

MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL



NOBEL CAUSE

Dr. Thomas Südhof, who won the 2013 Nobel Prize for Physiology or Medicine for his work with autism research, meets Timmy Supple, 9. As part of his research, Südhof has been studying the genes of Timmy and his brother Stuart since 2009.

PATIENT PROFILE Page 8

MAKE AN IMPACT

ANNUAL MEETING TO LAUNCH STRATEGIC PLAN THROUGH 2020

The agenda is coming together for the MassBio Annual Meeting, being held April 3-4 at the Royal Sonesta Hotel in Cambridge. Program sessions will include discussions on rebuilding the industry's reputation, mobile technologies and 3D printing, clinical trial design and the future of gene therapy.

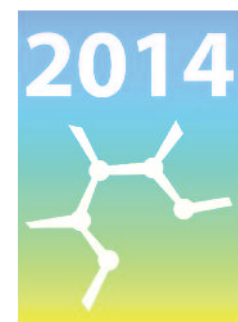
The agenda also includes elections for the MassBio Board of Directors, two keynote speeches and the annual awards luncheon.

The meeting will kick off with the release of Impact 2020, a new strategic plan for the industry and MassBio. Based on nearly 100 stakeholder interviews, survey data and original research, the plan will assess the critical events, trends, policies and discoveries that will impact the life sciences sector through 2020. The report also makes recommendations to ensure that the industry thrives in Massachusetts and continues to advance science for generations to come.

A panel discussion on value in healthcare will analyze the implication for business leaders as the result of the changing landscape in hospital reimbursement, regulations and priorities. This will be a continuation of the conversation begun at MassBio's 2014 Policy Leadership Breakfast.

Business track panels will discuss:

- New ways to fund your early-stage company
- What the life sciences industry needs to do to rebuild its reputation



**ANNUAL MEETING
APRIL 3-4, 2014**

See **ANNUAL MEETING** Page 7

SUCCESS IS A RESULT OF INNOVATION AND TALENT



ROBERT K. COUGHLIN

Coming off a very busy year on Beacon Hill and Capitol Hill, this is a good opportunity to take stock of the policy and regulatory accomplishments of the past year, while also taking a look at what is in store for 2014. From implementation of the Affordable Care Act to approval pathway for biosimilars, we faced new changes, challenges and concerns in 2013. We continue, however, to advocate for policies that will help bring beneficial therapies to the market and make them available to all.

This year, as always, MassBio will persist in advocating for legislative initiatives and regulations that protect the incredible ideas that are born right here in the Commonwealth. We know that the industry's success in Massachusetts is a result of innovation and talent — the scientists, venture capitalists and entrepreneurs

researching, developing and manufacturing groundbreaking therapies and technologies that improve the lives of patients near and far. As we look ahead to 2020, Massachusetts is poised to stay at the top, but we will not rest on our laurels. We know we face competition, both locally and abroad. It is our job to ensure that innovation thrives and our biotechnology supercluster remains second to none.

I thank all of you who attended our Policy Leadership Breakfast — the biggest and most successful in years. We welcomed Boston Mayor Marty Walsh to the podium for the first time and had an engaging discussion on the implications of defining “value” in healthcare with our panel of experts in the field. It was also a privilege for me to present Nick Littlefield, long-time champion of the

industry, with the MassBio Policy Leadership Award. Nick has been an attorney, a philanthropist and an advocate — and, to me, an invaluable mentor. He is a man who has done more for this industry than almost anyone I can think of, and I could not think of a more deserving recipient.

In the coming months, we will be building off the energy from that breakfast at our other events, including our Annual Meeting in April and our CRO/CMO Symposium in May. I hope you can join us for both, as well as the BIO International Convention in San Diego.

Thank you for your continued support. I look forward to a successful 2014.

Robert K. Coughlin is President & CEO of MassBio.



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SAVE THE DATE: MA CRO/CMO SYMPOSIUM MAY 5 AT THE BURLINGTON MARRIOTT

Now in its third year, the Massachusetts CRO/CMO Symposium shines a spotlight on the strength and success of the CRO and CMO communities in the Commonwealth.

The Symposium draws more than 300 attendees to discuss and evaluate the best alternatives that have evolved in shortening time to market. A series of case studies, interactive panel discussions and networking sessions will provide ample time



to explore the various techniques and approaches being used by both virtual and established companies.

