During the last few years, Life Sciences Pennsylvania has grown to include a membership of more than 730 organizations – pharma, biotech, medical devices, diagnostics, CROs, and healthcare IT companies, plus academic research organizations, investors, patient advocacy groups, and the myriad service organizations who support our life sciences community. Our advocacy activity is stronger than ever, and our programs are recognized as world-class events.

This growth, diversity of membership, and broad appeal led us to change our name last October to Life Sciences Pennsylvania, a name that reflects the geographic reach we have achieved across the Commonwealth and the unique mix of life sciences stakeholders we enjoy among our membership community.

What has not changed, however, is our unwavering commitment to membership services and member success.

To further our mission of providing membership services and facilitating strategic connections, we have expanded our offices in Wayne, PA to create @LifeSciencesPA – The Life Sciences Center for Pennsylvania. Our new, 8,600 square foot center will offer our members networking and educational events; happy hours; meeting space; Center-wide internet service; quiet, secluded rooms for phone calls, videoconferences or teleconferences; and conference and training rooms – all complimentary to members of Life Sciences Pennsylvania.

If you are interested in seeing the new space, Life Sciences PA will be hosting two Breakfast Open Houses - Tuesday, July 18 from 7:30 a.m.-9:30 a.m. and Wednesday, July 26 from 7:30 a.m.-9:30 a.m. Registration is available on the Life Sciences PA website - www.lifesciencespa.org.

The square footage of @LifeSciencesPA is considerable, but so is our membership, so we will cap registration at 75 for each of the two Open House periods. Be sure to register as soon as possible.

If you are not available July 18 or 26 – and since the Center and its amenities are benefits to all members of Life Sciences PA – you should feel free to come by and visit at any time. We are proud to open new doors for our members - furthering our mission - and providing more space for members to collide, create and form new strategic connections to help further the success of Pennsylvania’s already successful life sciences community.
On June 27, Life Sciences PA officially opened the doors to @LifeSciencesPA - The Life Sciences Center for Pennsylvania - to former and current members of its Board of Directors, VIPs, elected officials, and members of the media, for what became a night of socializing, reflecting on the association’s growth and evolution, and celebrating its achievements in membership and advocacy. Attended by more than 30, guests received tours of the newly created and meticulously appointed meeting space; enjoyed live music, food and drink; and caught up with friends and friendly faces from the past.

“We are very delighted to showcase this new space to a group of individuals who have all contributed to its success in one way or another,” said Christopher Molineaux, president and CEO of Life Sciences PA. “Now that @LifeSciencesPA is open, we look forward to our membership putting it to good use - hosting meetings, programs, happy hours, trainings, and whatever other events the space can accommodate.”

@LifeSciencesPA is located at the Life Sciences Pennsylvania offices at 650 East Swedesford Road in Wayne, PA. For more information, visit www.lifesciencespa.org, contact Anne Hart at ahart@lifesciencespa.org, or call 610.947.6800.
A Plymouth Meeting, PA biotech firm is breaking bold new ground in the development of a first-ever vaccine for the HIV virus. Life Sciences PA member Inovio Pharmaceuticals recently completed a clinical study that showed the highest overall levels of immune response rates ever demonstrated in a human study by an HIV vaccine.

In early patient tests, the company’s innovative DNA-based vaccine candidate, PENNVAX-GP, produced a nearly 100 percent immune response in study volunteers. The study was supported by the HIV Vaccine Trials Network (HVTN) and the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health (NIH). The results mirror the near 100 percent response rates Inovio saw for its DNA-based immunotherapies for Ebola, Zika and MERS.

“These results are among the highest ever responses we’ve seen with an HIV vaccine,” said Dr. J. Joseph Kim, Inovio President and Chief Executive Officer. “Furthermore, our newer and more tolerable intradermal vaccine delivery device showed that we can elicit very high immune responses at a much lower dose.”

Inovio is developing vaccines in collaboration with, and based on, DNA vaccine technology developed by David Weiner, director of the vaccine center at Philadelphia-based The Wistar Institute, the nation’s first independent medical research institute. Development of PENNVAX-GP has been funded through a $25 million NIAID contract previously awarded in 2009 to Inovio and its collaborators.

The HIV study results hold promise for the long-term development of a functioning vaccine that will ultimately stop the global pandemic. Nearly 35 million people have died from HIV-related causes and over 36 million are living with HIV, a retrovirus that causes acquired immunodeficiency syndrome (AIDS). Currently, there is no HIV vaccine on the market.

Next steps for Inovio in the development of PENNVAX-GP include studying the long-term immune responses in its early patient volunteers one year after vaccination. The company then plans to advance the vaccine into later-stage clinical development to determine its efficacy to reduce or prevent HIV in patients.

MEMBERSHIP NEWS

INOVIO PHARMACEUTICALS MAKES STRIDES TOWARD BREAKTHROUGH HIV VACCINE

FIBROCELL DEVELOPING GENE THERAPY WITH TRANSFORMATIVE POTENTIAL FOR PATIENTS WITH RECESSIVE DYSTROPHIC EPIDERMOLYSIS BULLOSA

Fibrocell Science, Inc., a gene therapy company, has achieved several key milestones this year in the development of FCX-007, a potentially transformative new treatment for recessive dystrophic epidermolysis bullosa (RDEB)—a devastatingly painful, genetic skin disease with a high mortality rate. The Exton, PA-based company is leveraging its distinctive autologous fibroblast technology in collaboration with Intrexon Corporation’s synthetic biology to develop FCX-007—its gene therapy candidate for the treatment of RDEB—to target the underlying cause of the disease.

