

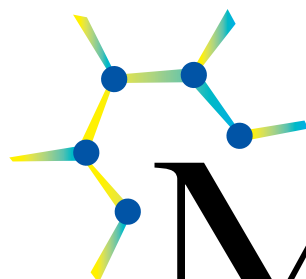


**BIOSIMILARS LAW SIGNED** Page 3



**IN SEARCH OF A CURE** Page 8

Vol. 7, No. 3 | A publication of the Massachusetts Biotechnology Council | Summer 2014



# MassBio news

MASSACHUSETTS BIOTECHNOLOGY COUNCIL

## BUILDING A GREENER FUTURE

Page 7

MIT's Koch Institute was designed to be energy-efficient.



## PARTNERSHIPS TO GROW AT BIOPHARM AMERICA

MassBio, in partnership with EBD Group—the leading partnering firm for the life sciences industry—will host the seventh annual BioPharm America™ conference September 22-24 at the Boston Marriott Copley Place.

BioPharm America's robust program features keynote addresses from Flemming Ornskov, CEO of Shire, and Gary Nabel, CSO of Sanofi. This year BioPharm America will again celebrate the announcement of the annual *FierceBiotech* Fierce 15.

"We are proud to host BioPharm America again in Boston," said Robert K. Coughlin, President and CEO of MassBio. "This is a high-caliber event that capitalizes on the full range of industry innovation available in Boston. And there is a wide range of partners who attend, from small start-ups to mid-size biotech companies, service providers and international pharma. Companies can tie up their partnering strategies for the year in just a couple days. There is no other industry gathering quite

like it."

One company that will be in attendance at BioPharm America is Johnson & Johnson. Robert Urban, who leads the Boston Innovation Center for Johnson & Johnson, spoke with EBD Group's partneringNEWS ahead of BioPharm America. Urban will be speaking as a panelist at the conference and also participating in partnering meetings during the event.

See **BIOPHARM AMERICA** Page 6



Robert Urban, who leads the Boston Innovation Center for Johnson & Johnson, will speak at BioPharm America.

# DEDICATED TO MAINTAINING STRENGTHS



**ROBERT K. COUGHLIN**

Inside this edition you will see a snapshot of Massachusetts' industry strengths—from seed-stage investments and deals to federal research funding, venture capital investments and R&D jobs. This stems from a heritage of innovation and rich density of leading organizations across sectors, from top NIH-funding hospitals to world-ranked universities, biotech pioneers, big pharma companies and highly respected investment firms. But with success comes responsibility, and we at MassBio are prepared to be the greatest advocate for the Mass. life sciences cluster. We will

continue to champion innovation, ensure a vibrant startup environment and maximize access to funding options. You can read more about our plans, outlined in Impact 2020, at [www.MassBio.org](http://www.MassBio.org)

As we look ahead to the fall, we have a number of exciting events lined up. It is our goal to make BioPharm America 2014 the most successful deal-making event on the East Coast and the primary event to showcase therapeutic and research innovation. I encourage you to register today.

I also invite you to join us for our annual golf

outing on Sept. 5 at Pinehills Golf Club in Plymouth. Proceeds support MassBioEd's work advocating for and funding programs and services proven to build the Massachusetts biotechnology workforce.

As we say goodbye to summer, best wishes for an enjoyable and productive fall.

*Robert K. Coughlin is President & CEO of MassBio.*



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## FALL FORUM SERIES WILL BEGIN CONVERSATION ON DEFINING VALUE



The Impact 2020 report lays out a strategic, five-year plan for MassBio and the life sciences industry in Massachusetts. One of the key recommendations from Impact 2020 is for

MassBio to begin driving the conversation on defining value and reward for innovation in the era of outcomes-focused medicine.

To start this conversation, MassBio's Forum Advisory Board and Working Groups are hosting a three-part Forum series this fall focused on

value and how we define value from the perspectives of all stakeholders involved—investors, providers, payers, patients and patient advocates. In this series, industry leaders will discuss how these perspectives on value impact and shape company strategy and focus. The series will include:

Part 1 – Introduction & Overview (Oct. 7)  
Part 2 – Manufacturing & R&D (Nov. 20)  
Part 3 – Science & Business (Dec. 9)

*The Forum series speakers will be confirmed soon. Visit [www.MassBio.org](http://www.MassBio.org) for details.*

## NEW MEMBERS

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*For more information on membership, contact Laura Hamilton at 617-674-5100 or [laura.hamilton@massbio.org](mailto:laura.hamilton@massbio.org)*

## 5 GREAT REASONS TO BE...AT THE BETC



### 1 PILOT-SCALE LAB

### 2 FLEXIBLE, CUSTOMIZED EDUCATION

### 3 NEWEST TECHNOLOGIES FOR TRAINING

### 4 MASTER TECHNIQUES HERE

### 5 HANDS-ON EXPERIENCE

The Biomanufacturing Education and Training Center (BETC) is a pilot-scale training facility with state-of-the-art classrooms and labs at Worcester Polytechnic Institute. The BETC's practical, hands-on approach includes courses developed collaboratively by WPI and industry partners—and we customize training as needed to best serve biomanufacturers and their employees.