Early-bird registration is available now, with rates as low as \$95 for members through Feb. 28 online at www.MassBio.org.

Sponsorship and exhibitor opportunities are also

available. Contact Elizabeth Steele at 617-674-5100 or elizabeth.steele@massbio.org.

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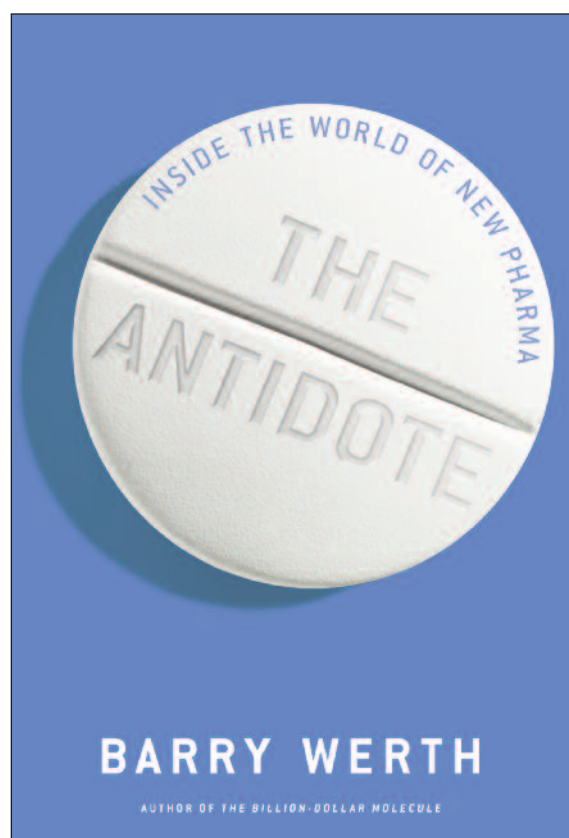


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Q & A WITH BARRY WERTH

In his new book, a follow-up to “The Billion-Dollar Molecule,” author Barry Werth delivers a riveting account of how Vertex and its founder, Joshua Boger, struggled for two decades to bring out a breakthrough drug and, in the process, revolutionized the drug industry.

Q The biopharma industry has certainly gone through dramatic changes in the last 20 years and is rapidly evolving today. Will we ever see another Vertex? Could a company start and grow the same way in today's environment?

A The revolution is biopharma since the early nineties has been nothing short of breathtaking. I'll give you just one example. When Boger left there in 1989, Merck was the most admired corporation in America. It also was a tower of vertical integration, owning everything it felt it needed to design and develop blockbuster drugs. It had vast resources: huge PK and toxicology labs, a juggernaut sales force. Not too many years before that it even owned the rail cars that transported raw chemicals to its manufacturing sites and the trucks that delivered its pills to pharmacies. Now fast forward to 2014. Boger is executive chairman of Alkeus, which has a promising treatment for the leading cause of childhood blindness. The company has an open IND, it has manufactured drug, it has orphan drug status and clinical sites ready and a verbal agreement with the FDA into Phase II trials. And it's two people. In other words, the old organizational structures are all gone – smashed – and innovation favors smaller and smaller, albeit highly networked, collaborative entities.

Could today's environment produce another Vertex? I don't know. The key elements are the familiar ones: people, time, and money. The reason Vertex is successful so far is that it set out to build a broad portfolio of transformational medicines for people with serious illnesses. It took 20 years and almost \$4 billion dollars to become profitable, and as it is, it's been losing money again in the past year, and won't be in the black again probably until 2015. That's a long time to string investors along, especially in a capital market that exalts shareholder value. That said, hope springs eternal. There's so much excitement in the field right now about the future that investors are happy to bet and bet big.

Q The book summary references an “industry under siege and in crisis.” What did you see in researching this book that leads to that categorization? Is the future bleak or bright for biotech?

A These are disruptive times for US pharma. The blockbuster era – which has engendered much of the bad behavior in recent years; the fraud, me-tooism, off-label marketing and other aggressive attempts to squeeze value out of aging product categories – is over, which is not to say that there won't be drugs that generate

billions of dollars in sales, but that these drugs will be specialty products, for a relatively small number of patients. The industry is facing a productivity crisis just as payers worldwide are pressing down on prices, emphasizing outcomes that have demonstrable economic value. Factoring in the 30 to 1 failure rate, it now costs about \$5 billion to bring a new drug to market. What this means that all companies have to increase their hit rates while dramatically bringing down costs. For the big the challenge is to restructure, focus down on their core strengths. For small and mid-sized biotechs like Vertex, it's to stay in the race long enough, investing billions, in a mad dash to come up with a product or products that can make them sustainable.