A congenital, progressive disease with no cure, RDEB is caused by a mutation or a lack of the protein, type VII collagen (COL7). Without this protein, skin layers do not connect which causes severe blisters and wounds to form at the slightest friction or bump. FCX-007 is an autologous dermal fibroblast genetically-modified to produce functional COL7—that is missing or deficient in RDEB patients—and enable formation of “connectors” to hold the layers of skin together.

RDEB affects approximately 1,100 to 2,500 patients throughout the United States. Children who inherit the disease are often called “butterfly children” because their skin is as fragile as a butterfly’s wings.

Current treatments, including daily wound care and surgery, only address symptoms. With no therapy approved by the U.S. Food and Drug Administration (FDA) for the treatment of RDEB, FCX-007 offers potential to be a significant advance in RDEB patient care. During the first half of 2017, Fibrocell attained four major milestones in the development of FCX-007:

In January, the FDA granted “Fast Track” Designation to FCX-007, which could facilitate more frequent interaction
with the FDA and help accelerate the regulatory approval process, if relevant criteria are met. In addition to the Fast Track Designation, FCX-007 was previously granted Orphan Drug and Rare Pediatric Disease Designations by the FDA.

In February, Fibrocell announced that the first adult patient was dosed in the Phase 1 portion of the Phase 1/2 clinical trial of FCX-007.

In May, the Data Safety Monitoring Board (DSMB)—an independent group of experts monitoring patient safety and treatment efficacy data as part of the clinical trial—recommended continuation of the Phase 1/2 clinical trial of FCX-007 following a planned review of safety data from the first patient treated. No product-related adverse events were reported.

In June, the company announced that the remaining two adult patients in the NC1+ cohort were dosed in the Phase 1 portion of the Phase 1/2 clinical trial of FCX-007. Dosing additional patients followed the DSMB’s recommendation that was announced in May.

“We are pleased with the progress of FCX-007,” said John Maslowski, Chief Executive Officer of Fibrocell. “By addressing the underlying cause of RDEB, we believe FCX-007 has the potential to be transformative for patients and their families.”

CHURCHILL COMPLETES MILESTONE NDA SUBMISSION, EXPANDS COMMERCIALIZATION TEAM

King of Prussia, PA-based Churchill Pharmaceuticals, a clinical-stage biopharmaceutical company focused on the development of orally delivered oncology products, has recently completed its first New Drug Application (NDA) to the U.S. Food and Drug Administration. The NDA submission is for the company’s lead portfolio product YONSA for the treatment of metastatic castration resistant prostate cancer (mCRPC).

YONSA offers hope for improving the lives of men living with mCRPC. An advanced form of advanced prostate cancer that is resistant to medical or surgical treatments and has spread to other parts of the body, mCRPC afflicts approximately 10 to 20 percent of men diagnosed with prostate cancer. YONSA is an investigational oral agent that can serve as a first-line treatment for mCRPC. The new drug attacks prostate cancer cells by blocking an enzyme that the cells use to make androgen in small amounts.

The company is also aiming to solve the solubility challenge that has impeded the effectiveness of oral cancer treatments. Solubility is a drug’s ability to dissolve in the gastrointestinal fluid and is one element essential to consistently achieving optimal efficacy and safety. 90 percent of oral anticancer medications in development have low solubility and 50 percent of available oral anticancer drugs may be compromised by poor solubility.

Churchill addressed this challenge by formulating YONSA through the SoluMatrix Fine Particle Technology. A proprietary manufacturing process, SoluMatrix enhances the potential of certain oral drugs by changing how well they dissolve and how efficiently they are absorbed.

SoluMatrix uses a patented dry-milling process that grinds organic/ pharmaceutical active compounds into a fine powder, reducing the particle size of poorly soluble drugs to the submicron level—10 to 200 times smaller than conventional drug particles. At the same time, a custom blend of excipients both aids in the grinding process and protects the active particles from subsequent agglomeration.

“Our collective experience working in large pharma and small biotechs, and built largely from our Pennsylvania roots, gives us an expansive look at what great looks like, which we mobilize in our strategic decisions and day-to-day work practices at Churchill,” said Scott Megaffin, President, Churchill.

Following the NDA submission, Churchill expanded its commercial leadership team in anticipation of YONSA’s marketplace launch. New team members include Sharlene Cirillo, Vice President, Marketing; Jim Datz, Vice President, Market Access; Matt Battin, Vice President, Sales; Frank Barrett, Head of Patient Support Services and Nate Ide, Head of Trade and Distribution. This highly experienced team joins Ben Steinmetz, Senior Vice President, Commercial who has been with the organization since 2015.

More information on Churchill Pharmaceuticals, and this NDA submission, can be found on the company’s website at www.churchillpharma.com.
Neuronetics, Inc., a privately-held medical device company widely recognized as a leader in transcranial magnetic stimulation (TMS) therapy, has closed a $15 million funding round in early June, coming at a time when NeuroStar(R) Advanced Therapy is making major strides in improving access to its non-drug technology for adult patients with Major Depressive Disorder (MDD). With widespread insurance coverage and enhanced scheduling convenience as the only FDA-cleared TMS treatment that can be delivered in under 19 minutes*, NeuroStar Advanced Therapy has delivered 1.4 million treatments to more than 50,000 patients, providing greater accessibility to a growing patient population.

“Investors continue to recognize the transformative work we are doing with NeuroStar Advanced Therapy in helping patients with Major Depressive Disorder, and see the broad potential in expanding access to this important therapy to the more than four million treatment-resistant adults living with depression who aren’t currently benefiting from their current antidepressant treatment plans. With the recent launch of our next generation NeuroStar system and the broad-based support we are receiving from doctors, patients and insurance companies, the future is bright at Neuronetics,” said Chris Thatcher, president and CEO of Neuronetics.