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## POLICY UPDATE

### Governor Patrick signs biosimilars legislation



PHOTO: Eric Haynes/Governor's Office

In June, Massachusetts Governor Deval Patrick signed legislation designed to create a pathway for the substitution of interchangeable biologic medicines in Massachusetts that will ensure both patient access and patient safety.

The new law aligns with all five of BIO's principles on biologic substitution. Governor Patrick announced that the bill had been signed at the 2014 BIO International Convention, very welcome news for the Massachusetts contingent attending the convention.

"We're so excited that once again Massachusetts is leading the way in adopting policy that not only supports a growing industry, but more importantly allows patients access to the most cutting-edge therapies that have been proven safe and effective," said Robert K. Coughlin, President & CEO of MassBio. "We are fortunate to have visionary leaders like Governor Patrick, Senate President Therese Murray and House Speaker Robert DeLeo who are willing to stand up for innovation and patient safety."

The Massachusetts law preserves patient access to accurate prescription information, maintains incentives for innovation and promotes a competitive market for biologic therapies.

While the FDA is currently developing a pathway for the development and approval of safe and effective interchangeable biologic products in the United States, decisions on substitution practices and other implementation details are being legislated at the state level.

Patients and physicians managing chronic conditions are generally aware of which biologic treatments work best in their unique circumstances. Communicating with patients and physicians allows everyone involved the opportunity to discuss past treatment experiences so that any possible unexpected issues can be better understood and avoided.

MassBio, working in partnership with BIO, will continue to advocate for full transparency in the substitution process, as patients and their physicians should have the right to know what biologic medicine the patient receives from the pharmacy.

## Q & A WITH DR. LEONARD ZON

### Stem cells, ghosts and sharks



Dr. Leonard Zon, MD, is the founder and director of Boston Children's Hospital's Stem Cell Program and a pioneer in the use of zebrafish in the drug discovery process. He will be a judge for the Innovation Tank, a "Shark Tank"-style competition, taking place during Boston Children's Global Pediatric Innovation Summit +

Awards Taking on Tomorrow, Oct. 30-31, in Boston.

cell, it's very powerful and can make all of the tissues in the body, which offers tremendous potential for patients. We would like to replace patients' diseased organ cells with their own good cells. If I took a cell from a patient and then I made the stem cells, it would be her cells. She wouldn't reject it or need immunosuppression drugs. And we would have an unlimited source of cells.

We have made hundreds of stem cell lines from patients at Boston Children's, and we have begun to study their diseases in a dish. Consider a patient with muscular dystrophy. We can do a chemical screen to try and find a chemical that would correct the disease in a dish. Someday we also hope to generate muscle and then put good muscle back into the diseased muscle.

**Q What does a zebrafish named Casper have to do with stem cells?**

**A** We started doing stem cell research in 1991 and focused on blood stem cells. These cells, located inside the bone marrow, not only make blood but also self-renew. This capability becomes important in mitigating the collateral damage among leukemia patients treated with

high-dose chemotherapy.

Chemotherapy erases the immune system along with the cancer. A stem cell transplant, often from a donor sibling, kick starts the immune system and can provide patients with a functional immune system.

But stem cell transplants are risky, with a 25 percent mortality rate. Cells sometimes go awry after transplant and miss the intended target—bone marrow. When I do a transplant, I have no idea where the cells go after I transplant them.

Several years ago, we made a transparent zebrafish named Casper. Now, I can see where the stem cells go. We found they like to go to the bone marrow on a certain route through the blood vessels. We're trying to use Casper to identify drugs that can increase the rate stem cells engraft to the bone marrow.

**Q What are personalized stems cells? What potential do they hold for patients?**

**A** A personalized stem cell is an adult cell, typically a skin cell. I can trick that skin cell into thinking it's an embryonic cell by transiently inserting some genes into it. Once the skin cell believes it's an embryonic

**Q What is the most exciting thing you have found studying embryos?**

**A** One of our most exciting discoveries has to do with a screen. We added a library of different chemicals to the fish water and found one chemical—Prostaglandin E2—could increase blood stem cells.

