

## LIFE SCIENCES FUTURE



**LIFE SCIENCES FUTURE  
BioPharm**  
OCTOBER 3, 2019

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## LIFE SCIENCES FUTURE - BIOPHARM

### HIGHLIGHTS INSPIRATION AND INNOVATION IN PA LIFE SCIENCES COMMUNITY

**King of Prussia, PA** – Life Sciences Pennsylvania hosted *Life Sciences Future - BioPharm* October 3 to bring together hundreds of attendees interested in the latest industry research and trends within the biotechnology and pharmaceutical fields. This event provided an opportunity for industry leaders to learn from and inspire one another as their collective effort drives life sciences innovation in the Commonwealth. The event featured partnering meetings, patient spotlights, and rare disease advocates whose stories personify industry accomplishments.

The program included an invitation-only discussion with Seth Joyner, former NFL linebacker for the Philadelphia Eagles and breast cancer patient advocate, during Breakfast in the C Suite.

David Fajgenbaum, MD, MBA, MSc, was this year's keynote speaker and he shared his awe-inspiring story about how he came to dedicate his life to uncovering new treatments for deadly hyperinflammatory disorders like idiopathic multicentric Castleman disease (iMCD). David was diagnosed with iMCD during medical school. After spending months in the hospital in critical condition, having been read his last rites, and having four deadly relapses, he is now in his longest remission ever due to a drug that he identified as a candidate treatment in the lab. He launched a clinical trial to evaluate this drug, sirolimus, in other treatment-refractory iMCD patients. Additionally, he recently published his memoir: *Chasing My Cure: A Doctor's Race to Turn Hope into Action* and was featured on *CNN*, *STAT* and *The Philadelphia Inquirer* to name a few.

Eli and Ella Vivian, Metachromatic Leukodystrophy (MLD) gene therapy patients gave a powerful account of their experience during the patient spotlight. The audience was captivated by the story of these two brave children, who were told that there was no cure or treatment for MLD. However, thanks to a Facebook connection, their family learned of a gene therapy trial in Italy. That study allowed Eli and Ella to become some of the first patients in the world to undergo gene therapy for their rare disease with Dr. Alessandra Biffi.

Guests networked and learned about new technologies throughout the day, in addition to meeting with companies pitching their business plans for potential investments and partnerships on the Presentation Stage. Major players including BrightEdge, Merck, Janssen, GlaxoSmithKline and Pfizer also gave reverse pitches to highlight the products and technologies they are seeking to fill their portfolios.

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LIFE SCIENCES FUTURE  
**BioPharm**  
OCTOBER 3, 2019

## EVENT RECAP

### FEATURED KEYNOTES & SPEAKERS



#### BREAKFAST IN THE C SUITE & CONFERENCE KICKOFF

**Seth Joyner**

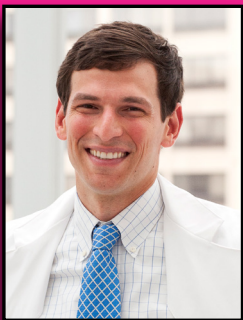
Former NFL Linebacker,  
Philadelphia Eagles  
Breast Cancer Patient Advocate



#### PATIENT SPOTLIGHT

**Eli and Ella Vivian**

Metachromatic Leukodystrophy  
Gene Therapy Patients



#### SPEAKER

**David Fajgenbaum, MD, MBA, MSc**

Assistant Professor of Medicine in  
Translational Medicine & Human Genetics,  
Penn Medicine

Co-Founder and Executive Director,  
Castleman Disease Collaborative Network  
(CDCN)

Castleman Disease Patient

Author, *Chasing My Cure: A Doctor's  
Race to Turn Hope Into Action; A Memoir*,  
Released September 10

503

TOTAL ATTENDEES

266

ORGANIZATIONS

15

STATES REPRESENTED  
+ D.C.

25   
POSTER  
PRESENTERS

103   
PARTNERING  
MEETINGS

88%  
RATED 'VERY  
GOOD' OR  
'EXCELLENT'

87   
FLU SHOTS



REVERSE  
PITCHES:



/BrightEdge





## CONTINUED FROM COVER

Attendees were able to listen in on various Knowledge Bursts throughout the day. Topics and speakers included cell and gene therapy in Pennsylvania with James M. Wilson, PhD, University of Pennsylvania, increasing success through genetics with Carolyn Buser-Doepner, GlaxoSmithKline, deal-making trends with Jon Civitarese, SVB Leerink LLC, and regulation of medical cannabis with John Collins, M.B.A., R.T.(R)(T), C.N.M.T., PA Department of Health.

“America’s Got Talent” semi-finalist Christian Guardino closed out the conference with a phenomenal speech and unforgettable performance during the closing plenary. Christian was born with a rare inherited genetic blindness. He regained his vision after receiving LUXTURNA, the first FDA-approved gene therapy, developed by researchers at the Children’s Hospital of Philadelphia (CHOP) and Spark Therapeutics.

LSPA thanks our sponsors and our planning committee for yet another successful event.



Christian Guardino, LUXTURNA patient, performing during the closing plenary



Seth Joyner and Dan Fitzpatrick, Citizens Bank, during Breakfast in the C Suite



The Vivian family and Seth Joyner





# PRESIDENT'S COLUMN



**CHRISTOPHER P.  
MOLINEAUX**

PRESIDENT & CEO  
LIFE SCIENCES PENNSYLVANIA

Life Sciences Pennsylvania was formed 30 years and strong as ever as the Pennsylvania Biotechnology Association for the purpose of connecting scientists, life sciences professionals, business leaders and others to encourage collaboration and unify the voice of the industry in Harrisburg.

When the organization originated in 1989, biotechnology and pharmaceuticals were the focus. These two life sciences components played a significant economic role, as they still do today, throughout the Commonwealth, and had particular concentration in and around the state's two largest cities - Philadelphia and Pittsburgh. Pennsylvania was, and continues to be, home to large and small companies and academic institutions pioneering innovative medicines for patients around the world. The Association's two founders recognized the importance of these innovators to our health, and to the vitality of communities around Pennsylvania, and started an organization to showcase that work to elected officials in Harrisburg. Those individuals knew that a common voice was needed to ensure public officials were informed about this growing and complicated sector of Pennsylvania's economy, and that creating new laws or processes could either enhance or impede the growth of the industry and innovation.

