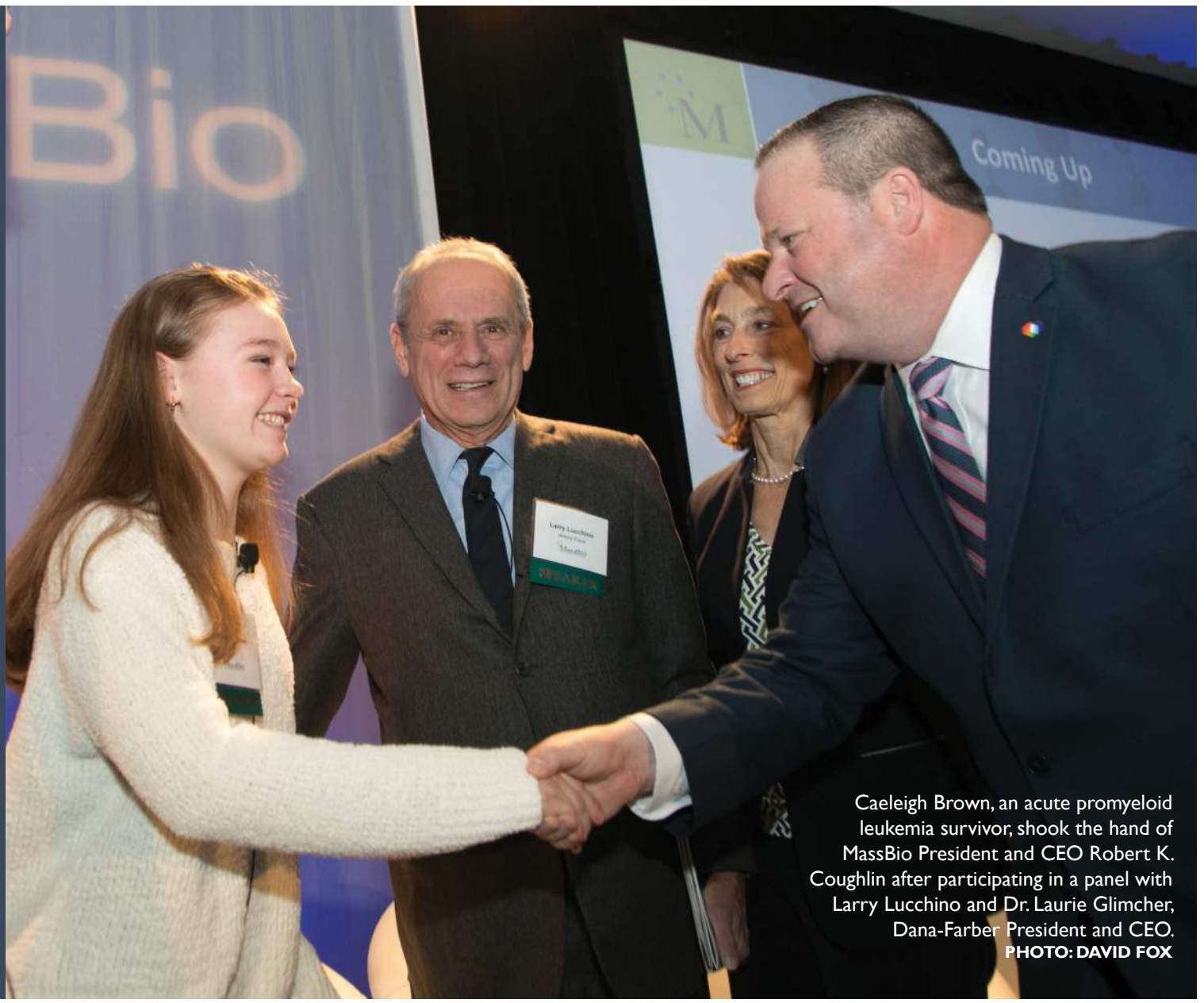
Vol. 11, No. 1 | A publication of the Massachusetts Biotechnology Council | Spring 2018

MassBio news
MASSACHUSETTS BIOTECHNOLOGY COUNCIL

MassBio Annual Meeting celebrates progress

From breakthroughs in scientific endeavors to startups and new business ventures, MassBio's Annual Meeting covered the most innovative advancements in the industry, as well as the most timely and critical challenges. The two-day event, held at the Royal Sonesta Boston, drew more than 400 industry leaders. It not only celebrated the progress, innovation and success of the last few decades, but it carefully examined what's next.

PAGES 4-5



Caeleigh Brown, an acute promyeloid leukemia survivor, shook the hand of MassBio President and CEO Robert K. Coughlin after participating in a panel with Larry Lucchino and Dr. Laurie Glimcher, Dana-Farber President and CEO.
PHOTO: DAVID FOX

Welcoming BIO back to Boston to celebrate the life sciences

ROBERT K. COUGHLIN

The BIO International Convention is coming back to Boston this year, bringing tens of thousands of life sciences professionals who are looking to network, partner and learn more about the biggest issues facing the industry. Attendees will hear directly from Massachusetts innovators who have helped make our cluster the best in the world, and MassBio is honored to be the regional host.

At its core, Massachusetts' growth and success stems from the unique environment we have – leading academic research facilities, world-class universities and colleges, and a supportive government. This combination allows research and early-stage companies – the backbone of our industry – to thrive. What's changed dramatically over the last 10 years is that big pharma has changed their R&D model to include more external partnerships than in the past; and, they are all here looking for their next opportunities. The thriving partnering environment extends to med tech and digital health, where companies are looking at the possibilities relating to convergence, digital therapeutics and drug delivery.