I'm an optimist. I think smart companies will figure out how to use new advances in technology and new scientific insights and clever management to make this happen. Not a lot, mind you, but enough to prove the concept and make a difference. The good news is that the future is here. Advances in personalized medicine like Vertex's Kalydeco are showing the way towards high-value, transformational medicines for small numbers of very sick people. This is great for patients, but in the near term at least these breakthroughs are going to drive the costs of drugs even higher. For now payers are willing to foot the bill, but we're on the verge of a

reckoning. Society is going to have to decide whether the trend is “worth it.”

Q What inspired you to return to Vertex 20 years after publishing “The Billion-Dollar Molecule,” your riveting first book on the biotech startup, and continue the story? Why is “The Antidote” an important story to share with the community, and why now?

A Hanging out inside a trailblazing start-up was easily the most fun I've had as a reporter. The pace was breakneck, the personalities fantastic, not to mention that scientists by and large are wonderful sources. Really smart, really interesting – born teachers. So I was looking for that again. But the main thing was I sensed an opportunity. I thought the time was right to see whether after a quarter century of predicting that Vertex could do what Big Pharma couldn't, founder Josh Boger had turned out to be a visionary after all. Boger, an outsized showman as CEO, has often been accused of arrogance. One of the first things I heard him say publicly to the company when I returned in 2011 was: “Arrogance is only a problem if it doesn't turn out to be true. If it turns out to be true, it's just persistence.” I wanted to discover if Boger was right.

What I found was — to a remarkable degree — Vertex has done just what Boger and the founding scientists set out to do. This is no small thing, given the steep risks, enormous obstacles, stupendous costs, and staggering odds. At a time when America is struggling to maintain its edge in innovation, I think the company's success can be a touchstone for recognizing what works and what doesn't in trying to build a sustainable, research-driven business. That it's a biopharmaceutical company, where the difference between success and failure can be the difference between life and death for patients, for me only heightens the drama and urgency of the story.



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MassBio 2014 POLICY LEADERSHIP BREAKFAST



Nick Littlefield, front, receives the 2014 MassBio Policy Leadership Award from MassBio President and CEO Robert K. Coughlin; InVivo Therapeutics CEO Mike Astrue; Wendy Everett, president of the New England Healthcare Institute; and Andrew Tarsy, president of the Edward M. Kennedy Institute for the U.S. Senate.



Andrew Dreyfus, left, President and CEO of Blue Cross Blue Shield Massachusetts, and Geoff MacKay, President and CEO of Organogenesis Inc., participated in a panel discussion.



Attendees took in the discussion on the value of innovation in an era of cost containment.

EXPERTS GATHER TO DISCUSS IMPACTS OF HEALTHCARE COSTS AND PATIENT ACCESS

By MEAGHAN CASEY

Addressing a crowd of nearly 300 gathered for the 2014 MassBio Policy Leadership Breakfast, Boston Mayor Martin J. Walsh took to the podium to share his deep-felt gratitude and respect for the life sciences industry.

Walsh’s investment is two-fold: as a policy maker and as a patient. He battled Burkitt’s lymphoma at age 7 and credits his survival in part to the experimental treatments and extraordinary care he received at Children’s Hospital and Dana Farber Cancer Institute.

“In 1974, the survival rate for Burkitt’s lymphoma was about 15 to 20 percent,” said Walsh. “Now it’s about 90 percent. There’s probably someone in this room who contributed to that, and for that I’m especially thankful. The research you do every day is saving lives.”

Walsh, who took office just a few weeks prior to the Jan. 29 event at the Omni Parker House, went on to announce his commitment to growing the Innovation District and attracting more businesses—particularly medical device manufacturing—to the region.

“You certainly have my commitment, here and in Washington, to try to bring in more funds for research and brick-and-mortar projects,” said Walsh.