Much has changed about the Commonwealth and its life sciences community since this organization was formed, but Life Sciences Pennsylvania's mission expanded to include other facets of the life sciences community and remains largely unchanged - maintain public policy clout among our elected officials and facilitate strategic connections that advance the Commonwealth's life sciences community.

In fact, many of the same messages from the organization's founding still resonate with elected officials today - Pennsylvania's life sciences community is a leader in developing cures, therapies and technologies for patients, and these organizations continue to be a driver of economic growth for the Commonwealth.

Our public policy advocacy efforts, which now extend beyond Harrisburg to Washington, D.C., ensure our members are heard by those who make legislative decisions at every level of government. As Life Sciences Pennsylvania's membership continues to grow (842 members at the end of 2019), the organization has a louder and stronger voice than ever in both capitals.

Unfortunately, that voice has become increasingly necessary as we continue to find ourselves the target of bad public policy ideas.

For instance, the Medical Device Tax, which passed as part of the Affordable Care Act (ACA, otherwise known as "Obamacare") in 2010, imposed a 2.3% tax on medical device and diagnostic manufacturers. However, the funding generated by this tax did nothing to support the individual insurance marketplaces established by the ACA. Subsequently, the repeal of the tax became one of Life Sciences Pennsylvania's top advocacy priorities.

This innovation-inhibiting tax was in effect from 2013 to 2015 during which time the U.S. medical technology industry lost approximately 29,000 jobs. Due, in part, to our advocacy efforts and that of Pennsylvania's medical technology community, the tax was suspended from 2016 to 2018 and again from 2018 to 2020. Finally, on December 20, 2019, eleven days before it was set to go back into effect on January 1, 2020, it was permanently repealed as part of a deal to fund the government for the coming year.

While we're extremely relieved it has been permanently repealed, it took almost 10 years to reverse this poorly conceived policy. Given the glacial pace at which items tend to move in our legislative bodies this is not entirely uncommon, but it is a reminder that a clear and consistent voice - like the one this association was created to provide - is crucial to our advocacy efforts.

Through multiple congresses and significant turnover in our congressional delegation, I am happy to say the Medical Device Tax was reversed with significant support from the Pennsylvania delegation. In the end, 13 (of 18) U.S. House members from Pennsylvania co-sponsored the legislation to repeal the tax, and both U.S. Senators from PA co-sponsored the companion bill.

It cannot be overstated that the consistent, bipartisan and strong support from the Pennsylvania delegation was thanks to the unified voice with which Life Sciences Pennsylvania and our medical technology community spoke to legislators. It was very clear that this tax would divert resources away from investing in new technologies to improve the lives of patients and would require companies to either lay-off current employees or delay hiring new employees.

While it may have been a different time, and in a different capitol building, the messages of improving the lives of patients and economic growth continue to resonate with elected officials - 30 years after this organization's founding.

As we reflect on the previous three decades at Life Sciences Pennsylvania, we continue to build upon the tenets on which this organization was founded. Even in the midst of policy and political attacks, the Commonwealth's life sciences community offers the hope of groundbreaking medicines and technologies to patients around the world. A continued focus on collaboration and our persistent and collective voice will propel Pennsylvania's life sciences into the next thirty years.



# ADVOCACY

## FEDERAL

2.3%

### MEDICAL DEVICE TAX

The permanent repeal of the Medical Device Tax was included in a year-end government funding package that was signed by the President December 20, 2019. Barring any action by Congress, the Medical Device Tax would have gone back into effect on January 1, 2020. The deal to repeal the tax was part of a compromise reached on permanently repealing two other Affordable Care Act (Obamacare) taxes: the Cadillac Tax and the Health Insurance Tax.

The repeal of this 2.3% tax is the result of many years of advocacy activity by Life Sciences PA, our medical device and diagnostic member companies, and our national partners AdvaMed and MDMA – all of whom consistently pushed for permanent repeal of this innovation-inhibiting tax. From its inception in 2010 to implementation in 2013 and suspensions in 2016 and 2018, it has been nearly a decade since the tax has been enacted. This is a tremendous development for our membership and is the direct result of the hard work, leadership and tireless support of Pennsylvania's medical technology community.



### ROUNDTABLE WITH CONGRESSWOMAN SCANLON

In partnership with BioAdvance, Life Sciences PA held a roundtable discussion with Congresswoman Mary Gay Scanlon (D-PA 5th District) October 1. The discussion featured several BioAdvance portfolio companies and early stage life sciences companies from the district the Congresswoman represents. The discussion was intended to give the Congresswoman a better understanding of the life sciences makeup in her district, and provide her with background on the time, effort and resources it can take to bring a new medicine or device to patients. As Speaker Pelosi's drug pricing plan, H.R. 3, had just been introduced, the Congresswoman had several questions about how the proposals put forth in that legislation affect small and growing life sciences companies.



RADNOR – (clockwise from left) Martin Philips, Opsidio; Carol Burns, Cagent Vascular; Al Gianchetti, XyloCor; Chris Molineaux, LSPA; Cong. Scanlon; Malcolm Yates, Ofc. of Cong. Scanlon; Barbara Schilberg, BioAdvance; Eric Sugalski, ArchiMedic; Wes Kaupinen, Palvella; Mark Land, Boiron)



### AMERISOURCEBERGEN TOWN HALL WITH CONGRESSWOMEN DEAN AND SCANLON

Life Sciences PA was pleased to attend a town hall discussion with Congresswomen Madeleine Dean (D-PA 4th District) and Mary Gay Scanlon (D-PA 5th District). The discussion, which included about 100 attendees, provided AmerisourceBergen employees with a unique opportunity to hear directly from two of Pennsylvania's federal elected officials. The Congresswomen gave an update on several prominent issues in Congress, and took time to discuss drug pricing, and some of the policy proposals put forth to address it.