Looking forward, 2018 has the potential to be an inflection point for the Massachusetts life sciences industry. The original 10-year, $1 billion Life Sciences Initiative expires this year and is up for re-authorization by the Legislature for another five years, $500 million. The Life Sciences Initiative made government a true partner to the industry. That was and remains a major differentiator among Massachusetts and every other competing cluster. Continuation of this initiative and government's ongoing support will be critical to the future success of our industry.

It's within this spirit of collaboration that we celebrate all that we've achieved, and the amazing innovations that are still to come. I hope that you'll join us in welcoming the industry to Boston in June and will visit the Massachusetts Pavilion on the Convention floor. Visit StateofPossible.com to sign up for updates on the Pavilion, and to secure your ticket for the community concert that will feature Little Big Town at the Blue Hills Bank Pavilion. We hope to see you there!

Robert K. Coughlin is President & CEO of MassBio.

Honored and grateful to be serving this industry

DAVID LUCCHINO

It is an honor to lead MassBio as chairman for the next two years. But any pride I might feel is tempered by tremendous gratitude – gratitude for the scientists, physicians, business leaders and others who enable the companies that make up our industry to deliver life-changing therapies and treatments to patients around the world.

I am one of those patients. Five years ago, I was diagnosed with multiple myeloma, the second-most-common blood cancer and one that was once uniformly fatal. But a breakthrough drug developed in Massachusetts has enabled patients like me to live with cancer as a chronic condition instead of a death sentence. I believe we all do our best work when we keep the patient in mind – and this drives my work as CEO of Frequency Therapeutics.

These are good times in the life sciences industry, with ample flows of money and record numbers of drug approvals by the FDA. But we haven't done enough to ensure that the opportunities and benefits that flow from our success reach all the cities and towns in Massachusetts.

I will strive to ensure that people from all socioeconomic backgrounds have an opportunity to join our industry, and will build on the powerful work of outgoing chair Abbie Celniker to increase diversity and inclusion across our industry. This will mean partnering with more educational institutions at the associate and community college level, and working with lower-income communities to see that success in life sciences has a positive impact on the entire Commonwealth.

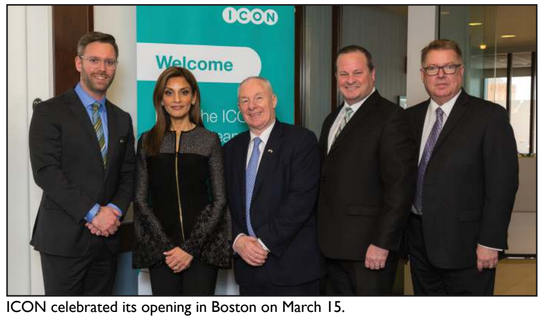
Ultimately, our focus must remain on patients. We are at our best when we remember that our goal is to alleviate suffering and help people live longer, healthier lives. When we do that, the benefits of our world-class life sciences cluster are felt not just in Cambridge and not just in Massachusetts, but around the world.

David Lucchino is Chair of the MassBio Board and CEO of Frequency Therapeutics.

MassBio news

A PUBLICATION OF:
Massachusetts Biotechnology Council
300 Technology Square
Cambridge, MA 02139
617-674-5100 ■ www.massbio.org

OPEN FOR BUSINESS



ICON celebrated its opening in Boston on March 15.

Versatope Therapeutics celebrated its new laboratory space at the M2D2 incubator at the University of Massachusetts Lowell on March 16.

NEW MASSBIO MEMBERS

Acer Therapeutics
Akriveia
Aldeyra
Allergan
Aminex Therapeutics Inc
Apellis Pharmaceutical
Avantik
BAIM Institute
Beam Therapeutics
Beeken Biomedical, LLC
Biocytogen
BioDirection, Inc.
Biological Industries USA
Boston Therapeutics
Burns and Levinson
Compass Consulting Group
Contracts Associates
CureVac, Inc.
Cyteir Therapeutics
EightSpokes, Inc.
Eloxx Pharma
Enko Chem
EverCell Bio, Inc.
Exonics Thereapeutics
The Bill and Melinda Gates Medical Research Institute
Gemini Therapeutics
Genoskin
HelixBind, Inc.
Histogenics
Hogan Lovells
Human Metabolome Technologies
Hummingbird Bio
ICON
Institute for Protein Innovation
Iontas, Ltd.
Ixcela, Inc.
JETRO
KingFisher Talent
Kytopen
Lavoie Communications
LayerBio
LevLane
Locke Lord LLP
Lumind Research Disease Foundation
Lyndra
Mansfield Bio-Incubator
MBHB, LLP
Medix
MSM Protein Technologies
Nelson Mullins
Nest Bio Labs
NeuroInitiative LLC
Nilogen
NovoVita Histopath Laboratories
Oracle
Orionis Biosciences
Ovizio Imaging Systems
Partner Therapeutics
PharmaEssentia
Planet Pharma
Poretta and Orr
Premier Data Center Services
ProMIS Nuerosciences
Publicis Health
Quorum Review
Recipharm Laboratories Inc.
reSTORbio
Rheos Medicines
RowAnalytics
RxCelerate
Service Bio Inc.
Sherpa Clinical
Slalom Consulting
SMOC Therapeutics
Softworld, Inc.
Spero Therapeutics
Stemcell Technologies
Symbiotix Bio
Transperfect Translations
Trevie Research
Valerion Therapeutics
VL51
WCG
Xcell Biosciences
YourEncore
ZappRX

Pre-approval access in Europe — the secret to success?