This was the very first small molecule in any system that could increase a stem cell. Next, we showed adding Prostaglandin E2 to a mouse's bone marrow would increase the number of stem cells that could engraft by four-fold. We moved to human cells and showed they also responded. We moved to clinical trials and treated 12 patients with leukemia. It worked in 10 of 12 patients. Now, Prostaglandin E2 is in a phase II clinical trial.

**Q What is the Innovation Tank? What excites you about the Innovation Tank?**

**A** This is Boston Children's spin on "Shark Tank." We're inviting aspiring innovators to pitch their concepts to a panel of judges during our Global Pediatric Innovation Summit + Awards—coming up in October—and compete for \$30,000 in startup funds.

I'm excited because I'm a big fan of ABC's "Shark Tank." It's captivating to see entrepreneurs pitch their ideas.

I have started two companies—Fate Therapeutics and Scholar Rock—which are doing very well. I have also been involved in trying to start a few companies that have not made it. I'm interested in sharing some of my wisdom in how to start a company, how to interact with venture capital firms, how to assemble the right group of people. I am very interested to see the ideas that get pitched.

I'm also a huge fan of Daymond John from ABC's "Shark Tank," and look forward to connecting with him at the Innovation Tank.

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# MASSACHUSETTS SEED-STAGE FUNDING AT CRITICAL JUNCTURE

## WHILE DEAL FLOW IS HEALTHY, 2014 WILL BE PIVOTAL YEAR TO ASSESS TRENDS IN FUNDING STARTUPS

Seed-stage funding in Massachusetts companies continues to grow, but 2014 investment must near \$200 million to sustain five-year averages and ensure a solid startup environment into the next decade, according to an annual industry report published by MassBio.

The 2014 MassBio Industry Snapshot shows Massachusetts leads in federal research funding and venture capital invested per capita, as well as research and development jobs, but that recent dips in seed-stage funding could negatively impact innovation long-term.

“Massachusetts’ strength in the life sciences depends on its vibrant startup and early-stage research activities, but funding for those activities—the fuel for industry growth here—is getting more difficult to come by,” said Robert K. Coughlin, President & CEO of MassBio. “The Industry Snapshot and MassBio’s recent Impact

2020 report demonstrate that we must find new avenues for companies to seeking seed funding to ensure life-changing treatments make it out of the ‘valley of death’ and into the hands of patients in need.”

In aggregate, Massachusetts seed-stage deals have grown in number and size since 1999. In the last five years, Massachusetts companies have received 33.4 percent of all seed funding for biotech in the U.S. Over the past decade, Massachusetts has outperformed the nation in percentage of deals and investment at the seed-stage as a percentage of total venture funding.

While this is positive news, there has been a drop in MA seed-stage funding since the 2008-2010 highs and 2014 figures will determine whether the current five-year average is equal to that of the prior five-year period.

Other industry statistics are trending positive and the industry’s economic impact as measured by MA-based payroll topped \$7.2 billion.

Massachusetts employment in the biopharma industry rose to 57,642 in 2013, an increase of almost 1,200 jobs over 2012, based on data from the U.S. Bureau of Labor Statistics’ Quarterly Census of Employment and Wages (QCEW). Employment in the industry has grown nine to ten times faster than state and national growth rates for employment.

Postings for open biopharma industry positions on MassBio’s online job board are trending up, with an average daily number of listings above 1,400 jobs in June and July of 2014. MassBio.org is not a comprehensive listing of all jobs available in the industry in Massachusetts but given its significant volume, it does provide a statistically strong sampling in determining job hiring trends.

Massachusetts still leads the nation in biotechnology R&D jobs, a segment of the larger industry employment, with more than 28,042 positions in 2013 and maintained its position as the leader in R&D in biotechnology, as defined by industry concentration.

In 2013, NIH funding was reduced by 12.2 percent nationally, but just 7.3 percent, or \$182 million in Massachusetts. While research in the Impact 2020 report shows biotech venture investment overall is trending downward, in 2013 venture investment in Massachusetts rose to \$984 million. Massachusetts received more than 21 percent of all VC investment in biotechnology in the U.S.

New this year, MassBio also analyzed the reach of Massachusetts-developed medicines. Massachusetts-headquartered companies have developed therapies that focus on patient populations of 232 million in the U.S. and more than 1.5 billion around the world.