CONSHOHOCKEN – Cong. Scanlon and Cong. Dean speak to AmerisourceBergen employees

## FEDERAL CON'T



## NEW FDA COMMISSIONER

Stephen M. Hahn, MD was sworn-in as the newest Commissioner of the Food and Drug Administration on December 17, 2019, after being confirmed by the U.S. Senate December 12. At the helm of the FDA, he will oversee 17,000 federal employees and a budget of \$5.7 billion. He is replacing Scott Gottlieb, MD who retired last year. Most recently, Dr. Hahn served as the Chief Medical Executive of the University of Texas MD Anderson Cancer Center in Houston. Dr. Hahn is a Philadelphia native, and prior to his time at MD Anderson Cancer Center, held positions in radiation and medical oncology at the University of Pennsylvania. He also received his medical degree from Temple University.



Stephen M. Hahn, MD





## HR 3

Disappointingly, the U.S. House passed H.R. 3 – *Elijah E. Cummings Lower Drug Costs Now Act* December 12, 2019. This legislation was introduced September 19, 2019 by U.S. Speaker of the House Nancy Pelosi, and other Democratic leadership. It passed largely on a party line vote with 230 House members voting for it and 192 voting against it. While the plan does institute some policies to reduce out-of-pocket pharmacy costs, we are concerned it will ultimately inhibit innovation and limit patient access to the groundbreaking therapies and cures our member organizations are working to develop.

Life Sciences PA worked with many of its Federal Policy Committee members and member companies to send letters to and hold meetings with representatives, and their staffs, in the Pennsylvania House Delegation to express our concerns about this bill.

It is important to note that this bill is unlikely to move beyond the House. There does not appear to be any appetite in the U.S. Senate to take up this legislation or any legislation closely resembling it.

For further background, here is a list of how the Pennsylvania delegation voted:

<b>In favor of H.R. 3</b> 	<ul style="list-style-type: none"> <li>• Boyle</li> <li>• Cartwright</li> <li>• Dean</li> <li>• Doyle</li> <li>• Evans</li> <li>• Fitzpatrick</li> <li>• Houlahan</li> <li>• Lamb</li> <li>• Scanlon</li> <li>• Wild</li> </ul>	<b>Opposed to H.R. 3</b> 	<ul style="list-style-type: none"> <li>• Joyce</li> <li>• Keller</li> <li>• Kelly</li> <li>• Meuser</li> <li>• Perry</li> <li>• Reschenthaler</li> <li>• Smucker</li> <li>• Thompson</li> </ul>
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## USMCA

After it was announced that an agreement was reached between U.S. House lawmakers and the Trump Administration on the U.S.-Mexico-Canada Agreement (USMCA), sometimes referred to as NAFTA 2.0, the House passed the updated agreement on December 19, 2019. The Senate is expected to ratify this agreement in early 2020. Life Sciences PA was supportive of the USMCA and its provisions strengthening patent and other IP protections while also increasing market access and regulatory standards in Canada and Mexico. Specifically, it granted biologic medicines 10 years of exclusivity in those two countries. Unfortunately, the final agreement did not include these provisions germane to Pennsylvania's life sciences industry.

## SUPPORTED PATIENT ADVOCACY EVENTS

To learn about upcoming events in 2019, see page 20 or visit <http://www.lifesciencespa.org/connect/events/>



## FEDERAL CON'T



## BUDGET DEAL INCREASES FUNDING IMPORTANT TO THE LIFE SCIENCES

Before recessing for the holidays, President Trump signed two spending bills that will keep the government operating through September 30, 2020. Government funding was set to expire at 11:59 PM on Friday, December 20. The \$1.4 trillion spending package funds many facets of government operations, but there are several specific, positive changes that affect Pennsylvania's life science community:

- National Institutes of Health (NIH) will receive a \$2.6 billion increase (7%) that will bring its total budget to \$41.7 billion. Life Sciences PA is pleased to see this increase, which marks the fifth year in a row of multibillion-dollar increases for the NIH.
- National Science Foundation (NSF) saw a \$203 million increase (2.5%) that will bring its total budget to \$8.28 billion.
- Centers for Disease Control and Prevention (CDC) will see a \$636 million increase that will bring its total budget to \$8 billion - this includes additional funding to fight antibiotic resistance and a bill, the End Neglected Tropical Diseases Act, meant to incentivize investment in medicines for neglected tropical diseases.
- Food and Drug Administration (FDA) will receive a \$91 million increase (3%) that will bring its total budget to \$3.16 billion - \$75 million of which is earmarked for advancing expedited medical product development and review as put forth in the 21st Century Cures Act.

## STATE



## LIFE SCIENCES EDUCATIONAL BRIEFING

Chris Molineaux, President and CEO of Life Sciences PA, convened an informational briefing on the life sciences industry and its footprint in Pennsylvania for members of the General Assembly and staff in Harrisburg in October. With 50 new legislators in the 2019 Pennsylvania General Assembly it was, and continues to be, important for Life Sciences PA to ensure new and incumbent elected officials have a strong understanding of Pennsylvania's life sciences community - specifically, what it means for patients and for the Commonwealth's economy. Additionally, intimate conversations like these allow for candid conversations on priority issues for our member organizations. Life Sciences PA was pleased to have many of our Life Sciences Caucus Co-Chairs from the Senate and the House in attendance and appreciates their assistance in organizing the briefing.



HARRISBURG - (clockwise from left) Rep. Toepel; Sen. Mensch; Rep. Longiotti; Rep. Gaydos; Chris Molineaux, LSPA; Rep. Thomas; Rep. Schroeder; Sen. Stefano



## VACCINE ROUNDTABLE WITH PA HEALTH SECRETARY

Life Sciences PA was pleased to meet with Pennsylvania Secretary of Health, Rachel Levine, MD on October 30. Given much of the disinformation swirling around public officials on vaccines, Life Sciences PA thought it beneficial to organize a meeting with Secretary Levine and public health experts from the University of Pittsburgh, Pennsylvania State University and the Children's Hospital of Philadelphia. Secretary Levine is a pediatrician and is clearly committed to educating Pennsylvanians on the importance and safety of vaccinations. In fact, Pennsylvania vaccination rates are among the best in the country, but it was important to have a discussion about where there may be room for improvement. Life Sciences PA stands ready to work with the PA Department of Health, where appropriate, to ensure the public has access to and is adequately informed about the importance of vaccinations.