The event also featured a discussion on the value of medical innovation in an era of cost containment. Moderated by WBUR health reporter Martha Bebinger, a panel—consisting of Robert J. Beall, President and CEO of the Cystic Fibrosis Foundation; David M. Cutler, Professor of Applied Economics at Harvard University; Andrew Dreyfus, President and CEO of Blue Cross Blue Shield of Massachusetts; Geoff MacKay, President and CEO of Organogenesis and Chairman of MassBio; and Dr. Elizabeth Nabel, President of Brigham and Women’s Health Care—discussed efforts to get a handle on the ever-rising cost of healthcare, as well as new approaches to ensure patients receive quality care and breakthrough therapies.

“Our charge is to infuse innovation and discovery in everything we do—in research space, in education models and in delivery of care,” said Nabel.

“The last thing anyone wants to do is stifle innovation,” said MacKay. “We have to accept that we’re going to get it wrong. But with industry, academia, hospitals and private insurers working together, we can make corrections as we go. If we

get it right, Massachusetts will be the blueprint for the rest of the country.”

The panelists agreed that in order to conserve and contain costs, healthcare providers will have to ensure the right patients are receiving the right therapies 100 percent of the time.

“What people really want is better value for what they’re spending,” said Dreyfus. “And for patients, the best value is the best outcome. In the past, we haven’t successfully targeted therapies to specific patients who will absolutely benefit.”

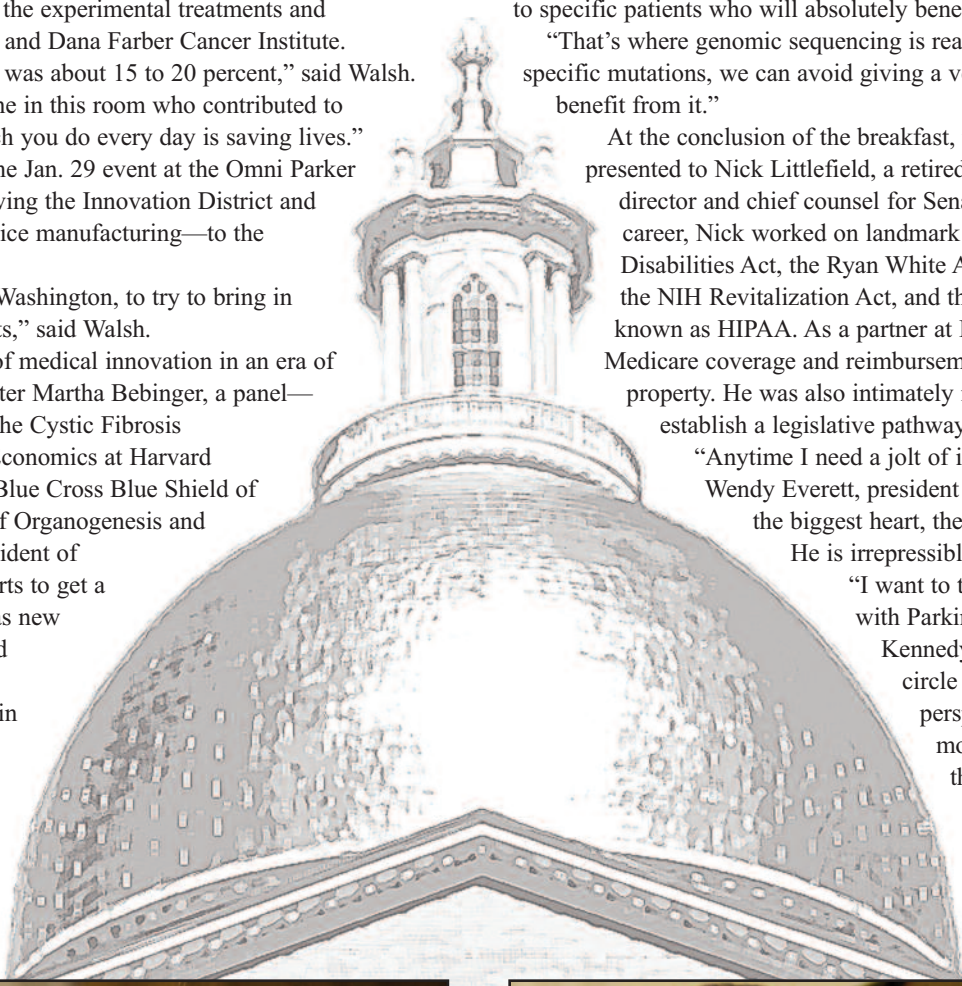
“That’s where genomic sequencing is really going to play a role,” said Nabel. “By targeting specific mutations, we can avoid giving a very expensive drug to a patient who wouldn’t benefit from it.”