Pre-approval access (early access, expanded access, named patient supply etc.) is a hot topic at present, especially with the high-profile discussions around Right-to-Try, which are making the headlines around the world. Regardless of the merits of Right-to-Try, pre-approval access to medicines is high on the agenda for many pharmaceutical and biotech companies.

Successful pre-approval access for biotech companies can be a game-changer. There are several crucial advantages for biotechs who embark on pre-approval access in Europe. Firstly, it is possible in most EU countries to open a pre-approval access program whilst the product is in PII. Crucially, in most countries it is also possible to charge a commercial price for your product. Such programs also provide the opportunity to capture clinical outcomes data, to develop relationships with physicians, and to demonstrate demand for, and efficacy of, your product in a real-world patient population. Furthermore, an EU access program can continue indefinitely if you have no plans to commercialize outside of the U.S.

There are many examples of companies securing long-term benefits through the strategic use of a pre-approval access program. The opportunity to generate some early revenues, the implications of generating outcomes data from European patients, and the benefits of a positive physician experience of your product ahead of commercial availability are only a few of the many potential benefits of allowing pre-approval access in Europe, whilst you continue on your clinical development pathway.

Whilst at first glance the regulatory landscape in Europe can look daunting, it is relatively straightforward to navigate. All EU countries have adapted overarching EU legislation on pre-approval access to fit within the specific environment of their own healthcare systems, with the intention of making it as easy as possible for companies to provide access to patients in need.

The key thing is that you are in control. The regulatory environment allows you to open a pre-approval access program in one, many or all EU countries. You decide what approach suits your organization best. It is possible to 'test the waters' by opening a program in a selected few countries initially and expand to others as demand dictates. You have full control over which patients and physicians you allow on to any program, and unlike the U.S. EAP system, there is no requirement for monitoring, or for a protocol (with the exception of a few countries) to be developed. It is also possible to control the numbers of patients entering a program, until you have a more detailed understanding of demand.

Partnering with an experienced vendor relieves you of the burden of the day-to-day challenges of any pre-approval access program and allows you to focus on your key objectives of clinical and commercial development.

However, before embarking on an EU access program, it makes sense to validate in advance the pros and cons of such an approach, as a number of product- and disease-specific considerations can inform the go or no-go decision.

In order to understand whether it is feasible, or practical, to put in place a pre-approval access program a detailed feasibility analysis is recommended. A product-specific, per country feasibility analysis will allow you to understand the disease-specific and regulatory-specific issues in countries of interest, allowing you to make informed decisions regarding whether or not to implement a pre-approval access program in Europe. The more detailed the up-front understanding, the better your decision-making.

Examples of the topics which should be considered in a pre-approval access feasibility analysis include:

- In what countries can we open a pre-approval access program at PII/PIII?
- Does the data (efficacy and safety) support the commencement of a pre-approval access program?
- What existing therapies are available in different EU countries for our indication?
- Where can we charge for our product at PII/PIII?
- What is the awareness/demand for our product?
- What are the implications for supply chain?
- What is the existing treatment landscape?
- What competitor clinical trials and development strategies do we need to take into account?
- What are our peer-group companies doing with regard to pre-approval access?
- What is the environment for clinical data capture?
- Are there any existing data sets in any country that could be used as historical controls for our pre-approval access program data?

Regardless of what happens with Right-to-Try, the environment in Europe for pre-approval access is well developed, clearly structured and well controlled for those companies who wish to make their innovative assets available to patients early.

This opinion piece is sponsored by Inceptua.

Stuart Bell is Head of Inceptua Medicines Access Consulting. He has more than 20 years of healthcare and pharma consulting experience, with a particular focus in unlicensed medicines and pre-approval access. His experience covers global corporate strategy development, real-world evidence, market access and product launch, particularly in the areas of rare diseases, orphan products and oncology.

Contact Inceptua Medicines Access Consulting: Stuart Bell
stuart.bell@inceptua.com
or ring direct on: +44 73 8726 5293

There is power in numbers!

By aggregating the purchasing power of the member companies within MassBio, the MassBio Purchasing Consortium allows members to have a strong presence in the marketplace so they can bring more to their bottom lines. These case studies show savings actually achieved by MassBio members.

MassBio continuously evaluates the needs of member companies and the existing contracts to ensure the best value.