Rachel L. Levine, MD

# EVENTS

## CONNECT@ COOK MYOSITE

OCTOBER 17, 2019 | PITTSBURGH, PA

Sixty guests gathered at Cook MyoSite's Pittsburgh campus for a Connect@ LSPA members gathering. Guests networked with members in the region and heard remarks from Dr. Michael Chancellor, Director of Neurourology Program in the Department of Urology at the William Beaumont Hospital in Royal Oak, Michigan, and the co-founder of Cook MyoSite.



Guests at Cook MyoSite enjoy conversation and refreshments

## SUPPLY CHAIN SUMMIT

OCTOBER 29, 2019 | WAYNE, PA

Nearly one hundred people in the supply chain industry gathered for Life Sciences Pennsylvania's annual Supply Chain Summit October 29 in Wayne for an educational and informative day. Attendees heard from leaders in the industry, who discussed what the supply chain looks like today, good practices and solutions to implement, blockchain, and key considerations for cell therapy in supply chain.

The day ended on a sweet note with master chocolatier and owner of Éclat Chocolate, Christopher Curtin, discussing how a small business sources and ensures quality from cocoa bean growers around the world.



Attendees congenially chatting at the 2019 Supply Chain Summit

## CFO & CEO DINNER

NOVEMBER 7, 2019 | KING OF PRUSSIA, PA

Chief Financial Officers and Chief Executive Officers from Life Sciences Pennsylvania's core member companies convened for a relaxing evening to meet new colleagues, reconnect with familiar ones and hear from guest speaker Jeffrey Aronin, Chairman and CEO of Paragon Biosciences. Jeffrey has invested in and launched several successful portfolio companies, including LSPA member, Harmony Biosciences.



Attendees engaging with one another at St. Davids Golf Club in Flourtown

## @LSPA OPEN HOUSE

NOVEMBER 12, 2019 | WAYNE, PA

Life Sciences Pennsylvania hosted an open house for the life sciences community to tour the state-of-the-art 8,600 square foot **@LSPA - The Life Sciences Center** while networking and enjoying coffee. The Center offers members space to host educational events and happy hours, as well as secluded rooms for phone calls or meetings. If you are interested in utilizing The Center, please email [thecenter@lifesciencespa.org](mailto:thecenter@lifesciencespa.org).

## CRO & CMO SUMMIT

NOVEMBER 22, 2019 | WAYNE, PA

Life Sciences Pennsylvania welcomed 131 guests to West Pharmaceuticals in Exton, PA, for its fifth annual CRO and CMO Summit to educate, network, and showcase talent and trends within Pennsylvania's life sciences community. Heather Malicki, PhD, Executive Director, Analytical Development & Testing Technologies, Wuxi Advanced Therapies; Erin Van Winkle, Senior Director, Clinical Programs, Adaptimmune; and Alex Lynes, PhD, Manager, Research and Technology, West Pharmaceutical Services, Inc. discussed current topics in the contract research and contract manufacturing industry. Keynote speaker Dr. Usman Azman, president and CEO of Tmunity Therapeutics, also gave a captivating presentation on the history and future of T Cell immunotherapy.



Presentations on industry topics being given at the CRO & CMO Summit



## CEO DINNER

DECEMBER 3, 2019 | ARDMORE, PA

Leaders in the life sciences industry gathered for Life Sciences Pennsylvania's annual Chief Executive Officer Dinner, held at the Merion Golf Club in Ardmore. Daniel J. Hilferty, chief executive officer of Independence Health Group, one of the nation's leading health insurers, gave powerful remarks about his experiences in the health care industry.



Dan Hilferty, CEO of Independence Health, addressing the crowd at the CEO Dinner

## BASICS OF BIOTECH

DECEMBER 11-12, 2019 | WAYNE, PA

About two dozen people from a range of professions spent December 11 and 12 at the LSPA office learning about the basics of biotechnology and drug development. Jason Carstens, PhD, and Ben Fryer, PhD, respectively the COO and CEO of Pluristyx, Inc, co- led the workshop. Beginning with the fundamentals of cell biology, through the broad categories of therapies and the history of select now-common treatments like insulin, Carstens and Fryer gave a detailed course on how a drug moves from discovery to commercial availability. Their audience, which ranged from chemical engineers to finance professionals, gained a deeper understanding of both the scientific and industrial considerations involved, including cell and gene therapy development and manufacturing.



Guests diligently taking notes during the workshop at the LSPA office

## LUNCH PANEL – GRANT ME THE MONEY

DECEMBER 12, 2019 | PITTSBURGH, PA

Leading industry players, Michael Emery of Tyber Medical, Jim Fendrick of Rockland Immunochemicals and Kelly Wylam of Innovation Partnership, shepherded a conversation about Pennsylvania-specific grants and tax incentives, approaches to securing SBIR & STTR funding, free education and guidance opportunities to help win grants, and other funding opportunities.



Discussions ensuing over lunch at the Hofbräuhaus Pittsburgh Brewery & Restaurant

## HOLIDAY MIXER HOSTED BY LSPA BOARD OF DIRECTORS

DECEMBER 18, 2019 | WAYNE, PA

More than 200 guests attended the annual Holiday Mixer Hosted by LSPA Board of Directors December 18 in **@LSPA - The Life Sciences Center**. The life sciences community welcomed LSPA's 118 new members, met the Board of Directors and celebrated together as we closed out the year (and decade!).



A full house socializing and celebrating the quarter's close at the 2019 Holiday Mixer at **@LSPA - The Life Sciences Center**



## LSPA MEMBERS FEATURED IN PBJ'S POWER 100 AS PA'S MOST INFLUENTIAL

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LSPA is proud to announce that 27 members appeared on Philadelphia Business Journal's Power 100 list. Congratulations to all those who have displayed influence, respect, skill and effectiveness, including:

