufacturing Technician I at Shire.

“These endorsements ensure that community college biotechnology students and biotechnology certificate earners are provided with the information and experience they need to be successful candidates for careers in the life sciences industry,” said Lance Hartford, Executive Director of the MassBioEd Foundation. “Designing educational

programs off of the competencies that life sciences companies require from employees ensures that students learn skills relevant and applicable to the research and manufacturing jobs available.”

“The Massachusetts life sciences industry depends on highly trained workers at every stage of the drug development and manufacturing process,” said Robert K. Coughlin, President & CEO of MassBio. “By producing graduates ready to join the industry, these endorsed programs are helping fill the pipeline of industry workers to ensure that our life sciences supercluster can continue to grow and get therapies to

patients around the world.” Each educational program was evaluated based on program overview and scope of services, demonstration of laboratory practices, lab techniques and competencies. Also evaluated were workforce pathway development including the utilization of an advisory board, career services offered, and opportunities for work simulations and internships. Programs that met the minimum requirements were then rated Platinum, Gold, or Silver, based on an assessment across these areas.

The programs to the right are now endorsed by the life sciences industry. These programs produce graduates and certificate earners who are ready to join the industry.

To learn more about the MLSEC or the MassBioEd Foundation, visit www.MassBioEd.org.



Alex Wilson, a graduate of Quincy College, now works at Shire.



College representatives celebrated endorsements by the MLSEC for their biotechnology programs.

SCHOOL	PROGRAM	AWARD
Berkshire Community College	Associate Degree	Silver
Bunker Hill Community College	Associate Degree	Gold
Middlesex Community College	Associate Degree, Certificate Program	Platinum, Platinum
Mount Wachusett Community College	Associate Degree, Certificate Program	Gold, Gold
North Shore Community College	Associate Degree, Certificate Program	Silver, Silver
Northern Essex Community College	Associate Degree	Platinum
Quincy College	Associate Degree, Certificate Program	Platinum, Silver
Quinsigamond Community College	Associate Degree, Certificate Program	Gold, Platinum
Roxbury Community College	Associate Degree, Certificate Program	Platinum, Platinum
Springfield Technical Community College	Associate Degree, Certificate Program	Silver, Silver

THE MASSBIO EMPLOYEE PERKS PROGRAM IS EXPANDING

The MassBio Employee Perks Program offers employees of MassBio member companies significant discounts at local retailers and service providers. The program is an expansion of the MassBio Purchasing Consortium, a member benefit which has provided deep discounts on lab supplies and other corporate expenses for 25+ years.

MassBio is constantly enhancing the Perks Program by adding new savings opportunities. Our newest partners include Brandeis University, the Museum of Fine Arts, Residential Mortgage Services, and Something GUD.

Brandeis University offers MassBio members and their families special tuition pricing on Brandeis University’s online master’s degrees in informatics. This benefit includes include a scholarship of up to \$6,400 on Brandeis University’s online Master of Science in Bioinformatics or a scholarship of up to \$4,600 on the online Master of Science in Health and Medical Informatics.

The Museum of Fine Arts, Boston offers MassBio members special rates through a corporate membership. Benefits include general admission tickets, corporate



Brandeis University
Graduate Professional Studies



membership cards, invitations to corporate partner events and VIP tickets to select exhibitions

Residential Mortgage Services, Inc. (RMS) offers MassBio members closing cost discounts of up to \$500 when home-financing is completed with RMS. Other benefits of this program include quick and easy loan pre-qualifications, free credit analysis consultations and homebuyer or refinancing education.

Something GUD offers MassBio members a \$20 discount on a trial box of their farm-to-table delivery service, with an additional five percent off if members select the pickup option. Something GUD offers a full menu of food items sourced from the area’s top local farms and artisan foodmakers.

The MassBio Employee Perks Program gives members access to savings with more than two dozen partners. MassBio members can see all the current offerings and access these savings by downloading a MassBio Employee Perks Card in the Member Portal on www.MassBio.org.



MassBio President Robert K. Coughlin joined patient Megan Duff, parent Lisa Cimino, VHL Alliance Executive Director Ilene Sussman and NORD President Peter Saltonstall.



Ken Dhimitri moderated a discussion on rare disease research with panelists Dr. Norman Barton, Dr. Ed Kaye, Karen Peluso and Dr. Steve Uden.

PATIENTS ARE RARE ALONE, STRONG TOGETHER

BY MEAGHAN CASEY

For the millions of people living with rare diseases every day, Rare Disease Day is not a one-day event. It is, however, an opportunity to unite with other patients and stakeholders and share their unique stories.