PITTSBURGH LIFE SCIENCES GREENHOUSE AND HELOMICS TO LAUNCH DIAGNOSTICS AND PRECISION MEDICINE INCUBATOR

In a joint news release available on both organizations’ websites, Helomics Corp. and the Pittsburgh Life Sciences Greenhouse (PLSG) announced a joint agreement to establish an incubator focused on diagnostics and precision medicine located at Helomics’ offices in Pittsburgh’s Lawrenceville neighborhood.

“A startup process begins with validating that the technology works,” said PLSG President and CEO James Jordan. “The process is aided by the ecosystem, which frequently is composed of a progression through incubator funding and angel capital funding, followed by venture capital.”

“We are very excited about this strategic collaboration with the PLSG because Helomics is in a position to help at both the very beginning of the process, by validating the technology, and the end of the process, with commercialization,” said Gerald J. Vardzel Jr., President and CEO of Helomics. He added, “Our missing piece was a business partner who could both fund and assist companies in developing the infrastructure towards commercialization. The PLSG provides that missing component.”

Helomics is an integrated clinical contract research organization that aims to improve patient care by partnering with pharmaceutical, diagnostic and academic entities to bring clinical products and technologies to the marketplace.

Life Sciences Pennsylvania commends both organizations on this tremendous advancement for the life sciences community in Pennsylvania, and looks forward to continued innovation, growth, partnerships, and technology in the Pittsburgh region.
According to a news release published on its website, OraSure Technologies, Inc. entered into an agreement with the Bill & Melinda Gates Foundation that will enable OraSure to offer its OraQuick® HIV Self-Test at an affordable price in 50 developing countries with funding from the Gates Foundation.

Under a four-year Charitable Support Agreement, OraSure will make its OraQuick® HIV Self-Test available for purchase in 50 developing countries located in Africa and Asia. These countries include Malawi, Zambia and Zimbabwe, where the Company has been providing tests for the “Self-Testing in Africa” or “STAR” Project implemented by Population Services International (“PSI”).

Through this agreement, funding from the Gates Foundation will enable non-governmental organizations in eligible countries that receive funding from government or public sector agencies and donors such as the Children’s Investment Fund Foundation (UK), the Global Fund, PEPFAR and UNITAID to access HIV self-testing at reduced pricing. The funding from the Gates Foundation will be in an aggregate amount not to exceed $20 million over the four-year term or $6 million each year of the agreement.

“We are pleased to work with the Bill & Melinda Gates Foundation on this important initiative designed to support the rapid scale-up and adoption of the OraQuick® HIV Self-Test in the fight against HIV/AIDS,” said Douglas A. Michels, President and CEO of OraSure Technologies. “We are witnessing the benefits of HIV self testing through the use of our product by PSI under the STAR Project. The support payments provided by the Gates Foundation will help us expand our relationship with PSI and substantially accelerate the adoption of the OraQuick® HIV Self-Test in many more developing countries.”

“Diagnosing HIV quickly and accurately is critical to both improving treatment access and preventing new infections,” said Emilio Emini, Director of the HIV program at the Bill & Melinda Gates Foundation. “This initiative will allow for increased access to self-conducted HIV screening, resulting in a substantially improved ability to recognize and treat individuals living with HIV.”

The OraQuick® HIV Self-Test is a rapid, point-of-care test that allows an individual to detect antibodies to both HIV-1 and HIV-2 with an oral swab, with results in as little as 20 minutes. It is the same test used in the Company’s OraQuick® In-Home HIV Test, the first and only FDA-approved in-home HIV test for consumers, except that the packaging and product instructions have been tailored for use by individuals in developing countries.

More information on this agreement can be found on OraSure’s website, www.orasure.com.

Particle Sciences, located in Bethlehem, PA, will receive an investment from their parent company - The Lubrizol Corporation - to strengthen its contract services capabilities according to a company news release issued in early June. Leveraging the company’s knowledge in complex formulations and production, the new commercial drug product manufacturing facility will be adjacent to the existing development and clinical trial manufacturing site, offering its customers a seamless flow from development through manufacturing. This new space, which is expected to be operational in the fourth quarter of 2017, will accommodate both sterile and non-sterile products, highly-potent compounds, and organic solvent processing.

This advancement and growth is important for not only the life sciences community in Pennsylvania, but the life sciences industry in the U.S., as it offers an added resource to help streamline and optimize the drug development process - right here in Pennsylvania. Additionally, this growth and investment signifies the continued importance and evolution of the contract services industry in the commonwealth.

“We have significantly enhanced our capabilities through the combination of strong polymer technology, application know-how and world-class manufacturing,” said Deb Langer, vice president, Lubrizol Personal Home and Health Care. “As healthcare companies look for total solution providers, we continue to invest in the right areas to provide valuable offerings where our customers are experiencing the most growth.”

More information about this expansion can be found on the company’s website, www.particlesciences.com.
Repeal? Replace? REALLY?

As everyone takes time off and winds down during the summer months, the debate in Washington, DC continues to heat up. Much of the current “circular firing squad” in our nation’s capital revolves around proposed plans to repeal and replace the Patient Protection and Affordable Care Act (ACA), colloquially known as “Obamacare.”

Before I discuss the interest of our member companies in the health insurance reform debate, it’s important to highlight the original goals of the ACA, signed into law in March of 2010. The ACA was created ostensibly to have more Americans covered under health insurance by providing tax credits for private insurance, by expanding Medicaid, and by supporting ways to make health care less costly and more effective.