- **Madeline Bell,**  
President & CEO, Children's Hospital of Philadelphia
- **Tim Block,**  
President of the Hepatitis B Foundation,  
Baruch S. Blumberg  
Institute and Pennsylvania Biotechnology Center
- **Mike Bowman,**  
CEO, Valley Forge Tourism and Convention Board
- **Deanna Byrne,**  
Philadelphia Office Managing Partner,  
PricewaterhouseCoopers
- **Julie Coker Graham,**  
President & CEO, Philadelphia Convention  
& Visitors Bureau
- **Steven Collis,**  
Chairman and CEO, AmerisourceBergen Corp.
- **John Fry,**  
President, Drexel University
- **Amy Gutmann,**  
President, University of Pennsylvania
- **Michael Heller,**  
Executive Chairman and CEO, Cozen O'Connor
- **Daniel J. Hilferty,**  
CEO, Independence Health Group
- **Ali A. Houshman,**  
President, Rowan University
- **Dr. Carl June,**  
Director of Translational Research at the Abramson  
Cancer Center, University of Pennsylvania
- **Dr. Stephen K. Klasko,**  
President, Thomas Jefferson University and CEO,  
Jefferson Health
- **Kevin Mahoney,**  
CEO, University of Pennsylvania Health System
- **Jeff Marrazzo,**  
CEO, Spark Therapeutics
- **Christopher P. Molineaux,**  
President & CEO, Life Sciences Pennsylvania
- **Henry Nassau,**  
CEO, Dechert
- **George Norcross,**  
Executive Chairman, Conner Strong & Buckelew
- **RoseAnn Rosenthal,**  
CEO, Ben Franklin Technology Partners of  
Southeastern Pennsylvania
- **Bill Sasso,**  
Chairman, Stradley Ronon Stevens & Young
- **Gary W. Smith,**  
President & CEO, Chester County Economic  
Development Council
- **Gerard H. Sweeney,**  
CEO, Brandywine Realty Trust
- **Ellen Weber,**  
Executive Director, Temple University Fox School  
of Business Innovation and Entrepreneurship  
Institute; Executive Director, Robin Hood Ventures  
Philadelphia
- **Dr. James M. Wilson,**  
Director of the University of Pennsylvania  
Gene Therapy Program
- **Jami Wintz McKeon,**  
Chairwoman, Morgan Lewis & Bockius
- **Rob Wonderling,**  
President & CEO, Chamber of Commerce  
for Greater Philadelphia
- **Steven Zarrilli,**  
President & CEO, University City Science Center



# MEMBERSHIP

## LIFE SCIENCES COMPANIES CONTINUE TO GROW AND PROSPER IN PA SEVERAL COMPANIES ANNOUNCE THE OPENING OF NEW LOCATIONS STATEWIDE

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This quarter has been marked by notable expansion and growth throughout the state. This is one of many ways the life sciences community is flourishing in Pennsylvania. From brand new research facilities and lab spaces to specialized health centers – each new opening allows our community to take another step toward scientific breakthroughs, health and wellness.

### INTEGRAL MOLECULAR DOUBLES FOOTPRINT IN UNIVERSITY CITY WITH SECOND RESEARCH FACILITY



In light of an uptick in antibody discovery partnerships and its ever-growing therapeutic antibody pipeline, Integral Molecular has expanded to a second location in Philadelphia featuring state-of-the-art lab space and equipment to facilitate antibody research.

Philadelphia Mayor Jim Kenney attended the opening of the new facility in October. “We are thrilled to see Integral Molecular’s expansion and continued success in Philadelphia,” Mayor Kenney said. “Integral Molecular’s growth represents the extraordinary innovation, job creation, and retention of talent in the vibrant and flourishing University City biotechnology hub.”

### PENN MEDICINE BREAKS GROUND ON \$48M PROTON THERAPY CENTER



A new proton therapy center, the second in Pennsylvania to offer this sophisticated form of radiation therapy, is slated to open in the fall of 2021. Penn Medicine Lancaster General Health started construction on the \$48 million facility in October as part of its Ann B. Barshinger Cancer Institute. The center will be housed in a nearly 8,000-squarefoot, four-story building located adjacent to the existing cancer institute.

Proton therapy protects healthy tissue from the harmful effects of radiation by varying the dose of radiation in the body to more accurately target the cancer. “Current patients who may benefit from proton therapy — especially for hard-to-treat cancers — can only receive this therapy at a handful of specialized centers across the country. This project represents the next phase of proton therapy, further enhancing patients’ access,” said James Metz, MD, the chairman of radiation oncology at Penn Medicine. This new project not only expands patient access to topnotch care, but it is also a reflection on PA’s growing talent and investment in proton therapy and cancer care.

### TYBER MEDICAL EMBARKS ON \$8M EXPANSION PROJECT



In early December, Tyber Medical celebrated its \$8 million project to expand its manufacturing facility with a ribbon cutting ceremony. The medical device company will use the new structure to support its efforts in orthopedics. This helps further establish the company’s strong presence in Lehigh Valley as a major employer.

The company was joined by public officials at the ceremony, including Secretary Dennis Davin of the PA Department of Community and Economic Development. “This expansion is a testament to their success and shows a commitment to continue bringing opportunity to Pennsylvanians in the surrounding community,” Davin said. Tyber’s expansion project, like other companies in this roundup, paints a picture of a vibrant community of likeminded individuals committed to the life sciences, their patients and novel discoveries facilitated by statewide growth.

## ACROSS DISEASE AREAS, NEW DATA AND SCIENTIFIC ADVANCES RISE IN NUMBER

FROM PATENTS TO CITIZEN PETITIONS, MEMBER COMPANIES MAKE HEADWAY TOWARD NEW TREATMENT OPTIONS

The member companies of Life Sciences Pennsylvania consistently showcase their commitment to science, innovation and improving patient outcomes. This quarter is no exception. Over the past several months, there have been major wins across various disease areas. While some companies won appeals and sought approvals, others launched brand new divisions and earned grants to continue their groundbreaking research.

### KIBOW BIOTECH LAUNCHES PHARMA DIVISION TO DEVELOP DRUG FOR KIDNEY DISEASE



On the heels of its 22nd anniversary in October, Kibow Biotech announced that it would create a wholly owned pharmaceutical division to further its mission to support kidney and gut health. Previously, the company focused on the research and development of kidney health dietary supplements. These supplements help to offset failing kidney function by utilizing the microbiome of the bowel to remove toxins.

In addition to the prebiotics, probiotics and multi-fibers currently in its portfolio, Kibow has developed a Live Biotherapeutics (LBP) formulation designed to be scientifically advanced as a U.S. FDA approved and clinically validated pharmaceutical product. Currently, the company is entering the Investigational New Drug (IND) process and planning clinical trials. If approved, this will be the first LBP drug for chronic kidney disease patients in the country.