At the conclusion of the breakfast, the 2014 MassBio Policy Leadership Award was presented to Nick Littlefield, a retired partner at Foley, Hoag LLP and longtime staff director and chief counsel for Senator Edward M. Kennedy. Over the course of his career, Nick worked on landmark legislation including the Americans with Disabilities Act, the Ryan White AIDS Care Act, the Prescription Drug User Fee Act, the NIH Revitalization Act, and the bipartisan legislation reforming health insurance known as HIPAA. As a partner at Foley Hoag, Nick advised clients on issues like Medicare coverage and reimbursement, orphan drug status and protection of intellectual property. He was also intimately involved in the 2007 Congressional effort to establish a legislative pathway for FDA to approve biosimilars.

“Anytime I need a jolt of inspiration, I sit across from Nick Littlefield,” said Wendy Everett, president of the New England Healthcare Institute. “Nick has the biggest heart, the biggest brain and the biggest dose of enthusiasm. He is irrepresible.”

“I want to thank everyone here,” said Littlefield, who now lives with Parkinson’s disease. “When I was working with Sen. Kennedy on orphan diseases, I never thought I’d come full circle and be here in the patient seat. From this perspective, I can fully recognize that what you do is the most important thing. There is simply no substitute to the policy work and research you all are doing.”

As part of his award, Littlefield was presented with a piece of artwork from Art in Giving, which will hang in his honor at the Edward M. Kennedy Institute.



Mayor Martin J. Walsh



Dr. Elizabeth Nabel



Geoff MacKay



Martha Bebinger



John Nash, CEO Franciscan Hospital for Children (FHC), center, welcomed MassBio President & CEO Robert K. Coughlin, left, and state Rep. Steven M. Walsh for an event at the hospital on Nov. 20 to discuss collaborative partnership opportunities with technology start-up entrepreneurs, life sciences industry leaders, and other members of the healthcare community.

MEMBER EMPLOYEES SAVE \$\$\$ ON BOSTON AREA GOODS

Employees of MassBio member companies are now eligible for significant discounts at more than a dozen local retailers and service providers.

The MassBio Employee Perks Program is designed to bring MassBio members the best deals and pricing available while also helping retailers in the community increase their visibility and expand their loyal customer bases.

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 more than 25 years.

Current Employee Perks program partners offer a range



of deals and discounts and include Ambit Creative Group, Art in Giving, Blue Man Group, Boston Celtics, BostonCoach, Boston Duck Tours, Cambridge Athletic Club (CAC), Champions Sports Bar, Dependable Cleaners, Milk Street Café, Salvatore's Restaurant, Silverbrook Farm, and Zipcar.

The Employee Perks program's offerings are primarily based in the Greater Boston area, but MassBio plans to continue to expand the program to include offerings in other biotech hubs.

MassBio members can access these savings by downloading a MassBio Employee Perks Card in the Member Portal on MassBio.org

Learn more and download your MassBio Employee Perks Card in the Member Portal at www.MassBio.org.



BUILD YOUR BUSINESS SKILLS IN 2014

MASSBIOED OFFERING NEW COURSES IN 2014 TO MEET YOUR LEADERSHIP- DEVELOPMENT NEEDS

Presentation Skills for Professionals

Presenting in front of an audience can be invigorating or deflating depending on one's presentation skills. The Presentation Skills class will teach participants to overcome their fears of presenting in front of an audience, refine their expertise, increase their confidence and help make presenting an energizing and rewarding experience.

This six-week course, developed at Harvard University, is designed to prepare researchers, scientists, faculty and biotech professionals develop and deliver presentations for conferences, seminars, workshops and business meetings internally, externally, and globally. The course uses a combination of in-class videotaped presentations, and self, peer and instructor evaluations.

Dates: Thursdays, March 27 – May 1, 2014

Time: 5:30 p.m. - 7:30 p.m.