Those are admirable goals, and in fact, more than 20 million additional individuals gained health insurance as the law was implemented. Ensuring individuals have coverage is the first step in helping to be sure our therapeutics and devices are accessible to the patients who need them most. By expanding access to coverage, and making changes to fill the Medicare Part D “donut hole” by 2020, the Affordable Care Act made important strides to help patients obtain treatments that may have previously been out of reach.

Aside from generally increasing access for patients, another positive outcome of the ACA is its inclusion of the Biologics Price Competition and Innovation Act. This legislation provided branded manufacturers and innovators of biologics 12 years of data exclusivity, which is critical to protecting the incentives necessary to drive future research and development in these complex drugs.

Unfortunately, as with many ideas generated in government by well-meaning individuals with limited or no business experience, the ACA is a large and complex piece of legislation that includes many ill-conceived provisions which jeopardized jobs, innovation, and American leadership in medical innovation. Just plain bad ideas that would have the unintended bad consequences of hurting the very patients we hope to help. To expand – essentially require – insurance coverage for more individuals, the bill included several “pay-fors” with which we have, and continue to have, concerns. Two taxes or fees are of particular concern: the Medical Device Tax, and the Branded Prescription Drug Fee.

The Medical Device Tax imposed a 2.3% excise tax on non-retail medical devices, which we have strongly opposed since its inception. Thanks to the efforts of our member companies, our national affiliates, and nearly our entire Pennsylvania congressional delegation, the tax is currently under a two-year suspension, which ends December 31, 2017. The tax was in effect for two years, from 2013 through 2015, during which time it cost the industry roughly 29,000 jobs nationally – jobs that were either eliminated or went unfilled. We have continued to proactively oppose this legislation and, thankfully, the House version of the repeal and replace bill includes a full and permanent repeal of the Medical Device Tax.

The Branded Prescription Drug Fee is an excise tax included in the ACA to help fund the federal government’s expanded role in health care, specifically its expansion of the Medicare Part D program and the Medicaid program. Branded prescription drug companies pay a “tax” on their proportionate share of the branded prescription drug market. Thankfully, again, the House-passed version of the repeal and replace bill also includes a full repeal of the Branded Prescription Drug Fee.

In addition to these two fees/taxes, the ACA also imposed two cost-saving measures about which we have expressed concerns since the provisions of the ACA first came to light. The first is the Independent Payment Advisory Board (IPAB), which is a fifteen-member agency with the exclusive task of achieving savings in Medicare spending. It is triggered when Medicare spending reaches a certain threshold. Because this threshold could be reached in 2017, our industry and many of our partners have continued to oppose IPAB due to the cuts it could make to reimbursements and the effect those cuts could have on patient access to care. Interestingly, no members have ever been appointed to the 15-person board, so if IPAB is triggered, the responsibility will fall to the Secretary of Health and Human Services.

The other cost-saving measure the ACA implemented was the Center for Medicare and Medicaid Innovation, or CMMI. CMMI is an office within the Centers for Medicare and Medicaid Services created to test innovative ways to maintain or improve the quality of care while decreasing program costs. For example, CMMI proposed a demonstration project designed to test payment changes to drugs in Medicare Part B. Part B covers outpatient drugs, those that are administered in a doctor’s office or hospital, like chemotherapy drugs, and this proposal would have lowered the payment rate from average sales price (ASP) plus 6 percent to ASP plus 2.5 percent. It was met with opposition from our organization, other state associations, national life science associations, our member companies, specialty doctor groups and patient groups, among others. The CMMI was never implemented.

In response to these issues, and significant concerns about the sustainability of the Affordable Care Act, Republicans in Congress have been adamant about its repeal. Now with control in the House of Representatives, the Senate, and the White House, they have made a strong push to try and repeal the ACA. While this situation is very fluid, the House passed its repeal of ACA, the American Health Care Act, on May 4, by a margin of only four votes: 217 – 213.

The Senate Republicans released a draft/discussion text of their healthcare bill, the Better Care Reconciliation Act, on June 22. It contains some differences from the House version, but has been met with increased trepidation from some Senate Republicans. In fact, a vote was expected to take place prior to the July 4 holiday recess, but the vote was pulled as Republicans in the Senate try to iron out their differences.

Thanks to the continued advocacy of our members and our national partners at PhRMA, MDMA, AdvaMed, BIO and ACRO, there are some positive provisions in both the House and Senate “repeal and replace” bills. They both repeal the Medical Device Tax and the Branded Prescription Drug Fee; they maintain the 12 years of data exclusivity for biologics; and they ensure a continued reduction in the Medicare Part D coverage gap. Unfortunately, neither the House nor the Senate bill repeals IPAB, or CMMI. Nevertheless, we will continue to actively oppose any attempts of those offices to adjust reimbursements in a way that could limit patient access to the lifesaving therapeutics and devices developed by our member companies.

Though it remains unclear exactly how this debate will proceed, we monitor the proceedings closely, weighing-in whenever necessary and appropriate. We will continue to work feverishly with our member companies, national affiliates, and various partners to ensure all patients have access to the fruits of innovation of our life sciences community.
Paul Perreault is CEO and Managing Director of CSL Behring - a global biopharmaceuticals leader headquartered in King of Prussia, PA, focused on innovating and delivering therapies used to treat rare and serious conditions. CSL Behring employs more than 14,000 employees in 30 countries. The Newsletter had the opportunity to chat with Paul about CSLs vision for growth; its commitment to innovation and patients; and its steadfast dedication to the global orphan and rare disease community.