### ZYNERBA RECEIVES PATENT FOR CANNABIDIOL DRUG CANDIDATE FOR FRAGILE X SYNDROME



In November, Zynerba was issued a patent for its synthetic cannabidiol medicine by the U.S. Patent and Trademark Office. The patent expands the company's intellectual property portfolio to include Zygel, an experimental gel therapy that is applied to the skin.

Zygel is now in late-stage clinical trials with 204 patients ages three to 17. The drug is being evaluated as a potential treatment for fragile X syndrome, a genetic condition causing developmental issues like learning disabilities and cognitive impairment. These clinical trials demonstrate the life sciences community's dedication to uncovering new data and treatments for patients.

### CORIELL INSTITUTE MOVES TO NEXT PHASE FOR PERSONAL GENOME INFORMATION PROJECT



In other data news, twelve years after its inception, the Coriell Institute for Medical Research has announced that it will enter a new phase in its DNA project. The goal is to understand how to use personal genome information in clinical decision-making. Existing reports on the topic discuss how an identified genetic risk for certain cancers or heart diseases can serve as a motivator for preventive or healthy behaviors.

Over 7,000 participants have volunteered for this study, which will require them to share their DNA samples, lifestyle choices and medical histories for analysis. Each participant receives personalized health reports including information about genetic and non-genetic risks for diabetes, obesity, heart disease, drug responses and interactions and more.

The institute will begin conducting more extensive research to decipher the data it has gathered. "The ultimate goal is to give physicians tools to be able to deliver care that is tailored to meet the needs of each individual patient," said the late Michael Christman, former CEO of the Coriell Institute.



## WISTAR INSTITUTE RESEARCHER GETS \$2.6M GRANT TO STUDY LINK BETWEEN DIET, ALCOHOL AND CANCER



Zachary T. Shug, PhD, of The Wistar Institute received a \$2.6 million dollar grant from the National Institutes of Health (NIH) to continue his research on the metabolic link between high sugar and fat diets, alcohol consumption and cancer.

Obesity and heavy drinking are two of the largest risk factors for cancer, and this research will seek to uncover how diet and alcohol affect acetate metabolism and gene expression in cancer, as well as understand the role of microbiota. "Zachary's research has the ambitious goal of unraveling the diet-induced metabolic and epigenetic changes that underlie tumor growth and progression, and may have important clinical implications," said Dario C. Altieri, MD, Wistar's president and CEO.

## BRAEBURN SEEKS APPROVAL FOR OPIOID ABUSE DISORDER DRUG FOLLOWING CITIZEN PETITION



Braeburn Inc. will now seek marketing approval from the FDA for Brixadi, the company's opioid abuse disorder treatment, after the FDA granted its citizen petition to revoke Indivior's orphan designation of buprenorphine. If approved, Braeburn will be able to market Brixadi as of December 2020.

"On behalf of patients and their families, we are grateful to the FDA for allowing additional treatment options to become available sooner," said Mike Derkacz, CEO of Braeburn.

Previously, the FDA classified opioid abuse disorder as an "orphan disease," or a rare condition deemed not profitable for pharma companies to treat. This gave Indivior market exclusivity over opioid abuse treatments. However, Braeburn argued that, by definition, orphan diseases are those that affect less than 200,000 people and over two million Americans have been diagnosed with opioid abuse disorder.

## RECRO PHARMA WINS FDA APPEAL OF NEW DRUG APPLICATION FOR NON-OPIOID PAINKILLER



Malvern specialty pharmaceutical company Recro Pharma won an appeal of its new drug application (NDA) for intravenous meloxicam, which was previously rejected by the FDA. IV meloxicam is intended to be used as a non-opioid treatment for the management of moderate-to-severe pain for patients in hospital or acute-care settings.

The FDA has rejected the NDA twice due to failure to meet prescriber expectations around the delayed onset of IV meloxicam, regulatory concerns about the role of IV meloxicam as a monotherapy in acute pain, prescription information labeling negotiations and how it would meet patient and prescriber needs in acute care settings.

## NOVARTIS RECEIVES FDA APPROVAL FOR SICKLE CELL TREATMENT ADAKVEO



Novartis announced in a November press release that Adakveo, a medicine for sickle cell disease, received approval from the FDA. Adakveo offers hope to both adult and pediatric patients 16 years and older with sickle cell disease. The approval decision comes as a result of promising data from a 52-week, randomized, placebo-controlled trial.

"We know this drug can decrease the frequency of sickle cell pain crises in a significant and clinically meaningful way," said Dr. Kenneth Ataga, Director of the Center for Sickle Cell Disease, University of Tennessee Health Science Center at Memphis, and Principal Investigator of the SUSTAIN trial. "The approval of [Adakveo] is an important advancement for people living with this very difficult condition."

An estimated 100,000 people, mostly of African descent, are living with sickle cell disease in the US and the complications associated with the disease can often be life threatening. According to the 2018 Census, 12% of the population in PA and 43.7% in Philadelphia are African Americans. Adakveo therefore has the potential to touch many lives in our community.

## NEW PARTNERSHIPS DEMONSTRATE COLLABORATIVE NATURE OF SCIENTIFIC COMMUNITY

DEALS BETWEEN SEVERAL PA PLAYERS SUGGEST BIG THINGS FOR COMING YEARS

No breakthrough happens without collaboration between scientists, pharmaceutical companies, partners and stakeholders. The very nature of the life sciences community requires cooperation from numerous players in order to achieve significant change and scientific advancements. The companies below have entered deals that will undoubtedly promote research, development and discovery in Pennsylvania and beyond.

### TMUNITY THERAPEUTICS AND ONCORA MEDICAL COLLABORATE TO IMPROVE CANCER CARE



Two Philadelphia-based member companies have come together to work toward advancements in cancer care. The deal grants Tmunity access to Oncora's proprietary data platform. This will allow the companies to blend their skills in T-cell immunotherapies and oncology analytics to improve the design and execution of clinical trials to develop next-generation CAR-T therapies.

"We believe that use of real-world data and evidence has the potential to change the way cancer therapies are developed and to further personalize outcome predictions that will guide treatment decisions," said David Lindsey, Oncora's CEO and co-founder.