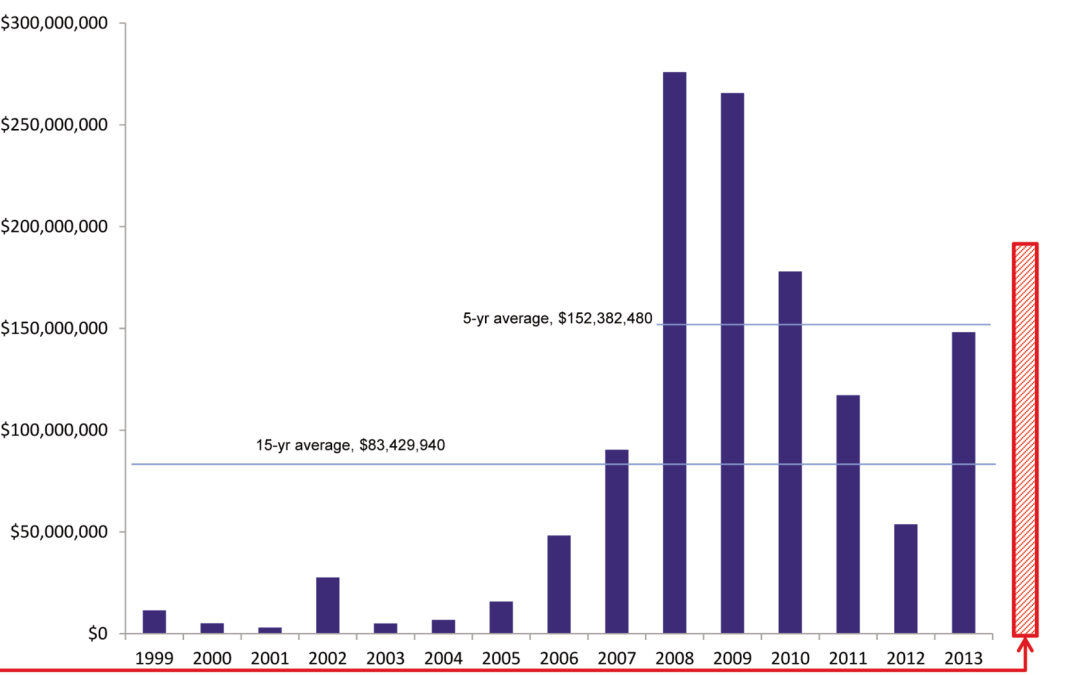
Additional highlights from the report include:

- Massachusetts-headquartered companies have a total of 1,384 drug candidates at some stage of R&D. Oncology drug candidates continue to make up 35 percent of that pipeline with systemic anti-infectives, central nervous system, and musculoskeletal therapeutic areas as other strong areas of research.
- Massachusetts accounts for 12 percent of the U.S.-based drug development pipeline.
- Massachusetts-headquartered companies account for 6 percent of the global biologics pipeline.
- With 14 IPOs through July 2014, Massachusetts has beaten its previous biotech/pharma annual IPO record of 9 (2013).
- The top five NIH-funded independent hospitals in the U.S. in 2013 are in Boston.
- On an NIH-funding per capita basis, Massachusetts continues to far exceed other leading NIH-recipient states. Only California receives more total NIH funding.

2014 will be a revealing year for seed funding. Will the state move above the 5-year trending in funding?

\$198,917,200 in seed funding in Massachusetts is needed for the 5-year period ending in 2014 to equal that of the prior 5-year period.

### MA Seed-Stage Funding - Annual, 1999-2013



- In the last year, more than 2.5 million square feet of commercial lab space has been added to inventory in Massachusetts.
  - The estimated average salary in the biopharma industry is \$125,056.
  - Nationally, biopharma manufacturing employment has declined by 3.5 percent since 2004. Massachusetts grew by 24.2 percent in biopharma manufacturing in the same time period.
- This year’s Snapshot was produced in partnership with EvaluatePharma, the premier source for commercial analysis of the pharma and biotech sector. Snapshot statistics are compiled annually by MassBio from sources including EvaluatePharma, National Institutes of Health, and the U.S. Bureau of Labor Statistics, the Quarterly Census of Employment & Wages and others.
- For more information on the Massachusetts cluster, or Impact 2020, visit [www.MassBio.org](http://www.MassBio.org).

## REPORT HIGHLIGHTS

◆ In the last five years, Massachusetts companies have received **33.4%** of all seed funding for biotech in the U.S.

◆ Massachusetts biopharma industry employment reached an all-time high of **57,642** in 2013, continuing the industry’s nine-year pattern of growth, and now accounts for more than **\$7.2 billion** in payroll.

◆ The estimated average salary in the biopharma industry is **\$125,056**.

◆ Massachusetts- headquartered companies have a total of **1,384** drug candidates at some stage of R&D. Oncology drug candidates make up **35%** of that pipeline.