Check out the savings actually achieved by MassBio members:

Employees: 20
Agricultural Biotechnology

Items Purchased:

- *Hazardous Waste Management*
- *Lab Supplies*
- *Biomedical Waste Management*
- *Office Supplies*
- *Lab Equipment Maintenance*

Consortium Spend	$1,399,054
Est. Savings Off List Price	$ 903,157
+ Year-End Rebate	$ 26,691
- MassBio Annual Dues	$ 1,900
Bottom Line Savings	**$ 927,948**

Employees: 78
Drug Development

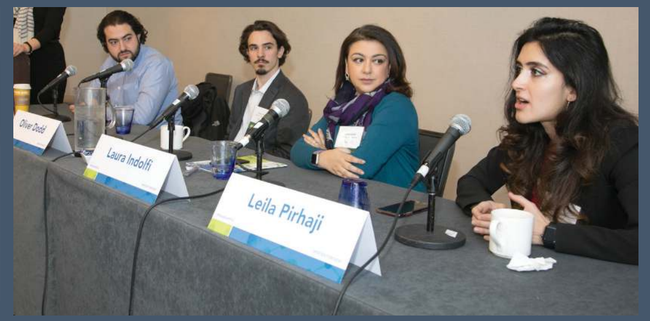
Items Purchased:

- *Hazardous Waste Management*
- *Lab Gases*
- *Biomedical Waste Management*
- *Lab Supplies*
- *Lab Equipment Maintenance*
- *Office Supplies*
- *Shipping Services*
- *Travel*
- *Pipette Calibration*

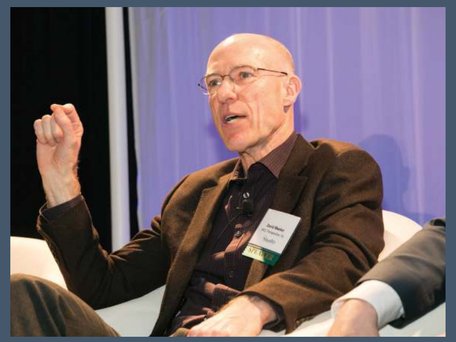
Consortium Spend	$2,599,347
Est. Savings Off List Price	$1,055,897
+ Year-End Rebate	$ 17,258
- MassBio Annual Dues	$ 7,291
Bottom Line Savings	**$1,065,864**

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

MassBioEd Executive Director Peter Abair and Michelle Mischke, director of Biotechnology Education Programs, presented Essex Technical High School with the Innovative School of the Year Award.



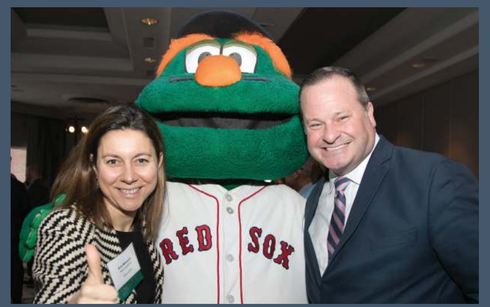
From right, MassConnect participants Leila Pirhaji, Laura Indolfi, Oliver Dodd and Mounir Koussa discussed their experiences with the mentoring program.



David Meeker joined Robert Coughlin on stage for a candid talk.

Dr. Stephen Sallan, Caeleigh Brown, Dr. Laurie Glimcher and Larry Lucchino celebrated 70 years of the Jimmy Fund.

Bloomberg News reporter Anne Mostue moderated a conversation on the idustry's reputation.



Ana Maiques, CEO of Neuroelectrics, joined MassBio President & CEO Robert Coughlin for a photo opp with Wally.

Vertex President & CEO Dr. Jeffrey Leiden was presented with the Innovative Leadership Award.

Penny Heaton, CEO of the Bill & Melinda Gates Medical Research Institute, delivered the opening keynote.

Anna Abram, deputy commissioner at the FDA, gave the closing address.

MASSBIO ANNUAL MEETING EXPLORES PATIENT ACCESS

By Meaghan Casey

Long before his name would become synonymous with Red Sox championships, Larry Lucchino was a just a young baseball executive living in Baltimore.

It was 1985; he was 40 years old and working as the Orioles' general counsel. Life seemed pretty good, and then he was diagnosed with non-Hodgkin's lymphoma. Turning to the Dana Farber Cancer Institute, he received an experimental bone-marrow transplant that destroyed and rebuilt his immune system.

"I was between Baltimore and D.C. and had plenty of opportunities to choose, but I decided I should go to Dana-Farber because it was an extraordinary place with extraordinary history," said Lucchino, who talked about his first interaction with Dr. Tom Frei, physician-in-chief at Dana-Farber from 1972 to 1985, who picked up his call at 11 p.m. the day he was diagnosed and walked him through his options until 1 a.m.

"You have to find people who genuinely care," he said, "and Tom Frei had empathy oozing from his pores."

Thirty-three years and three World Series titles later, Lucchino, now retired as president and CEO of the Red Sox, revisited that experience during MassBio's Annual Meeting, where he was joined on stage by his nephew, David Lucchino, a multiple myeloma survivor. David, MassBio's newly elected board chair, also shared his story of benefiting from a breakthrough drug developed at Dana-Farber.

Along with Dr. Laurie Glimcher, Dana-Farber President and CEO; Dr. Stephen Sallan, chair of the Department of Pediatric Oncology at Dana-Farber; and 13-year old Caeleigh Brown, an acute promyeloid leukemia survivor, the Lucchinos celebrated 70 years of the Jimmy Fund and Dana-Farber helping patients with cancer.

"We're living in a truly revolutionary time," said Glimcher. "Wouldn't it be wonderful if our grandchildren could be immunized with a vaccine against most cancers? We've had the first success with one of our faculty members who developed a vaccine for melanoma. She treated six patients with this personalized vaccine and all six of them — who were in late-stage, as the disease had spread — went into remission. We can do that for every cancer. I'm confident we can."

Improving patients' access to innovative new therapies was a theme that carried throughout the two-day Annual Meeting in March. From breakthroughs in scientific endeavors to new business ventures, the event covered exciting advancements in the industry, as well as the most timely and critical challenges.