On Feb. 23, more than 100 patients, advocates, biotechnology industry leaders and legislators gathered at the Massachusetts State House to mark the eighth annual Rare Disease Day. This year's theme "Living with a Rare Disease" paid tribute to the millions of family members and friends who are living day-by-day, hand-in-hand with rare disease patients. The complex nature of rare diseases, coupled with limited access to treatment and services, means that family members are often the primary source of solidarity, support and care for their loved ones.

Parent Lisa Cimino spoke about her 18-year-old daughter, Brianna, who was diagnosed at age 2½ with focal segmental glomerulosclerosis—a scar tissue in the filtering unit of the kidney. She has faced dialysis and a kidney transplant, which Cimino called "a treatment, not a cure."

"When my precious daughter was diagnosed, our lives changed. Our world changed," said Cimino. "Over the years I learned to comfort her through every injection, infusion or biopsy. She learned how to take 17 pills with one spoonful of applesauce. She is 18 today, and for that we are lucky. All too often, lives end before their time. I spoke about my daughter today, but I know there are so many who suffer."

Megan Duff, 31, spoke about her battle with neurofibromatosis—a genetic disorder of the nervous system that causes tumors to form on the nerves anywhere in or on the body at any time. She had numerous surgeries at a young age, dealt with learning disabilities and was diagnosed with an aggressive form of breast cancer at age 29. She is now in remission and shared an email that her mother, Kate, wrote

after her last round of chemotherapy.

"Megan has handled this journey with grace and courage beyond her years," wrote Kate Duff. "Life will never be the same, but I know Megan will find a new normal."

State Senator Karen Spilka thanked all of the patients in attendance for putting a face—and a voice—to their diseases.

"Each individual disease might impact a small amount of people, but as a whole, rare diseases affect 30 million Americans and countless others around the world," said Spilka. "Just by being here today, you're making a step to improve awareness and access."

Earlier in the day, MassBio hosted a panel discussion, "State of Rare: Taking Stock of Rare Disease Research Today and Tomorrow." Panelists included Dr. Norman Barton, vice president of clinical development at Shire; Dr. Ed Kaye, senior vice president and chief medical officer at Sarepta Therapeutics; Karen Peluso, executive director of Neurofibromatosis Northeast; and Dr. Steve Uden, senior vice president of research at Alexion Pharmaceuticals. It was moderated by Ken Dhimitri, vice president of operations at Boston Biomedical Associates.

Each of the panelists discussed the opportunities, such as RNA-based therapeutics, that didn't exist for patients with rare diseases 20-30 years ago.

"My entire adult life has been spent in rare diseases," said Kaye, who was previously at Genzyme. At Sarepta, part of his focus is on developing multiple drug candidates for Duchenne muscular dystrophy (DMD). "It took us a long time to have the tools to actually do something about the diseases—to offer patients hope. There are 20 different trials for DMD right now, which would have been unheard of 20 years ago."

"The Genome Project really transformed how we look at genetic disorders and how we can devise more effective drug

therapies against them," said Barton, who has worked in clinical development programs for rare disorders and underserved populations such as Gaucher, Fabry and Niemann-Pick diseases, metachromatic leukodystrophy, intractable gout, DMD and retinopathy of prematurity and bronchopulmonary dysplasia in premature infants.

"There's been a definite shift from just treating the symptoms to modifying the actual disease," said Dhimitri, whose 15 years of leadership includes the operational and financial oversight of drug, biologic and device trials.

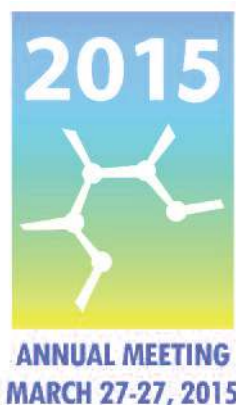
Uden, who has an extensive background in scientific strategy across R&D, credited the shift to a better understanding of the science of the diseases. "The industry is moving towards a transformative approach," he said.

Peluso's involvement with neurofibromatosis began 30 years ago, when she and her husband adopted a daughter who was diagnosed with the disease. Like Duff, who spoke later in the day, her daughter struggled from a young age, suffering from tumors attacking her organs.

"Her surgeon gave us the names of other families and that was the beginning of our organization," said Peluso, who helped to grow the not-for-profit from a grassroots, volunteer-driven organization to a leading resource for neurofibromatosis patients, clinicians and scientists in the Northeast. "At first, we worked out of my kitchen, trying to bring researchers to the field. We can look back now and say we generated more than \$500 million for research."

Peluso said there are currently drugs available to treat neurofibromatosis symptoms, but a long-lasting solution is still needed. She is hopeful—not only for neurofibromatosis patients, but for others.

"If you are a small organization or newly diagnosed with a rare disease, have faith," said Peluso. "These are great times to be living in."