You have been part of CSL Behring since 2004, and have held multiple positions including Executive Vice President, Worldwide Commercial Operations, and your current role as Chief Executive Officer and Manager Director. What drew you to CSL Behring originally, and what keeps you there as its chief executive?

I joined CSL as part of its acquisition of Aventis Behring in 2004. What compelled me to stay on with the company, and what keeps me engaged and committed to the business every day are the patients who benefit from our medicines. Given the complexity of the healthcare system, sometimes it is easy to lose sight of the people whose lives are literally changed by the work that we do. That's why it's so important to ensure that having a “patient focus” is woven in to the fabric of your business, from your culture to your growth objectives. Since the company's creation a century ago, our ultimate customer has been the patient and I value the personal relationships I have with patients I've met along the way.

CSL Behring is driven by its promise to discover and introduce innovations addressing unmet medical needs, and enhance existing treatments. As managing director and CEO, how do you encourage your global network of employees to fulfill this promise to your ultimate stakeholder – the patient?

More than a century ago, we made a promise to save lives and protect the health of people. Because we were created to serve the needs of people and because of the critical nature of our products, right from the start CSL innately understood the importance of serving a greater good and for us that meant being patient-focused.

Although the company has grown to approaching 20,000 employees, delivering medicines in more than 60 countries, I never want to lose connection with the people who rely on CSLs therapies.

So I make sure that I personally engage with patients and that our employees personally engage with patients. For example, every year CSL runs a sports clinic in Phoenix for more than 100 children with bleeding disorders. CSL employees organize and run these programs and I've personally caddied for the golf tournaments and shagged baseballs. But aside from fun and sport, these are important learning events and I attend them as often as I can because it's imperative that we hear from patients and their families firsthand. And this is just one program, we engage with patients in many ways.

We routinely bring patients to our global meetings, and to our sites around the world to meet with employees and talk about their needs and experiences. We also maximize the number of employees we reach by sharing their stories online through compelling videos.

I want everyone in the company to understand the importance of what we do for patients, and ultimately to work every day as if patients' lives depended on it, because usually they do.

In interviews, you reference the importance of the “voice of the patient” in drug development. Why is that such an important contributing factor to CSLs market success and impact?

Our 100-year track record would not have been possible without patients at the center of everything we do.

To truly serve patient needs, you need to hear from them directly. You need to understand the burden of their condition, and the challenges that they and their families face. So, at CSL the voice of the patient drives our innovation, and provides the direction for what we set out to develop.

Patient input led to the development of HAEGARDA, the first and only subcutaneous preventive treatment for Hereditary Angioedema (HAE). HAE is a rare genetic disorder that causes potentially catastrophic swelling, which can affect the face, mouth, lips, throat and other areas of the body. It is very unpredictable, exceedingly painful and if the swelling occurs in the throat or tongue it can cause suffocation and even death if not treated immediately. Patients are never sure when they're going to get an attack, or how severe that attack's going to be. HAEGARDA was developed to prevent those attacks with a simple injection under the skin rather than an intravenous infusion. When patients tell us that this is the closest thing they've seen to a cure, you know you've developed a remarkable product that will have a tremendous impact on patient's lives.

Serving the worldwide orphan and rare disease community is a primary R&D focus and business imperative for CSL Behring. How does CSL partner with other external organizations to effectively deliver breakthrough medicines to those who need them most?

As a company dedicated to saving lives and improving the quality of life for people with rare and serious conditions, we believe that helping our patient communities and advancing knowledge in our therapy areas is part of our corporate responsibility.

We are constantly on the lookout for how we can help those on the front lines of patient care. Earlier this year we announced a commitment to sponsor the Jeffrey Modell North African Network, which will establish diagnostic and research centers in Northern Africa, making it the first enterprise dedicated to helping primary immunodeficiency patients in Africa.

We also want to help promote great science, regardless of where it is taking place. To that end, we fund an annual, global program called the Interlaken Leadership Awards, which provides monetary grants and/or product supply for investigational use to support research focusing on the potential role of immunoglobulin therapy in the treatment of neurological/neuromuscular disorders. This program has awarded more than $5 million in grants and study drug over the past six years.

Community stewardship seems to be an important focus area for CSL Behring. What are some key areas of importance for the organization when it comes to serving the rare disease community? Nationally? Globally?
We have a role to play in contributing to humanitarian programs and relief efforts around the world.

We continue to support efforts to enhance care in developing countries and in April we donated 4 million international units (IUs) of medicines for hemophilia and/or von Willebrand disease to the World Federation of Hemophilia (WFH). This contribution is part of our three-year promise to provide the WFH with more than 10 million IUs of specialty biotherapeutics to treat patients in the developing world.

All around the globe our sites engage employees in local giving activities, with employees matching programs and community development support. Over the past year we've partnered with local, national and international patient organizations to raise awareness of serious diseases and improve access.

We support organizations like the Immunodeficiency Foundation (IDF) by contributing to their annual fundraising efforts. For a two-month period, CSL employees at our sites across the country organize a variety of programs to raise money for the IDF. This year, our employees raised $130K, which will be used for patient and family support services, educational initiatives, and research and development activities. Other programs we support include Alpha-1’s Walk for Breath, and Voice2Voice, a unique patient-support program that connects patients and caregivers with people who know what it’s like to live with Primary Immunodeficiency.

Our longstanding commitment to the rare disease community has not gone unnoticed and I think that’s because the rare disease community knows that we’re in it for the long haul. We are really pleased that just last month CSL Behring received the National Organization for Rare Disorders’ (NORD) 2017 Industry Innovation Award for pioneering new medicines, in particular our long-acting fusion protein for patients with hemophilia B.