During her keynote address, Penny Heaton, CEO of the Bill & Melinda Gates Medical Research Institute, encouraged attendees to think of those patients most in need outside of the U.S. The goal of the newly launched medical research organization is to combat diseases that disproportionately impact the poor in low- and middle-income countries by accelerating progress in translational science. It hopes to eradicate malaria, accelerate the end of the tuberculosis epidemic, and end diarrheal deaths in children.

"We still have 525,000 people dying from enteric and diarrheal diseases, along with 430,000 due to malaria and 1.7 million from tuberculosis," said Heaton. "These three tough diseases cause over four deaths every minute. We want to find solutions for these diseases so that everyone can have the opportunity to live a healthy life."

George Church, professor of genetics at Harvard Medical School and professor of health sciences and technology at Harvard and MIT, agreed that disease control and prevention has typically been underfunded. "There's a tendency for even investors to avoid preventative or diagnostic," he said.

That line of thinking came up again during a panel on cardiovascular diseases. Eighty million people in the U.S. are living with one or more types of cardiovascular disease, which is linked to more than 7 million hospitalizations each year. Yet, it also remains underfunded and in 10 years, only two drugs have been approved.

Industry veteran David Meeker, CEO at KSQ Therapeutics, connected the dots between funding, pricing and the journey that it takes to bring forward transformative therapies.

"There's a massive disconnect between that moment at the pharmacy window and the incredible importance of an industry that is trying to solve the biggest challenges — the devasting diseases that shorten our lives," said Meeker. "Alzheimer's is not going to be cured by a hospital bed or an imaging procedure. Those may be important elements of care, but the breakthrough is going to come from this industry. That breakthrough may be 10 years away, but if we don't have a system that is able to support that long-term investment, we will not get there. And if we don't get there, not only are we as individuals going to be devasted by that, but society as a whole is going to be devasted."

During the event's closing keynote, Anna Abram, Deputy Commissioner for Policy, Planning, Legislation and Analysis for the FDA, outlined the FDA's work to expand access to biosimilars and generics and to accelerate novel drug approvals. Last year, the FDA approved 46 new drugs— the highest number in more than two decades.

Breakout sessions during the event covered diversity in leadership, health economics, venture capital deal-making, regenerative technologies, digital currency and more.

Russ Wilcox, partner at Pillar, discussed the advantages of using blockchain technology to customize and enhance healthcare operations.

"It's a whole new way to see who's contributing data and who's using data, and all of the sudden we have giant databases of genetic information," said Wilcox. "If patients are able to have control of their own health records, we could have much more fluidity across healthcare."

Participants in MassBio's MassCONNECT program talked about how the entrepreneur mentorship program has helped bring their innovations to fruition.

"I came in with the science and the idea of what we thought was the killer app, but the most valuable thing was getting validation from people in the industry that we should be focusing on that idea," said Oliver Dodd, co-founder of Remora Therapeutics. He founded Remora, which is developing platelet-based cell therapies for the treatment of cancer and auto-immune disorders, while he was still a student at MIT.

"There's no substitute for having people there who are genuinely interested in the concept and technology," he continued.

In addition to examining what's next for the industry, the event also celebrated the progress, innovation and success of the last decades. MassBio founders David Glass, Marc Goldberg, Dana Ono and Rev. Dr. James Sherblom discussed the evolution from 1985 to today.

"It was a unique industry where we all liked each other and wanted to create a helpful, constructive community, and that's the culture that continued," said Goldberg.

"Back then, our goal was to get to 20 members to join MassBio and to get an audience of 60 people," he continued, looking out to a crowd of more than 400 attendees this year.

The event also recognized and awarded industry leaders and next-generation talent. Dr. Jeffrey Leiden, Vertex President and CEO was presented the Henri A. Termeer Innovative Leadership Award. The Joshua Boger Innovative School of the Year Award went to Essex Technical High School.

ANOTHER CHANCE AT LIFE

Kidney disease patient wishes for future treatment alternatives

By Meaghan Casey

When Hanaa Al-Dulaimi was diagnosed with end-stage renal disease, it was a shock.

Otherwise healthy, she had been experiencing some cramping, but nothing else out of the ordinary. That is often the case with patients like her. Since kidneys are highly adaptable and able to compensate for lost function, signs and symptoms unfortunately may not appear until irreversible damage has occurred.

Tests revealed Al-Dulaimi's blood potassium level was 9 millimoles per liter — significantly higher than the normal range of 3.6 to 5.2 mmol/L. Further examination revealed her kidneys had shrunk in size.

"No one knew why I had kidney failure," she said. "I never had diabetes. I had a healthy diet. It didn't make sense."

End-stage renal disease, also called end-stage kidney disease, occurs when chronic kidney disease — the gradual loss of kidney function — reaches an advanced state. Patients' kidneys are no longer able to work as they should, allowing dangerous levels of fluid, electrolytes and wastes to build up in their bodies.

Al-Dulaimi was given a startling prognosis. She would need chronic hemodialysis or a kidney transplant to live. Hemodialysis is a procedure that cleans the patient's blood by circulating it through a dialysis machine for three to four hours, three times a week. Successful hemodialysis requires a very high volume of blood, which is not feasible using normal blood vessels. It requires the creation of a specialized form of vascular access, most commonly an arteriovenous fistula, created by connecting a vein to an artery in a simple outpatient surgical procedure.