◆ Massachusetts-headquartered companies have developed therapies that focus on patient populations of **232,434,000** patients in the United States and **1,507,722,000** patients around the world.

◆ The top **5** NIH-funded independent hospitals in the U.S. in 2013 are in Boston.

◆ Venture investment in Massachusetts rose to **\$984 million** in 2013. Massachusetts received more than **21%** of all VC investment in biotech in the U.S.

◆ With **14** IPOs through July 2014, the annual record for IPOs for Massachusetts biotech companies has been surpassed.

SEE THE FULL REPORT AT [MASSBIO.ORG](http://MASSBIO.ORG)

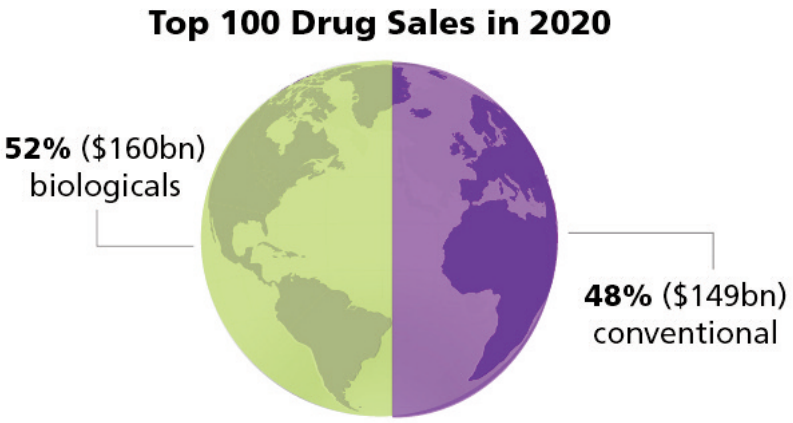
## World Preview 2014

### Outlook to 2020: Trends in Sales

**By 2020**  
Worldwide prescription drug sales forecast to be **\$1 trillion in 2020**

Compound Annual Growth Rate will be **5.1% between 2013 and 2020**

Biologicals will account for **52% of top 100 prescription & OTC drug sales in 2020 and 27%** of total worldwide market



## EvaluatePharma® publishes industry outlook

The seventh edition of life science market intelligence firm Evaluate’s annual pharma and biotech sector report was published in June. Here are some key highlights from the report which provides an industry outlook to 2020:

- Worldwide prescription drug sales forecast to exceed one trillion dollars in 2020 (CAGR: 5.1 percent between 2013 and 2020)
- In dollar terms, worldwide prescription drugs sales in 2013 relatively flat as the industry’s patent cliff tapers off
- Bumper year for new drug approvals in US: sales potential of \$24.4 billion, 43 percent higher than the class of 2012
- Value of industry’s R&D pipeline surges 46 percent to \$419 billion
- Bristol-Myers Squibb’s anti-PD-1 monoclonal antibody, Nivolumab, becomes the most valuable R&D product at \$23 billion
- Between 2014 and 2020, \$259 billion of sales at risk from patent expiration, but only 46 percent expected to materialize due to softer erosions of biological products
- Humira projected to be world’s largest selling product in 2020 with worldwide sales forecast to be \$12.7 billion

- Novartis becomes top company by worldwide Rx sales in 2013; Pfizer number two
- Novartis expected to remain number one, in terms of worldwide Rx sales, in 2020
- Worldwide pharmaceutical R&D spend forecast to be \$162 billion in 2020 (CAGR: 2.4 percent between 2013 and 2020)
- Oncology set to record highest worldwide sales growth of major therapy categories to 2020 (CAGR: 11.2 percent between 2013 and 2020)
- Within the top 100 prescription products in 2020, biological products expected to account for more than 50 percent of sales
- Teva Pharmaceutical remains leading generic drug maker in 2013
- Enterprise value of Gilead Sciences almost doubles over the course of the year

Full copies of the complimentary annual report can be downloaded at [www.evaluategroup.com/wp2014](http://www.evaluategroup.com/wp2014).

Source: 2014 EvaluatePharma® World Preview Report

# LIFE SCIENCES JOB POSTINGS REMAIN HIGH



Job postings in the life sciences industry remained at historic highs with more than 73,000 positions posted across the country last year, according to new research released by the Council of State Bioscience Institutes (CSBI) in partnership with the MassBioEd Foundation.

The 2014 Life Sciences Workforce Trends Report uses qualitative and quantitative data to assess current and projected talent needs in the life sciences industry.

The findings parallel a recent MassBioEd report on entry level jobs in life sciences in Massachusetts which can be found at [www.MassBioEd.org](http://www.MassBioEd.org).