The Pennsylvania Legislature recently established a joint Rare Disease Caucus, being only 1 of 4 states to do so. How important is it to the continued support of the rare disease communities across the United States for lawmakers to get involved and educated on the issues facing the rare disease community, and industry broadly?

I think the Pennsylvania’s State Legislature took a tremendous step in establishing a Rare Disease Caucus. Rare diseases are estimated to affect over 30 million Americans, with large impacts on the patients, families and society. Rare conditions while quite diverse, do share several commonalities – they are hard to diagnose, they are chronic and serious, and they can take a huge emotional toll on patients and their families. Actions that increase awareness of rare diseases and assure focus of public policies to advance diagnosis, treatment and access to care are especially needed given the challenges and impacts of these conditions. A society is often judged by how it treats its most vulnerable, and we are proud to see Pennsylvania taking a leadership role and establishing its rare disease caucus.

During the clinical progression of a compound, how important is it for innovator companies to start pricing conversations with insurance companies – to ensure patients have necessary access to the therapeutics they need most?

I can’t emphasize enough how important it is to understand what value means to the payer. You have to talk to payers before your product reaches the market so that you can demonstrate how the product will bring value in the real world.

It is also critically important that you build value measures into your clinical trial program because in today’s market to successfully launch new medicines you need a product label that demonstrates innovation and differentiation. Payers aren’t likely to cover products that don’t show significant value and benefit to patients and the healthcare system. 

But our voice is not the only one that matters.

Patients are often the most compelling advocates for access to care, which is why CSL awards Local Empowerment for Advocacy Development (LEAD) grants to support advocacy work of rare disease patient groups throughout the US. These groups tackle complex legislative and public policy issues and ensure patients’ voices are heard on Capitol Hill and in statehouses across the US.

Through our LEAD Grant program, we have awarded grants to a number of patient advocacy organizations totaling almost $1 million. These grants help support patient empowerment and grassroots initiatives to inform and impact public policy decisions to ensure all patients have access to the therapies and services they need to remain healthy.

What’s next for CSL Behring? Is it growth by way of acquisitions in targeted therapeutic areas? Continued exploration into the hemophilia market? Or is it something else?

Our focused R&D strategy, combined with a relentless commitment to expanding our commercial reach, position us to deliver sustainable growth.

We continue to see attractive projects and value in both plasma-derived and recombinant technologies. Last year we launched AFSTYLA and IDELVION to treat hemophilia A and B respectively, which were produced using recombinant processes rather than human plasma. These products have been designed with significant features over and above the natural proteins deficient in hemophilia patients.

We also have several potentially transformational product candidates in our pipeline. In November we reported the positive results of our phase 2B study for CSL112, a therapy that holds the potential to prevent recurring heart attacks and other coronary events. If successful CSL112 could represent an exciting new approach to treating high-risk patients following an acute heart attack and could improve outcomes for millions of heart attack survivors worldwide. We’re now actively planning the largest phase 3 clinical trial our company will have ever conducted as we await a bit more data in the second half of this calendar year.

On the recombinant side we recently entered into an exclusive research collaboration and worldwide license agreement with Momenta to develop and commercialize their Fc multimer proteins. Through this agreement, CSL has the potential to further grow and expand our long-term global leadership in immunoglobulins and in helping patients with autoimmune diseases.

As for acquiring assets, we do so only when it makes sense strategically. In July 2015 we completed the acquisition of Novartis’ influenza vaccine business, and integrated it with our subsidiary, bioCSL, to create Seqirus - the second largest influenza vaccine business in the world. And, just this June, we announced our intent to acquire a majority stake in Ruidie, a Chinese plasma fractionator. The plasma products market in China exceeded $3.3 billion in 2016, and it’s the fastest growing immunoglobulin (Ig) market in the world. The transaction will provide CSL with a strategic presence in the Chinese domestic plasma fractionation market and complements the leadership position that we’ve built over the past 20 years as a provider of imported albumin in China. Having a local manufacturing presence will now allow us to expand our efforts in delivering key therapies to patients with rare and serious diseases in this significant high-growth market.

Lastly, we are continuing to expand geographically and bring our medicines to new markets. CSL now supplies innovative medicines to patients in more than 60 countries.
Did you know? Rare diseases are not so rare: there are 7,000 rare diseases & disorders that combined affect 30 million Americans–1 in 10 of us–and more than half are children.

People with rare diseases have tremendous unmet needs, including misdiagnosis, a long time to finally receive a correct diagnosis, and when they do, 95% have no treatment with zero cures.

On May 24, 2017, Life Sciences Pennsylvania in partnership with the National Organization for Rare Disorders (NORD), patient advocates and members of the Rare Disease Caucus participated in Rare Disease Day - an annual event organized to raise awareness of the issues and problems facing those diagnosed with, and those innovating for rare diseases. A rare disease is categorized as a disease that affects one in 200,000 individuals. In Pennsylvania, it’s estimated that 1.2 million people are living with a rare disease. In conjunction with NORD and the Friedreich’s Ataxia Research Alliance, Life Sciences Pennsylvania organized meetings with state senators and representatives for patient advocates to share stories, testimonials, and data with the legislators and their staff. Patient advocates also discussed House Bill H.B. 239 which establishes a Rare Disease Advisory Council which will study the rare disease patient population, access to care issues, and the challenges of care givers in Pennsylvania - championed in the Pennsylvania House by Representative Marcy Toepel (R-147). This bill passed the legislature unanimously and was signed into law by Governor Wolf in July. Rare disease advocates had the opportunity to speak in the Main Rotunda, as well as various industry speakers including Dr. Marc Carr, Head of Clinical Development, Spark Therapeutics; Kristin Smedley, Carly Wolf, and Melanie Swick, NORD, and Marie Conley.