ANNUAL MEETING WILL ADDRESS VALUE, PRECISION MEDICINE

ANNUAL MEETING: from Page 1

Better Business Track breakout sessions will address:

- Not Your Grandfather's Manufacturing
- Innovative Ways to Fund Your Early-Stage Company
- Externalizing Pharma R&D

- The Evolving Reimbursement Landscape
 - Technology Transfer: Status Quo, Evolution, or Revolution?
- Trends in Science Track breakout sessions will address:**
- Trends in Healthcare Technology
 - From Bioterrorism to Superorganisms: Perils, Pitfalls and Promise

- Neuroscience Therapeutics Development: Challenges, Opportunities and the Road Ahead
- Immunotherapy: Oncology and Beyond
- Current and Future Impact of CRISPR, CART and Gene Therapy

The MassBio Annual Meeting also includes elections for the MassBio Board of Directors and will commemorate MassBio's 30th anniversary.

Registration is now open online at www.MassBio.org. Sponsorship opportunities are also available. Contact Elizabeth Steele at 617-674-5100 or elizabeth.steele@massbio.org.



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PROMISE FOR A BETTER FUTURE

BY MEAGHAN CASEY

Thirty years ago, Jean Walsh was diagnosed with Friedreich's ataxia (FA) — a debilitating, life-shortening, degenerative neuro-muscular disorder. There was little understanding of the disease at the time, and no promise for a cure. Today, a number of Massachusetts biotech companies, such as Voyager Therapeutics, are giving patients like Walsh hope.

"When I was diagnosed, doctors thought vitamin E might help," said Walsh. "Today, there's such great progress."

About one in 50,000 people in the U.S. have Friedreich's ataxia. Onset of symptoms can vary from childhood to adulthood. Childhood onset of FA is usually between the ages of 5 and 15 and tends to be associated with a more rapid progression.

Walsh was diagnosed at age 19, but her symptoms began about three years before that. Symptoms include loss of coordination in the arms and legs, fatigue and muscle loss, vision impairment, hearing loss, slurred speech, scoliosis, an enlarged heart and diabetes mellitus.

"At first, you lose your coordination and it just looks like you're a klutz," said Walsh. "I've even been accused of being intoxicated, which is a common misunderstanding for those of us with FA."

Walsh began using a walker at age 33 and a wheelchair at age 44. For her, the diagnosis was upsetting and confusing early on, but it is one she has learned to accept.

"I was depressed about it at first, but then I decided I could be happy with or without Friedreich's ataxia," said Walsh. "I chose to be happy. While disease and disability do not run my life, they are facts of my life. And to me, they have given me opportunities to be strong and give back in ways I might not have otherwise."

Walsh has been married for 19 years. She is in her final year at Boston University, earning her master's degree in social work. She holds another master's in community social psychology from UMass Lowell. She completed her undergraduate work at UMass Amherst and worked as a grant writer for a number of years. She is also an active member of the Friedreich's Ataxia Research Alliance (FARA)—a national, organization

dedicated to the pursuit of scientific research leading to treatments and a cure for FA.

For the past three years, Walsh has volunteered as a member of the FARA Ambassador Program, representing the FA community by blogging, speaking at events, meeting with potential donors or scientific groups and participating in other awareness and fundraising opportunities.

"Talking with and getting to know other people with FA has been a wonderful experience," she said.

Before FARA, which was founded in 1998, Walsh said there were no other organizations to turn to for support. Her younger sister, Tricia, also diagnosed with FA, became the only person with whom she could share experiences. Tricia was diagnosed at age 24, 11 years after Walsh.

"We can at least talk about it together and lean on each other," said Walsh.

Last year, through her involvement with FARA, Walsh attended the ribbon cutting for Voyager Therapeutics. The Cambridge-based gene therapy company was founded in February 2014 to develop life-changing treatments for fatal and debilitating diseases of the central nervous system. In the case of FA, patients have a genetic mutation in the FXN gene, which limits the production of the protein frataxin. Voyager's goal is to deliver a functional version of the FXN gene to targeted cells in the central nervous system, increase levels of frataxin and have a meaningful impact on the progression of the disease. The company is still in the pre-clinical phase.

"FARA has done a wonderful job cultivating a network, establishing a patient registry and making it appealing for the industry to work on a cure for FA," said Walsh.

In addition to Voyager, other local companies such as RaNA Therapeutics and Pfizer are working on therapies to increase levels of frataxin.

"It's huge," said Walsh. "Even if treatment doesn't happen for me—which it might—there are so many other people it could help. Earlier this year, I met an 8-year-old with FA who was already using a walker. To think he might not have to live his life that way is beyond exciting."

JEAN
WALSH

PATIENT
PROFILE