Several themes emerged among life sciences industry leaders and hiring managers who participated in the interviews including the need for:

- Individuals with strong science skills

combined with multidisciplinary academic training and experience

- Regulatory professionals who can help bridge the gap between regulatory functions and business activities

- Scientists, engineers and clinicians who possess cross-functional skills that promote strong communication and the ability to interface well with both internal and external partners

- People with policy acumen who can help navigate health economics and the Affordable Care Act as well as influence legislators

- Strong and informed partnerships between academia and industry to provide tailored and relevant training to effectively meet industry needs

MassBio, MassBioEd and the Massachusetts Life Sciences Education Consortium (MLSEC) will use data from this report to guide future workforce development conversations and initiatives.

See the full CSBI press release and download the full report at [www.csbinstitutes.org](http://www.csbinstitutes.org).

Learn more about MassBioEd's initiatives at [www.MassBioEd.org](http://www.MassBioEd.org).

## PURCHASING UPDATE

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At MassBio, we know that if we can save members money on supplies and services, you're able to put more of your resources into research. That's why we work to bring you discounts through our Purchasing Consortium, which expanded this month to include Evaluate Ltd., Nature Publishing Group and the *Journal of Commercial Biotechnology*.

Evaluate offers MassBio members significant savings on its EvaluatePharma® subscription-based service that includes global R&D and commercial intelligence, consensus forecasts to 2020, financial data and valuation tools, and most recently USA drug price and annual cost per patient data. Members will also have access to Evaluate's award-winning EP Vantage news and email alerts, exclusive industry reports, Quick Start needs analysis and EvaluatePharma training.

Nature Publishing Group (NPG) offers MassBio members with an R&D count of under 100 special pricing on their Articles on Demand Platform and reduced site license pricing for the journals *Nature* and *Nature Biotechnology*. NPG publishes high-impact peer-reviewed research in all fields of science along with news and commentary on trends affecting science, scientists and the wider public.

The *Journal of Commercial Biotechnology* offers new MassBio members a free one-year digital institutional subscription and 20 percent off personal or institutional subscriptions for existing MassBio members. The journal publishes peer-reviewed, authoritative, cutting-edge articles written by the leading practitioners and researchers in the field, addressing topics such as management, policy, finance, law, regulation, and bioethics.

Learn more and take advantage of these new offerings at [www.MassBio.org](http://www.MassBio.org).

## PARTNERSHIPS TO GROW

BIOPHARM AMERICA: from Page 1

*You have accelerated from zero to 60 deals in less than a year. Isn't that overwhelming for the biotech community?*

**Urban:** We are reporting 60 collaborations through the Innovation Center model today. We are delighted by how our model has been received. You can see the scale of that with the number of relationships we have been able to create in a short time and our expanding network. Johnson & Johnson has linked these centers through a global platform such that the Boston Innovation Center is very similar in structure to the centers in Menlo Park, in London and Shanghai. To be clear, the number of announced collaborations is for the global network. From Boston we are responsible for the eastern half of the United States.

We have a broad range of interests across the entire healthcare spectrum with top technical people now embedded in the Innovation Center team representing three of the company's business groups for Consumer products, Medical Device & Diagnostics products, and Pharmaceutical products. In the pharmaceutical sector we have five core businesses, the neuroscience franchise, oncology franchise, the cardiovascular metabolism franchise, infectious diseases and

vaccines franchise and our immunology franchise.

*How does someone become part of this?*

**Urban:** Where we are approached with a program that is post-proof-of-concept, we put it in the hands of a business development team associated with the appropriate business. However, everything upstream of that stage is now managed through the teams that are inside the Innovation Centers. We want to participate in the very earliest elements of the ecosystem. We are getting involved in a range of projects that hopefully increase the efficiency of getting from early research to the early validating data to support subsequent financing.

*Why do the Innovation Centers make Johnson & Johnson different?*

**Urban:** We are coming at this in a new way. In the past this has not been so obvious, that a company of our size can be open to the type of collaborative working relationship that the innovation centers have now made possible. We are building a highly collaborative infrastructure to move upstream in a quite methodical way. Paul Stoffels is really passionate about the power of collaboration, about building a world laboratory and how the Innovation Centers can be a tremendous contributor to making sure more innovation is actually available and exploited than it has been in the past.

Registration for BioPharm America is now open and partnering has begun. Visit [www.MassBio.org](http://www.MassBio.org) for more information. MassBio members receive \$200 off of registration by using the code MassBio in the comments field.

Robert Urban's answers above were excerpted from partneringNEWS.



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Contact Larissa.Fawkner@massbio.org or  
617-674-5100 to learn more and set up a date!