The fistula procedure has remained largely unchanged in the last 50 years, and up to 70 percent will require further corrective procedures such as surgical revision or angioplasty, typically because they do not achieve or maintain sufficient blood flow for dialysis.

Dr. Michael Germain, a nephrologist at Bay State Medical Center in Springfield, emphasizes the unmet need for patients undergoing dialysis.

"Improvements to vascular access mean a huge improvement to patients," said Dr. Germain. "There haven't really been any developments from an access standpoint, and [access–related complications] are traumatic and problematic."

Currently, there are more than 400,000 people undergoing hemodialysis in the U.S., with an additional 100,000 entering treatment annually. Al-Dulaimi began dialysis in 2010. Originally from Iraq, she was living in Egypt at the time. She moved to the U.S. in 2012 with her husband and their three children and continued dialysis at Baystate Medical Center.

"It's uncomfortable, but you have to do it to stay alive," she said. "It's like a job. You have to go and it's a very time-consuming process that takes time away from family."

When her vascular access hardened, knowing it would interfere with dialysis, she was fearful.

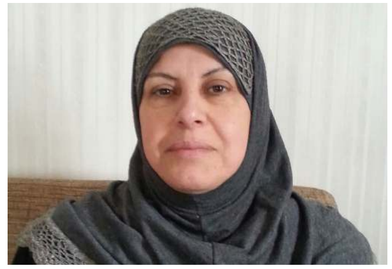
"There are patients who pass away after two months or a few weeks of dialysis because of infections and access issues," she said. "There's always a risk that something will go wrong."

There was hope though. Al-Dulaimi was put on a transplant list in 2013 and received a kidney transplant in 2016. For her blood type — blood type B — the average wait time is three to five years for a transplant.

"I remember the day I got the call," said Al-Dulaimi, who was 51 at the time. "It was so amazing."

It was a successful surgery, although Al-Dulaimi experienced pain and discomfort in the weeks to follow. A biopsy at six months revealed she was free of the disease. Her blood potassium level has consistently been back to an average of 5 mmol/L.

"The new kidney helped me to walk," she said. "It also helped my heart. Before the transplant, it was weak from dialysis, but it's been stable since the transplant. I'm grateful I don't have the infection anymore, but I feel my body is still weak from the damage it did. Six years of dialysis is a long time."



Hanaa Al-Dulaimi received a kidney transplant in 2016.

Today, she is vocal about the need for innovation in end-stage renal disease care. She is hopeful companies are working to deliver a new future for patients and families affected by kidney and vascular diseases.

"I wish no one would ever have kidney failure or dialysis, but I hope they can develop some way to make dialysis easier," said Al-Dulaimi.

Waltham-based Proteon Therapeutics is currently investigating a new therapy to improve fistula outcomes. "We understand the challenges these patients face and have worked diligently for many years to bring clinically meaningful improvements to their unmet needs," said Timothy Noyes, President and CEO at Proteon.

In the interim, both Al-Dulaimi and Dr. Germain encourage patients to become educated self-advocates for themselves.

"Patients have the best outcome when they're involved in their care," said Dr. Germain. "Get involved early. Learn what kidney disease is about and how to plan for it."

"You see some patients who don't do the diet, and that's so important," said Al-Dulaimi. "You have to take care of yourself. I'm grateful for my family and the staff at Bay State who supported me, held me and encouraged me through everything."

Q&A with Edie Stringfellow

MassBio's Director of Diversity & Inclusion

MassBio is thrilled to welcome Edie Stringfellow as our first Director of Diversity & Inclusion. As we often argue, diversity and inclusion should not be just another initiative, but placed at the core of everything a company does.

Q What drew you to this position?

A I've always had high regard for this organization and throughout the interview process, it became clear to me that MassBio really is committed to improving diversity and inclusion. The best indication of that commitment is how they are leading by example. Our leaders will not sit on all-male panels. We won't host any events where the panelists are all male. Fifty percent of all the new board members being elected at this year's Annual Meeting are women; and, this is just the beginning.

Q In light of the amplified attention on women's rights, is your role more relevant than ever?

A There's finally a concerted effort to bring more equality to the workforce. I'm proud that MassBio is taking the lead and having these discussions. Now it's time to move the conversation forward and turn it into actionable plans. Strides have certainly been made, but there's still a lot of work to be done.

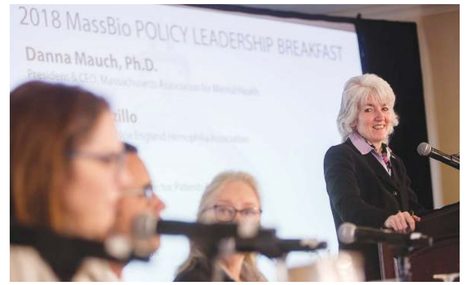
Q What are some next steps?

A Looking forward, we will support our members, industry partners and other associations by developing and disseminating diversity and inclusion best practices, while working with organizations who are already doing well in this area to expand our collective impact. We will encourage companies to embed diversity and inclusion in their growth strategies and culture; and to lead from the top and not by HR alone. A company's growth potential is boundless when everyone feels like they have a seat at the table. We will spread the message that while diversity is critical for strategy and business, it also has a positive effect on patient care.

Q Does this end with gender diversity?

A No. This is just our first initiative. We'll be pursuing initiatives that address racial, sexual orientation, age and other types of diversity. I invite all members to tell us how we may better support them regarding their diversity and inclusion initiatives and recommend ways we may improve our association.