Cost: \$1,500 for MassBio members; \$1,650 for non-members, \$1,300 for non-profits

Delivering Difficult Information

At some point in your career, you are going to have to tell someone something they don't want to hear. This course will help participants improve their ability to give specific feedback to individuals; learn how to effectively share information that may evoke a negative response; and prepare for the very human reactions that such sharing may involve. Participants will develop workable strategies to determine the underlying interactive preferences of others and to adjust their own style to interact most effectively with key constituents.

This workshop is highly interactive, and involves small-group discussion of case studies from the life sciences and technology industries. Participants will gain experience identifying the reactions to difficult information. They will develop strategies to work through the stages of shock, anger, and rejection to progress to acceptance, new learning, and changed behavior. They will receive easy-to-access handouts with reminders and strategies.

Date: Friday, May 9

Time: 9 a.m. - 4 p.m.

Cost: \$795 for MassBio members; \$945 for non-members

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

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

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REMEMBERING JAMES L. VINCENT, LONGTIME BIOGEN LEADER

BY PHILLIP SHARP AND VICTOR MCELHENY

Are they making innovators quite like James L. Vincent any more? This question, spurred by Vincent's death on Dec. 5, has more edge these days as we worry how Americans are going to keep inventing, making, and selling new things to earn a good living in a sharply competitive world. More bluntly, do the innovators of today have the fire in the belly to build and lead a major company?

Vincent, a big guy from a small town in Pennsylvania with experience of independent command at three big companies, who believed that you have to drive your career, not just let it happen to you, turned science-focused Biogen into an integrated, global, self-standing business. Under Vincent, Biogen began building its dominance of the array of drugs for multiple sclerosis through close and direct contact with tens of thousands of patients. And Biogen, founded in 1978 and led by Vincent from 1985 to 2002, through the launch of interferon-based Avonex for multiple sclerosis and beyond, is the oldest free-standing biotech company. Today, it offers three treatments for MS, including a newly introduced oral drug and two injectables.

In developing lasting independence for Biogen, now Biogen Idec, Vincent stands almost alone in biotechnology with another hard-driving big man from large companies, George Blatz Rathmann, who died in 2012. A son of privilege who helped develop Scotch-Guard at 3M and later worked with Vincent in the diagnostics arm of Abbott Laboratories, Rathmann put in 10 years as the inaugural CEO of Amgen. Armed with a gene patent on the blood protein erythropoietin (EPO), Rathmann defied Wall Street's nervous advice to settle a crucial suit with Genetics Institute. Victorious, Amgen achieved dominance in the market for EPO. With an array of products, Amgen is the largest free-standing biotech company.

How did Vincent's and Rathmann's earlier experiences in tech-built firms shape their strategies in biotech? Interestingly, they each decided to focus on a single new product—EPO for Amgen and beta-interferon for Biogen.

As is well known, most biotechnology startups have ended up within such larger enterprises as Roche and Johnson and Johnson (widely regarded as the most successful of the biotech absorbers). And the spectacle raises concerns. How



Jim Vincent, receiving the 1998 National Medal of Technology from President Bill Clinton.

can we maintain the kind of innovation that springs most naturally from a driven tribe of 150 in one large room, each with a voice but under a visionary leader, where the direction can change in a single day? Is it decreed in heaven, now that Bell Labs is dead, that big companies can only innovate by buying?

The disappearance of large, vertically integrated companies in recent decades, while the number of smaller, innovative firms has multiplied, is a striking change across the world of business. Clearly, Vincent and Rathmann anticipated the trend in pharmaceuticals. Now, the companies they built are becoming large and vertically integrated. Will they continue to innovate?

The story of Jim Vincent, an engineering graduate of Duke

with an MBA from Penn's Wharton School, who stepped determinedly from Texas Instruments to Abbott to Allied Signal before leading Biogen, shows a more complex relationship between big and small enterprises.

He knew he wouldn't be CEO where he was. And science-driven Biogen, operating competing research groups in Geneva and Cambridge, burning through cash, pulling in licensing revenue but not yet marketing its own products, was making its big investors, Schering-Plough and Monsanto, nervous. It was clear the company needed a new direction. The directors spent 10 months in 1985 searching for a new CEO. A strong voice in the process was board member Lou Fernandez, chairman of Monsanto, which had put \$20 million into Biogen in 1980, helping start the Cambridge lab. This bet was part of Monsanto's long search to shift from fossil hydrocarbon-based chemistry, commoditized products of ancient life, to complex proteins and nucleic acids produced by organisms living today. Interviewing leaders like Rathmann, the board eventually settled on Vincent.