**Nov.10 | Genzyme Center, Cambridge**

Join us for the first-ever MassBio Patient Advocacy Summit. We'll bring industry leaders together with patient advocates and other stakeholders to examine ways in which life sciences companies can more fully incorporate the patient voice into the work they do—not just approaching regulatory applications or at commercialization, but throughout the drug development cycle.

The day-long event will include four panel discussions, four case study presentations (spotlighting industry/patient partnerships), a keynote address, as well as a networking breakfast, lunch and cocktail reception.

Sponsorship opportunities are now available by contacting Elizabeth Steele at 617-674-5100 or [Elizabeth.steele@massbio.org](mailto:Elizabeth.steele@massbio.org). Registration will open Sept. 2. Visit [www.MassBio.org](http://www.MassBio.org) for details.

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# GREEN BUILDINGS SET THE NEW STANDARD



The Center for Life Science is equipped with energy sub-metering for users to monitor use.

Cambridge has joined Boston in enacting a building energy disclosure ordinance, under which owners of buildings more than 50,000 square feet will be required to report their energy use beginning in May 2015.

Life sciences spaces have higher necessary energy use than typical residential or office buildings, as they must accommodate complex air ventilation and water flow systems, as well as meet stringent safety requirements. But lab developers and users have long worked toward greater energy efficiency, and impressive developments in lab design and operations have made new lab spaces in Massachusetts showcase projects for replication throughout the world.

The Center for Life Science, an 18-floor research building in Boston, utilizes energy sub-metering that allows users to closely monitor use. It was no small effort; it required substantial investment in new systems, consensus protocols among users, and intensive data mapping.

“With more transparency and accountability, tenants are dialing down on their equipment usage,” said Peter Damiano, Sr. Facility Manager of BioMed Realty Trust, the company that owns the Center for Life Science. The system has changed operating conventions, reduced energy consumption, and become a roadmap for BioMed in improving efficiencies within its global building portfolio.

At MIT’s Koch Institute building in Cambridge, completed in 2011, cutting-edge efficiency design was at the forefront. The building is oriented east to west to maximize heat and light from the sun. Light-shelves bounce sunlight to the ceiling, bringing ambient light deep into the building to reduce dependence on electric lighting. Its ventilation system uses a “cascading design” by which office

cooling air is reused in lab hoods, air flow rates are at a reduced 80 feet per minute, and labs are aligned to reduce duct work. Electrical systems were “right-sized,” not overbuilt.

The results are striking. Anticipated 14.6 watts per square foot usage are at 3.8 watts instead. Steam heat that was projected at 35,000 pounds per hour for the coldest days is at 20,000 pounds. The building reduces total energy use by more than 30 percent as compared to a standard laboratory facility. Walt Henry, MIT’s Director of Engineering at the time, explained in an MIT News article, “To get a building that performs well requires only that you make intelligent choices.”

Intelligent choices like those made by Biogen Idec, which has already surpassed its goal of reducing its overall environmental footprint by 15 percent by 2015 even as it adds in facility square footage. Biogen Idec’s greenhouse gas intensity goal is to reduce Scope 1 and 2 emissions by 80 percent by 2020. Its two new Cambridge buildings achieved LEED Gold and Platinum certifications from the U.S. Green Building Council. Biogen Idec’s campus is powered by its cogeneration plant, which produces 75 percent of the campus’ electricity and 100 percent of its steam. Cogeneration has helped lower emissions by more than 150,000 metric tons of CO<sub>2</sub>e on the campus since 2006.

These examples of recent lab developments provide models in energy efficiency that set the standard and point the way for energy sustainability.

*MassBio seeks to share additional success stories and best practices as we collectively seek a sustainable future! Share your story by contacting Jessica Roche at [Jessica.roche@massbio.org](mailto:Jessica.roche@massbio.org).*

## UNIVERSAL PARTNERSHIPS ANNOUNCED AT BIO

Dr. Susan Windham-Bannister, President & CEO of the Massachusetts Life Sciences Center, joined Gov. Deval Patrick and Robert K. Coughlin, President & CEO of MassBio at the Massachusetts Pavilion at the BIO International Convention to announce the new Universal Partnerships Program. The program will provide grant funding to support Massachusetts companies who are forming milestone-based R&D collaborations with life sciences organizations throughout the world. Successful completion of the milestone is expected to contribute to the overall development of a new or significantly improved product or process intended for commercialization. Applicable collaborations must be in the fields of life sciences, which include biotechnology, pharmaceuticals, medical devices, diagnostics and bioinformatics.