Life Sciences Pennsylvania extends a special thank you to Representative Paul Costa, Representative Mark Longietti, Senator Andrew Dinniman, Senator John Rafferty, and Senator John Disanto, for taking the time to meet with everyone involved.

On May 9th, the Life Sciences PA advocacy team led more than 80 guests representing all facets of the Pennsylvania life sciences community through the halls of the PA Capitol building for 2017 Advocacy Day: Innovators Meet Legislators. During this full day event, guests met face-to-face with legislative leaders and their staff, and discussed the importance of a supportive government to a growing life sciences industry.

Life Sciences Pennsylvania thanks all members of the Pennsylvania Legislature and their staff who took the time to meet with the individual groups. We look forward to continuing the conversations started well into the future.

If you are interested in participating in next year’s Legislative Day, please contact Craig Tucker at ctucker@lifesciencespa.org.
Dear PA Life Science Community:

I was humbled and honored to be recognized by Life Sciences Pennsylvania as the recipient of the State Official of the Year Award on March 9, 2017. As Co-Chair of the bipartisan bi-cameral Pennsylvania Senate Life Science Caucus, I understand and recognize the importance of the biotechnology, medical device and diagnostic, pharmaceutical industries and academic research institutions in the Commonwealth.

Pennsylvania needs to be a leader in the life science industry. The economic impact and benefit of attracting and retaining life science companies is astounding. These companies provide good family-sustaining jobs and I am always impressed with the innovative drugs and therapies that these companies provide to patient care.

Thank you for your contributions to life sciences which will inevitably help Pennsylvania become a leader in this industry. Please do not hesitate to contact me should you have any ideas, thoughts, or input to share with me. Thank you again for the award and thank you for your hard work and dedication.

Sincerely,

Bob Mensch | PA State Senator, 24th District
Majority Life Science Caucus Chair
On May 3, 2017, Life Sciences Pennsylvania held its annual Spring Social - Welcome to the Derby - a sold-out networking event focused on facilitating strategic connections between the diverse make-up of Life Sciences PA’s membership. Held at the Sheraton Valley Forge, this year’s event theme was The Kentucky Derby - driving unbridled enthusiasm and participation from the more than 300 event attendees. Guests enjoyed mint juleps while placing bets on horse races; visiting with the more than 30 exhibitors offering everything from contract manufacturing support to marketing assistance; and visiting Ripley the Racehorse - taking photos with guests under the Sheraton portico.

The Spring Social is designed to be a fun and stress free way for Life Sciences PA members to socialize in an engaging and relaxing atmosphere - and by all reviews, the 2017 Spring Social was exactly that.

To maximize time, Life Sciences PA partnered with Regulatory and Quality Solutions (R&Q) to offer a Medical Device Regulatory 101 session, focused on Critical Medical Device Updates for 2017, held earlier in the day.

Save the date for next year’s Spring Social - scheduled to be held on Wednesday, May 2, 2018.

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**SUPPORTED PATIENT ADVOCACY EVENTS**

- **AMERICAN LIVER FOUNDATION**
  - **LIVER LIFE WALK**
    - September 9, 2017 | Philadelphia
  - **WESTERN PA CHAPTER - NATIONAL HEMOPHILIA FOUNDATION**
    - **2017 HEMOPHILIA WALK/RUN FOR THEIR LIVES**
      - September 9, 2017 | Allison Park
  - **NEPHCURE KIDNEY INTERNATIONAL**
    - **GREATER PHILADELPHIA NEPHCURE WALK**
      - September 10, 2017 | Allison Park
  - **UNITED MITOCHONDRIAL DISEASE FOUNDATION**
    - **UMDF ENERGY FOR LIFE WALKATHON**
      - September 23, 2017 | Cherry Hill, NJ
  - **MAGEE-WOMEN'S RESEARCH INSTITUTE**
    - **SAVOR PITTSBURGH**
      - September 28, 2017 | Pittsburgh

- **FRIDRICH’S ATAXIA RESEARCH ALLIANCE**
  - **RIDEATAXIA PHILADELPHIA**
    - October 15, 2017 | Philadelphia

- **CROHN’S & COLITIS FOUNDATION**
  - **ANNUAL BALL**
    - October 21, 2017 | Philadelphia

- **AMYLOIDOSIS FOUNDATION**
  - **2017 AMYLOIDOSIS RESEARCH BENEFIT**
    - October 27, 2017 | Pittsburgh

- **EMILY WHITEHEAD FOUNDATION**
  - **BELIEVE BALL**
    - October 28, 2017 | King of Prussia

- **AMERICAN LUNG ASSOCIATION - MID-ATLANTIC CHAPTER**
  - **40TH ANNUAL PENN MEDICINE RADNOR RUN**
    - October 29, 2017 | Wayne
LIFE SCIENCES PA COMMUNITY CELEBRATES THE SCIENTIFIC LIFE OF JULIUS S. YOUNGER, SC.D.

On June 7, 2017, Pennsylvania’s life sciences community joined together to celebrate the scientific life of Julius S. Youngner, Sc.D., a pioneer in the field of virology. Best known for his pivotal role on the Polio Vaccine team in the 1950s, Dr. Youngner most recently served as American Distinguished Service Professor Emeritus in the School of Medicine and the Department of Microbiology & Molecular Genetics at the University of Pittsburgh.