### SPIROVANT SCIENCES GETS NEW OWNER IN \$3B DEAL



Spirovent Sciences, a small Philadelphia gene therapy company focused on cystic fibrosis, has found itself part of a proposal for a \$3 billion deal with five larger biopharmaceutical companies. In April, Spirovent, formerly Talee Bio, was acquired by Roivant Sciences. Roivant has signed an agreement with Japanese company Sumitomo Dainippon Pharma, which would transfer ownership of five of Roivant's companies to Sumitomo.

Spirovent, Myovant, Urovent, Enzyvant and Altavant will then be folded into one new company owned by Sumitomo. These companies specialize in women's health, prostate cancer, urinary diseases, rare respiratory diseases and rare pediatric diseases. Additionally, Sumitomo will take stakes in Roivant's outstanding shares and have options to acquire up to six more biopharm companies. Together the 11 companies have more than 25 clinical programs and several potential products in their pipelines expected to launch from 2020 to 2022.

### ROCHE ACQUIRES PHILLY-BASED SPARK THERAPEUTICS IN DELAYED \$4.3B DEAL



The Federal Trade Commission officially cleared Roche's offer to acquire gene therapy company Spark Therapeutics in December, after 10 months of investigation and deadline extensions. Roche pushed the deadline back nearly ten times since the announcement of the \$4.3 billion offer was first extended in February 2019.

Spark Therapeutics is a Philadelphia-based company with offices in the University City Science Center. The company was created out of development efforts for commercial treatments for hemophilia using gene therapy, an area that Roche has cited as a strategic business interest.

### ACHILLION PHARMACEUTICALS ACQUIRED BY BOSTON COMPANY IN \$930M DEAL



Achillion Pharmaceuticals, a Connecticut-based pharmaceutical company developing therapies for orphan diseases, will be acquired by Alexion Pharmaceuticals Inc. in a \$930 million dollar deal. However, the deal still requires approvals by Achillion's stakeholders and regulators and until then the companies will continue to operate separately.

Achillion is relatively new to the Pennsylvania life sciences community. The company's CEO and 30 employees moved their offices to Blue Bell in the winter of 2018.



## GENOMIND AND SHARECARE COLLABORATE TO IMPROVE MENTAL HEALTH OUTCOMES FOR PATIENTS



In December, Genomind and Sharecare teamed up to combine their mental health and digital health specialties to improve the field of mental health and wellness. Soon, Sharecare will offer patients and providers access to Genomind's mental health education and pharmacogenomic (PGx) services via the digital health and wellness engagement platform.

"Genomind is committed to bringing patients' information, access to care, and innovative tools that support the tremendous unmet needs in mental health. We have some of the best minds in the world on the frontlines of this problem and are thrilled to bring our subject matter expertise to Sharecare – a company whose dedication to improving health and well-being has already changed millions of lives," said Shawn Patrick O'Brien, CEO at Genomind.

## BAYER ENTERS INTO A \$5M AGREEMENT WITH CHOP FOR HEMOPHILIA RESEARCH PROGRAM



Bayer and the Children's Hospital of Philadelphia have joined forces for a three-year agreement of \$5 million, which will serve as an investment in a joint hemophilia research program. The terms of the deal grant Bayer an option to exclusively license the collaboration results and continuing the collaboration with the agreement of both parties.

The partnership between these two member companies will look at the discovery and development of small molecule drugs for hemophilia A and B that can be taken orally. "Small molecule therapies could help thousands of people with hemophilia A and B and we are looking forward to combining our strengths in hemophilia research," said Dr. Joerg Moeller, head of research and development at Bayer.

This agreement is an example of how the PA life sciences community readily collaborates and combines its expertise across multiple areas to conduct groundbreaking research and discover new treatment options.

## THE WISTAR INSTITUTE AND LEIDEN UNIVERSITY OFFER NEW POSTDOCTORAL INTERNATIONAL EXCHANGE PROGRAM



The Philadelphia-based Wistar Institute has entered into a formal agreement with Leiden University Medical Center (LUMC) in the Netherlands that will create an exciting new opportunity for recent PhD graduates to participate in an exchange program in immunology, cancer research and vaccine biology. Officially known as The Wistar-Schoemaker International Postdoctoral Fellowship, the program will bring postdoctoral graduates from LUMC and Wistar to further their research with mentorship from biomedical research leaders.

"We are very excited by the prospect of an international exchange between Wistar and LUMC, expanding the Institute's reach and opening new opportunities for global collaboration," said Dario C. Altieri, MD, Wistar president and CEO and director of the Institute's Cancer Center.

The postdoctoral fellowship program expands Wistar's international partnerships in research and education and promoting scientific exchange between investigators in the Netherlands and the United States. Bringing new perspectives to the PA life sciences community and sending American researchers abroad to gain new insights and experiences will surely foster diversity in our community.

## FIBROCELL SUCCESSFULLY ACQUIRED BY CASTLE CREEK PHARMACEUTICAL HOLDINGS



Cell and gene therapy company Fibrocell Science was acquired by the privately held Castle Creek Pharmaceutical Holdings, Inc in December. CCP Holdings places emphasis on the development of medicines for important rare genetic disorders. While Fibrocell has a strong suit in transformational autologous cell-based therapies for skin and connective tissue diseases.

The combined organization is on track to continue late-stage development programs for rare genetic diseases like epidermolysis bullosa (EB). "Our organizations have a shared commitment to delivering new treatment options to underserved communities such as EB patients and their families who have no approved therapies. The addition of this late stage gene therapy will allow us to be the leader in rare genetic dermatology and additional genetic diseases as well," said Greg Wujek, chief executive officer of Castle Creek.

### SHARE YOUR UPDATES

Want to be featured here? Forward news articles, event recaps, or announcements to **McKenzie Belton** at [mbelton@lifesciencespa.org](mailto:mbelton@lifesciencespa.org) to be considered for our next issue.



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# LSPA IS PROUD TO SUPPORT LIFE SCIENCE CARES



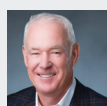
Life Science Cares (LSC), a collective effort of the life science industry to reduce the impact of poverty in select metropolitan regions, launched in Philadelphia, PA on December 16. During an event at Broad Street Ministry, local leaders representing a wide range of life science companies and non-profit organizations, came together in support of LSC's mission: to make a difference in the lives of Philadelphians by bringing the life science community together to offer both financial grants and hands-on support and volunteerism. LSC also announced its local board of managers, inaugural non-profit partners and founding advisory board members.