Jim arrived with a figurative sign on his back: "I'm boss. Follow me." He laid it down that the company was in crisis mode. Everybody was going to count every dollar, and everybody was going to squeeze into economy class seats—even him. He knew he had to know enough science to participate meaningfully in the priority-setting decisions. He firmly believed that teams were more efficient than individuals. But, with a lot of practice, he was totally comfortable making a command decision.

He sold off the European operations, boosted revenues from intellectual property, slashed the burn rate in half, and got Biogen into the black in three years, opening the path to raising funds through new offerings of both preferred and common stock. Injectable Avonex provided multiple sclerosis patients with a serious defense against relapses.

Jim Vincent retired in 2002, having led Biogen for more than 15 years. When he died at the age of 73, his daughter Aimee spoke his epitaph: "He was very proud that Biogen maintained its independence as a company."

Reprinted with permission from Xconomy. Phil Sharp and Vic McElheny are both Xconomists. Both work at MIT.

ON THE AGENDA

ANNUAL MEETING: from Page 1

- Winning strategies in business development and corporate development: what are they and how to from them
 - Mobile technology and 3D printing: technologies gaining traction in biotech and pharma
 - Evolving business relationships between biotech, pharma, investors, and academia
- Science track panels will discuss:
- Clinical trial design trends

- The second coming of gene therapy
- New approaches in the management of neurological disease
- Is there an industry opportunity cost to focusing on rare disease?

The MassBio Annual Meeting also includes the Innovative Leadership Award luncheon, which honors an industry leader for his or her contribution to moving the industry forward.

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.

SEEKING NOMINATIONS FOR LEADING IMPACT AWARD

MassBio's Economic Development Advisory Group (EDAG) established the MassBio Leading Impact Award to annually recognize significant contributions made by individuals and organizations that have successfully worked to improve the competitiveness of Massachusetts as a destination for the life sciences industry. Past winners include the Massachusetts Biomedical Initiatives incubator and Pfizer's Center for Therapeutic Innovation. Examples of potential honorees include municipal governments that have removed barriers to

entry for life sciences facility projects, organizations that have advanced industry growth through unique civic engagement, or developers or individuals whose exemplary actions over time or on specific projects have contributed to the growth of the industry.

Nominations for the 2014 Leading Impact Award must be submitted by 5 p.m. on Feb. 21. The awardee(s) will be recognized at the MassBio Annual Meeting in April. Submitting a nomination is easy: Go to MassBio's website at www.MassBio.org to find the submission link in the news section.

(RE)SEARCHING FOR A CURE

SUPPLES: from Page 8

with autism have our boys' same gene alteration, but even if it is rare it could be related to brain pathways that are common across many people with autism. We see some amazing treatments for symptoms every day; but it's really exciting to think of treatments for the disease itself."

Autism Speaks, the world's leading autism science and advocacy organization, is doing its part to try to meet the challenge. In 2011, the organization launched a collaboration with the world's largest genome sequencing institution, BGI (formerly Beijing Genomics Institute). The Autism Speaks-BGI 10K Genome Project builds on the work of Autism Speaks Autism Genetic Resource Exchange (AGRE), a collection of more than 10,000 anonymous DNA samples and robust clinical information from families affected with autism. In July, investigators released results from the project's pilot study, which focused on 32 families affected by autism and identified autism-linked gene changes—

including four new autism-risk genes and eight suspected ones—in 16 of the families.

"The fact that we found genetic variants in half of the families reflects our new ability to apply genome sequencing to find those missed in other approaches," said the study's senior author, Dr. Stephen Scherer of the University of Toronto. "This could allow for earlier diagnosis and treatment, particularly among siblings of children with autism."

Since it was established in 2005, Autism Speaks has funded more than \$173 million in scientific research — including Dr. Südhof's research — to investigate the causes, diagnosis, and treatments of autism. Chris serves on the board of the New England chapter of the organization and is chair of its advocacy committee.

"With people like Dr. Südhof and his team at his lab, and organizations like Autism Speaks, we're certain there will be great progress made," said Chris. "We just don't know if it will be in five years or 25 years or in any time period significant for our kids, but we're hopeful. For our family to be a part of something that could bring some relief some day to tens of thousands of families whose lives have been turned upside-down by autism would bring great meaning and value to the otherwise awful impact autism has had on us."