MassBio President & CEO Robert K. Coughlin presented the Legislator of the Year Award to Sen. Michael Rodrigues.



Martha Bebinger, healthcare reporter with WBUR, moderated a panel discussion on pricing and patient access.

Laurie Bartlett Keating, SVP and General Counsel of Alnylam Pharmaceuticals, joined in the discussion on the Life Sciences Initiative.

POLICY ON THE MENU AT MASSBIO BREAKFAST

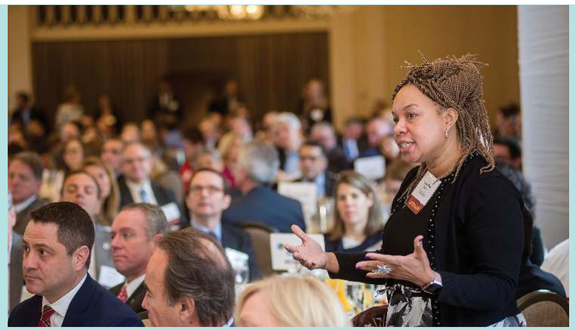
By Meaghan Casey

It was anything but politics as usual at MassBio's annual Policy Leadership Breakfast, which brought together policy makers, life sciences leaders and other key stakeholders to discuss the most pressing initiatives impacting the industry.

The event, held on Jan. 24, included two panel discussions — one on initiatives beyond pricing and transparency that are underway in Massachusetts that are focused on patient access to therapies, and the second on the Life Sciences Initiative and what is needed in the next initiative to ensure the continued success of the industry in the state.

"Over the last 10+ years, government has been a true partner to the life sciences industry and therefore to patients — not only here, but for the world," said MassBio President and CEO Robert K. Coughlin. "This theme runs through the program, with both panels exploring different ways that government impacts how patients gain access to new medicines."

Step therapy — a means of trying less expensive and often older options before "stepping up" to drugs that cost more — was the focus of much of the conversation during the first panel. The approach has faced much criticism within the industry and two Massachusetts legislators have filed separate bills in the House and Senate proposing a restriction on the ability of insurance companies to require step therapy. Panelist Richard Pezzillo, executive director of the New England Hemophilia Association, advocated for eliminating step therapy altogether.



Cherie Butts of Biogen presented the panelists with a question. **PHOTOS: SEAN BROWNE**

"In hemophilia care, there is no generics," said Pezzillo. "We don't have that option. And when you look at those living with hemophilia, it's a very small number — less than 750 in Massachusetts — but the costs associated with each person can easily exceed $350,000 a year prophylactically. For those forming inhibitors — meaning their body is forming an antibody to the clotting factor that they need — it could be over $1 million."

Pezzillo argued that approaches like step therapy, specialty tiers and non-medical switching, at least in the hemophilia community, are hurting patients' quality of life and ultimately costing the system more money.

"No one wins," he said. "Patients are put in a position that requires them to fail first. No one should be told to go on another prescription just because of costs or failures."

Danna Mauch, president and CEO of the Massachusetts Association for Mental Health, and Terry Wilcox, co-founder and executive director of Patients Rising NOW agreed that government should step in and ensure patients get the therapies they need without interference from insurers.

"It really is true that around 70 percent or so of patients fit in the lane that follows the path of whatever it is insurers are trying to do," said Wilcox. "But making a true effort to focus on the other 30 percent of patients as individuals and not delay their care ultimately helps to curb spending costs. You don't want them to go through treatments that they can't use and don't need, and that will delay their care. And you don't want unnecessary hospital bills to add up. You need the right path to allocate for the patients that don't fit in the mold."

To discuss the next phase of the Life Sciences Initiative, Travis McCready, Massachusetts Life Sciences Center president and CEO, joined Dr. Michael Collins, chancellor of UMass Medical School; Sandra L. Fenwick, president and CEO of Boston Children's Hospital; and Laurie Bartlett Keating, senior vice president and general counsel and secretary at Alnylam Pharmaceuticals.

"The human capital dimension is what's powered this ecosystem for as long as it has and we will continue to invest in internships and schools throughout the state," said McCready. "We believe Massachusetts can be the state that is fully integrated, where you can go from basic research all the way not just to commercialization, but to production. Where we're still a little soft is in manufacturing, so we're going to focus our time and attention and research into process and equipment innovation and capacity, capacity, capacity."

Also at the event, MassBio honored Senator Michael Rodrigues with the Legislator of the Year Award. Joanne M. Duncan, president of BIO's Membership and Business Operations Division, previewed the upcoming BIO International Convention in Boston.

RARE DISEASE DAY CELEBRATES THE ZEBRAS AMONG US

RARE: from Page 8

gene silencing. It's a first step. Will it save me? It just might. But it's going to save the two of my four kids who are carrying the same trait that I am."

Prior to the main event, MassBio hosted a panel discussion, "Shortening the Diagnostic Journey for Children with Rare Diseases." More than half of the 30 million Americans with a rare disease are children. The panel focused on the crucial role that physicians, patients, clinical technology and the complex healthcare system play in bringing down the existing barriers they face on the road to diagnosis, and looked at new interventions that might be needed.

Fred Walsh shared a personal story of his missed opportunity for a diagnosis years ago. After experiencing lingering respiratory infections and being re-diagnosed with asthma, he was finally correctly diagnosed in 1989 with Alpha-1, a genetic condition that may result in serious lung disease or liver disease. He later found out his uncle was diagnosed with Alpha-1 as early as 1966.