Despite its growth in population and household income, Philadelphia is the poorest large city out of the 10 most populous in America – with more than a quarter of the city's residents living below the poverty line. It is time to use our success, our leadership and our resources to tackle our region's great social issues. Life Science Cares is a platform for all companies and employees in our industry to expand the ways that we give back time, talent or monetary contributions, all while building relationships with fellow life sciences colleagues and talented non-profit partners.

## NON-PROFIT PARTNERS

**Broad Street Ministry | Cradles to Crayons | FirstHand at University Science Center | Philabundance | Year Up**

## LIFE SCIENCE CARES PHILADELPHIA BOARD OF MANAGERS



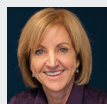
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*Spark Therapeutics*



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## LIFE SCIENCE CARES ADVISORY BOARD

Members of the LSC Advisory Board – many of whom also currently serve on our LSPA Board of Directors – support LSC by promoting the organization within their company, providing recommendations for non-profit partners and personally supporting administrative costs so that all money raised from companies and individuals can go directly to community grants.

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For more information on LSC Philadelphia, its leadership and opportunities to get involved, visit [lscphilly.org](http://lscphilly.org) or follow LSC Philadelphia on Twitter (@LS\_CaresPhilly) and LinkedIn.

\*Indicates members of LSPA's Board of Directors



# FINANCIAL BULLETIN Q4

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ANOTHER PROFITABLE QUARTER IS IN THE BOOKS FOR PENNSYLVANIA. THE LOCAL LIFE SCIENCES INDUSTRY CONTINUES TO BRING IN CAPITAL TO THE ALREADY THRIVING "CELLICON VALLEY" AND CONTRIBUTE TO THE STATE'S ECONOMY. BELOW IS A LIST OF FINANCIAL ACHIEVEMENTS FROM THE 4TH QUARTER OF 2019.

- **Oncora Medical** received a \$3 million investment from Silicon Valley medtech company Varian to work with California-based Varian on software tools that enable precision medicine in radiation oncology – October 2, 2019
- **Cabaletta Bio Inc.** raised \$74.8 million in an initial public offering, which made it the region's first life sciences IPO of 2019 – October 25, 2019
- **Allevi** raised \$1 million in a private stock sale and will use the funds to grow inventory and sales to promote development of its three-dimensional bioprinting applications – November 1, 2019
- **Galera Therapeutics** went public and raised \$60 million by selling 5 million shares of common stock at \$12 per share – November 7, 2019
- **Tela Bio** joins other Philadelphia life sciences companies in going public, raising a total of \$52 million after selling four million shares of common stock at \$13 each – November 8, 2019
- **Mobilion Systems Inc.** raised \$15.4 million in series A financing and will use the funds to advance the development of its ion mobility separations technology – November 12, 2019
- **Integral Molecular** was awarded a \$5.5 million contract over the next five years from the National Institute of Allergy and Infectious Diseases (NIAID) to define antibody responses to pathogenic viruses – November 14, 2019
- **Onconova Therapeutics** announced the successful closing of its public offering, in which it sold 55,000,000 shares of common stock and received \$11 million to fund its clinical and pre-clinical research efforts – November 25, 2019
- **Venatorx Pharmaceuticals, Inc.** received a \$4.1 million grant from **CARB-X** to support the development of a new class of oral antibiotics to treat multi-drug-resistant gonorrhea. The company may receive nearly \$9 million in additional funding for meeting certain milestones – November 25, 2019
- **Velicept**, a privately held Wayne, PA-based biopharma company, has raised \$6 million to advance its drug candidate – November 27, 2019
- **Coriell Institute for Medical Research** scientist Shoghag Panjarian, PhD, has received a grant from the W. W. Smith Charitable Trust to support her work in identifying potential epigenetic drivers of breast cancer – December 5, 2019
- **Jefferson Health** received a \$70 million gift from the Kimmel family, the namesake of Jefferson's Kimmel Cancer Center, to fund a new research tower in Philadelphia. It is the second largest gift in the health system's history – December 5, 2019
- Biotech and device maker **Windtree Therapeutics** completed a private placement of 8.75 million shares of stock, for which it raised \$26.4 million – December 9, 2019



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Greater Philadelphia's biomedical cluster is the natural out-growth of the region's proximity to the large pharmaceutical companies in southern New Jersey, the array of high-profile academic research and medical centers in Philadelphia, and the region's legacy chemical companies.

The region is home to over 800 life sciences companies, 15 major health systems, four NCI-designated cancer treatment centers, and seven medical schools. It is also one of the second-largest medical device clusters according to the industry association AdvaMed – ranking second behind California.

BigBio Communications is proud to showcase the news and developments from the Greater Philadelphia area with the launch of Big4Bio: Philadelphia!

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## SUPPORTED PATIENT ADVOCACY EVENTS

### 20<sup>th</sup> Annual For Pete's Sake Cancer Respite Foundation Beach Ball Gala

February 1, 2020  
Philadelphia, PA

### Community Liver Alliance's You Make a Difference Awards Luncheon

February 7, 2020  
Pittsburgh, PA

### Simon's Soiree

February 8, 2020  
Philadelphia, PA

### Bowling for Bleeding Disorders

February 9, 2020  
Pittsburgh, PA

### Leukemia & Lymphoma Society's Red & White Ball

February 29, 2020  
Philadelphia, PA

### AACR Runners for Research - 2020 Blue Cross Broad Street Run

May 3, 2020  
Philadelphia, PA

### Walk to End Lupus Now

April 19, 2020, May 17, 2020  
and June 14, 2020  
Harrisburg, PA, Ocean City, NJ  
and Wilmington, DE

### 2020 Susan G. Komen Philadelphia More Than Pink Walk

May 10, 2020  
Philadelphia, PA

### Interdisciplinary Symposium on Osteoporosis (ISO2020)

May 27-30, 2020  
Washington, DC

### The Great Chefs Event Philadelphia

June 6, 2020  
Philadelphia, PA

### rideATAXIA Philadelphia

October 11, 2020  
Blue Bell, PA

For more information about  
these and other upcoming  
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TURNER HEALTHCARE QUALITY  
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## 2020 DIAMOND MEMBERS





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