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**PATIENT
PROFILE**

(RE)SEARCHING FOR A CURE

BY MEAGHAN CASEY

Across the U.S., an estimated 1 of 54 boys (1 of 88 children overall) are diagnosed with autism.

For Natick residents Chris and Kate Supple, those statistics were dramatically higher: 2 of 2.

The Supples' older son, Stuart, was diagnosed with autism in 2005. Just a year later, their second son, Timmy, was diagnosed with autism as well.

A neurological disorder, autism is characterized by difficulties in social interaction, impaired verbal and nonverbal communication, and sensory integration deficits that can result in repetitive, and often difficult and socially awkward, behaviors. In the Supple family, 10-year-old Stuart has some limited language capabilities and attends public school, while Timmy, 9, is nonverbal and attends a private day school for children with autism.

"Timmy's much more significantly impacted," said Chris. "They both have autism, and in fact they both may have the very same medical issue that's causing their autism, but they're otherwise as different as two siblings could be."

Autism can be reliably diagnosed at 24 months and, in some cases, 18 months.

"Stuart was a late talker and walker, but every infant has his own pace," said Kate. "We didn't notice too much amiss with Stuart until Timmy was born. Stuart was 15 months at the time, and not really reacting to his new baby brother. You hope it's just delay, and then at some point you're told it's not delay, it's a disorder."

"After getting Stuart's diagnosis, we were hypersensitive to autism with Timmy," said Chris. "I was certain there was no way Timmy had autism, because as a baby and young toddler he was so alert and engaged. But in his middle-teen months you could start to see it, and then it was clear he was going to get the diagnosis, too, and he did."

While early detection and intervention are critical to improving outcomes, the cause of the disorder remains a mystery. Searching for answers, the Supples sought out genetic testing, which revealed that both boys carry the same gene alteration in a gene called Neuroligin 4 (NLG4), which produces a protein critical to neuron communication across gaps called synapses.

"We had no history of autism in either family, so it struck us as odd that we went 2-for-2 in the autism lottery," said Chris. "If



Dr. Thomas Südhof, left, meets with Kate, Stuart, Timmy and Chris Supple for the first time.

we'd had only one child with autism, we probably wouldn't have been as curious about the medical and biological reasons, but with two we wanted to see if we could get some answers."

The Supples reached out to Dr. Thomas Südhof, professor of molecular and cellular physiology at Stanford University School of Medicine, who had already been studying alterations in a related gene, Neuroligin 3, which had also been linked to autism. Südhof and his colleagues led a study and published a journal article in 2009 concluding that the NLG4 genetic alteration caused Stuart and Timmy's autism, but they are still working to determine exactly how, and why. He and his team are now analyzing it on two fronts: breeding mice that will have the same alteration, and recreating it in vitro using the boys' skin cells that are reprogrammed into stem cells and then brain cells. The neurons will provide different but complementary information about the function of NLG4 and how it will react to therapeutic drugs.

Südhof, who has been studying the Supple

boys' genes for more than five years now, was awarded the 2013 Nobel Prize in Physiology or Medicine this past October. He also received the 2013 Albert Lasker Basic Medical Research Award, which is among the most respected science prizes in the world. His work is fundamental to understanding and treating autism and other disorders of brain development.

"We'd like to understand how synapse communication leads to learning on a larger scale," said Südhof. "How are the specific connections established? How do they form? And what happens in autism when these connections are compromised?"

After a five-year coast-to-coast e-mail relationship, the Supples met Südhof in person for the first time in October, when the Supples organized and hosted a fundraiser in Boston in support of his research.

"Dr. Südhof had just won the Nobel Prize, so it was extraordinary to be able to celebrate that with him and for him to finally meet our boys," Kate said.

Outside of Südhof's independent lab



work, Stuart and Timmy have participated in several research studies. Clinicians can currently identify the genetic basis of autism spectrum disorder in 10 to 20 percent of cases. Those figures are expected to grow dramatically in the coming years as genetic discoveries continue to accelerate.

"The challenge is to get more autism families to do genetic testing and to get those results into a scientifically accessible database," said Kate. "Perhaps many others

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