"We were never tested, but we should have been," said Walsh. "Did it make a difference? Well, I probably wouldn't have been diagnosed when I only had 40-percent lung function. In that time, I probably would have changed certain habits and made more intelligent decisions based on my risk factors."

As is often the case, it is harder for rare patients to fight an unknown or unnamed enemy.

"We would love if there was a cure for every rare disease, but in most cases, there aren't," said Anne O'Donnell-Luria, associate director of the Center for Mendelian Genomics at the Broad Institute of MIT and Harvard. "Prognosis is hugely important. Families want to know what could happen. Having a diagnosis doesn't give a prescription of exactly what your child's life looks like, but it gives you some ranges of what might happen. I meet a lot of families who know something's wrong from early on and they want to know if their child is going to walk and talk; if whatever's going on is going to affect their life expectancy. Having a sense of that — while not always good news — lets families move forward to some degree."

MassBio
300 TECHNOLOGY SQUARE, CAMBRIDGE, MA 02139

MassBio's 10th annual Rare Disease Day celebrates the zebras among us

By Meaghan Casey

Across the country, nearly 30 million individuals are living with rare diseases — a number that surpasses all of those who have HIV, heart disease or stroke.

Yet, only 5 percent of rare diseases have treatments. People with rare diseases have tremendous unmet needs, including misdiagnosis, a long road to receiving a correct diagnosis and an even bigger challenge in tackling the question: What next? After all, there are fewer than 500 FDA-approved treatments for the more than 7,000 rare diseases that exist.

That's why research was the topic of discussion at MassBio's Rare Disease Day event on Feb. 28. More than 500 industry stakeholders gathered for the sold-out event at the UMass Club. It was MassBio's 10th celebration of Rare Disease Day in Massachusetts, and it reflected a dramatic increase in support from the first gathering of 75 attendees in 2009.

Katie Brandt, Director of Caregiver Support Services in the Frontotemporal Disorders Unit at Massachusetts General Hospital, emceed the event, reminding this year's attendees of the power in numbers.

"Alone we are rare, but together we are strong," Brandt repeated throughout the event.

Calling attention to what has become the rare mascot, the zebra, Brandt explained the maxim coined in the late 1940s by Dr. Theodore Woodward, a medical educator nominated for the Nobel Prize for his work in the field of infectious diseases. When he told his students, "when you hear hoofbeats, think of horses, not zebras," it was a reminder to diagnose the most common, likely causes. But for rare patients, there is nothing common about the road to diagnosis.

"We're here today thinking about the millions of individuals worldwide pounding the ground with the hoofprint of the zebra," said Katie. "Today is about them. Today is expecting the unexpected."

Jen Melanson knows all too well about the unexpected. The mother of three is a patient with hypoparathyroidism and CTLA4 haploinsufficiency, but it wasn't until her youngest son underwent three years of genetic testing that CTLA4 haploinsufficiency was detected in their family. This genetic disorder — which was only discovered in 2014 — impairs normal regulation of the immune system and results in autoimmunity, low levels of antibodies and recurrent infections.

"Research has been the key to our family's diagnosis and it remains the best hope for anyone who remains undiagnosed. While it took almost three years to diagnose my son, it took more than 45 years for me," said Melanson, who received her positive results in December. Her oldest son tested positive for the disorder in January, leaving only her daughter unscathed. "As you can imagine, my family feels a little like we've been broadsided by an oversized tractor trailer, but I actually think we're pretty darn lucky. Not only are both of my sons under the care of our very smart, very kind researcher/ rheumatologist at Boston Children's Hospital, but I also managed to find myself a very smart, very kind immunologist at Mass. General Hospital. My sons and I are now part of the ongoing research for our genetic disorder at both sites and my youngest son has already begun a treatment that has the potential to halt his disease progression."

More than 500 attendees gathered for MassBio's 10th celebration of Rare Disease Day. **PHOTO: SEAN BROWNE**

Another parent advocate, Michelle Hirsch Donovan, spoke out about the continued need to press legislators for federal research funding for rare diseases and to help young investigators who need startup funds and support.

"Awareness is the first step toward change, toward understanding and toward finding a cure," said Donovan, whose daughter, Riley, was diagnosed with neurofibromatosis type 1 (NF1) seven years ago. NF1 is a condition characterized by the growth of tumors along nerves in the skin, brain and other parts of the body.

"On a daily basis, we don't know if a stomachache is because she ate too many Cheez-Its or because she has a new tumor in her abdomen," she said. "Is a small headache just dehydration or a brain tumor? She sees 10 specialists regularly and surgery is often ineffective since the tumors frequently grow back."

While there is no known cure, the FDA granted Orphan Drug Designation in February for an oral inhibitor to treat NF1.

"This is progress, and hope and progress are two of the many reasons why we as a group never stop fighting," said Donovan. "I know I will never stop fighting for my Riley. She, like many of your loved ones, is worth each and every ounce of our fight."

ALS patient Richard Kennedy, who serves as president of the Angel Fund for ALS Research, lost both his father and his brother to ALS decades ago. Kennedy was just diagnosed two years ago.

"Don't feel bad for me," he said. "The best thing you can do for a patient with ALS or neurofibromatosis or any other disease is to hand them optimism. My dad and my brother Jimmy never had a chance. As early as this summer, I very likely will be the first patient to undergo what we call C9

See **RARE** Page 7