*Learn more at [www.MassLifeSciences.com](http://www.MassLifeSciences.com).*



# PARENTS ARE CHAMPIONS FOR CHARLEY’S CAUSE

**PATIENT:** from Page 8

HT-100. Charley went back again this July for a second dose. Seckler said the drug has been well tolerated.

“When we acquired the drug, we reformulated it with a coating and delayed release so that it isn’t broken down in the stomach, and there are no side effects,” said Seckler.

Designed to decrease the dystrophy, or the inflammation and fibrosis, HT-100 also promotes muscle fiber regeneration. Seckler describes it as a “mini cocktail,” targeting three pathways of the disease.

“It’s going to be an essential ingredient in the ultimate

cocktail that will treat these patients,” said Seckler. “The holy grail—gene replacement therapy—is still several years away, at least. The strategy in the meantime is to keep these muscles as healthy as possible.”

Also in Akashi’s pipeline is DT-200, an oral SARM (selective androgen receptor modulator) with positive phase 1 clinical data that has broad potential for multiple neuromuscular diseases. The next step in development is to assess the effects of DT-200 in increasing muscle mass, strength and motor function in healthy volunteers. Successful outcomes in this clinical trial will lead to further development in DMD or other neuromuscular disorders.

“Right now, the mission is to do what we did with HT-100—find another diamond in the rough,” said Seckler.

Much of their progress has been possible because of the Food and Drug Administration Safety and Innovation Act (FDASIA), signed into law on July 9, 2012, which expanded the FDA’s authorities and strengthened the agency’s ability to expedite patient-driven therapies for rare diseases.

“Congress understands we need to change the way we develop treatments,” said Seckler. “In this case, DMD is the poster child, Charley’s Fund is the engine and Akashi is the vehicle. We just need the medical science to fuel us to the finish line.”

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**PATIENT  
PROFILE**

# CHAMPIONING CHARLEY'S CAUSE

BY MEAGHAN CASEY

For Dr. Benjamin Seckler and his wife, Tracy, the decision to become influential players in the biotech world was driven by one goal: to save their child's life.

"Nothing can motivate you more than racing against the clock," said Seckler.

Their 13-year-old son, Charley, is battling Duchenne muscular dystrophy (DMD), a genetic disorder characterized by progressive muscle degeneration and weakness. Children with DMD cannot produce dystrophin, a protein necessary for muscle strength and function. As a result, every muscle in the body deteriorates. Skeletal muscle deteriorates first, and respiratory and heart failure follow, often by the time patients are in their late teens or 20s.

Although Duchenne is the most common fatal genetic disorder to affect children, there is no cure.

"We're in business to go out of business," Seckler said. "The end game is to cure or convert this rapidly fatal disease into a chronic, manageable illness."

Charley was 3½ when he was diagnosed with DMD. Seckler described his muscular tone as loose, and as Charley got older he had delayed milestones when walking and balancing. A visit to the neurologist confirmed he had elevated levels of creatine kinase, an enzyme that leaks out of damaged muscle.

"With one blood test, our whole lives changed," said Seckler. "Almost immediately, we revved into high gear on two fronts: getting Charley the best possible medical care and finding a cure in time to save his life. It didn't take long for us to come to the conclusion that we needed to start a foundation to expedite research and a cure."

Charley's Fund was born four months later, with a sole mission of funding a cure or treatment for Duchenne. The foundation invests its money in translational research – research that focuses on moving science from the lab into human clinical trials. To date, the foundation has directed more than \$25 million to support that research.

Once Charley's Fund was established, Seckler, a physician and radiologist, took the next step and launched DART Therapeutics to develop new therapeutics to treat DMD. On June 24, the Cambridge-based company was renamed Akashi Therapeutics, merging the operations of DART and its subsidiary Halo Therapeutics into a single entity.

"Akashi is a novel partnership between biotechnology veterans and patient organizations combining industry expertise with focus and urgency to expedite drug development and access to innovative medicines," said Seckler.

The company has received fast track FDA status for its lead drug candidate, HT-100 (delayed-release halofuginone), an orally available, small molecule drug candidate intended to reduce fibrosis and inflammation and promote healthy muscle regeneration in boys with DMD. It is enrolling and dosing DMD patients in an ongoing clinical study to evaluate the safety and tolerability of increasing doses of HT-100, and explore trends in a range of efficacy endpoints. Preliminary clinical data on the first patient cohorts in the study were presented at the 2014 New Directions in Biology and Disease of Skeletal Muscle Conference, which was held June 29 to July 2.

Last summer, Charley and 11 other patients received their first dose of



Charley Seckler is pictured taking his first dose of HT-100, Akashi's lead compound. He is participating in the clinical trial at Johns Hopkins/Kennedy Krieger Institute.