Earlier this year, at the 2017 Annual Dinner, Life Sciences Pennsylvania awarded Dr. Youngner with the Scientific Achievement Award - a lifetime achievement award heralding his work with Dr. Jonas Salk in developing the polio vaccine - saving and improving countless lives around the world.

Joined by his family, and his wife, the life sciences community celebrated the scientific achievements and life of Dr. Youngner at Innovation Works in Pittsburgh. Dr. Youngner, or his groundbreaking work, will never be forgotten.

THE LIFE SCIENCES PA PAVILION WAS ABUZZ AT THE 2017 BIO INTERNATIONAL CONVENTION

Every year, thousands of representatives from the international BIO community come together for the BIO International Convention. This year’s event was held in San Diego June 19-22. Life Sciences PA led the charge for the Commonwealth, organizing the Life Sciences PA Pavilion in conjunction with numerous industry partners and regional leaders - including University City Science Center, PHL Life Sciences, University of Pittsburgh Innovation Institute, Brandywine Realty Trust, Helomics, SCOPE International, Fisher Scientific, Novasep, KinderPharm, Shire, Children’s Hospital of Philadelphia, Almac, and CSM. Life Sciences PA thanks its partners for another wonderful meeting - and looks forward to attending the 2018 BIO International Convention in Boston.

Julius S. Youngner was born in New York City. A brilliant student, he graduated from high school when he was 15, and then went on to get his bachelor's degree at New York University and a master's and doctorate in microbiology at the University of Michigan. He met his first wife, Tula Liakakis, in Michigan, and they married in 1943.

During World War II, after he was drafted into the Army, Mr. Youngner worked for the Manhattan Project, testing the effects of uranium salts and plutonium on lab animals at the University of Rochester. During those years, he and his colleagues speculated that they were working on some kind of nuclear propellant for missiles.

After a brief stint at the National Cancer Institute, Mr. Youngner came to Pittsburgh in 1949 to continue his work on learning to grow viruses in live cell cultures, and that is where he met Dr. Salk, who told him he would fund Mr. Youngner's research, as long as one of the germs he grew was the poliovirus.

Mr. Youngner made three key advances in the vaccine research. He first figured out a way to break down monkey cells so that the team could grow large amounts of poliovirus in the lab. Then, after the Salk group began to work on a vaccine, Mr. Youngner developed a way to inactivate the virus so it could safely be injected as a vaccine, and then developed tests to see how effective it was in the first human patients. He never intended to remain here, but “I got so involved in [the polio vaccine effort] that I decided to stay in Pittsburgh.”

As the vaccine project moved forward, Mr. Youngner said, “I was Mr. Inside and Jonas was Mr. Outside. He went out and got the money and built the lab.” The success of the Salk vaccine was quickly evident. The number of polio cases went from an average of 35,000 a year before the vaccine to fewer than 2,500 two years later.

By now, polio has been virtually eradicated in the United States, except for occasional outbreaks among groups that don’t get the vaccine. Today, only three countries in the world, Nigeria, Pakistan and Afghanistan, have not stopped transmission of polio, according to the World Health Organization. Asked years later to describe his impressions of Dr. Salk, Mr. Youngner said he was “smooth, glib, dynamic and self-absorbed, and a man who believed his own press notices.”

In the end, he concluded, “there was some merit for me in not having the kind of adulation Salk did because I was able to keep working and being productive and actually, he never did another thing scientifically in his career.”

Dr. Salk, with funding from the paralysis foundation, which had become known as the March of Dimes, went on to head his own research institute in La Jolla, Calif., while Mr. Youngner stayed on in Pittsburgh for the rest of his career. His first wife died in 1963 after suffering from Hodgkin’s disease for nearly 20 years. A year later, he married his current wife, Rina Balter, who went on to become an expert in the use of industrial imagery in art. He has two children from his first marriage, Stuart, a psychiatry and bioethics professor at Case Western Reserve University in Ohio, and Lisa, a ceramic and computer graphics artist in Albuquerque, N.M.

Reflecting on his long career in Pittsburgh, Mr. Youngner said: “I’m not one to have regrets, because my desires have been fulfilled. The things I had no control over, like disease that killed my wife — I regret that was out of my control. What was in my control, I always managed to make the right decisions in my career. I was not one of these people who planned ahead. Opportunities, when I was ready for them, appeared for me.”
SPECIAL WELCOME TO OUR Q2 NEW MEMBERS

ACADEMY OF NOTRE DAME DE NAMUR
ALEXANDERWERK INC.
AMYLOIDOSIS FOUNDATION
ARIEL PRECISION MEDICINE
AZZUR GROUP LLC
BANDISH GROUP, LLC
BOUNDARY MEDICAL
BUCHANAN PUBLIC RELATIONS
CANCER GENETICS, INC.
COURAGE GROWTH PARTNERS
CURING RETINAL BLINDNESS FOUNDATION
E.M.M.A. INTERNATIONAL CONSULTING GROUP
EDSPIRE
FRONTIDA BIOPHARM, INC
HCP CONCIERGE, LLC
IMMUNOMET THERAPEUTICS INC.
IMVAX
LAWLEY
MASTERCONTROL, INC.
MAZARS USA, INC.
MEKHOS HEALTH LLC
MVM LIFE SCIENCE PARTNERS LLP
NEPHCURE KIDNEY INTERNATIONAL FOUNDATION
ONCORA MEDICAL, INC.
OPTINOSE
PENDULUM CLINICAL DEVELOPMENT CONSULTANTS, LLC
PROLIFAGEN LLC
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SD MEDED, INC.
TROVITA HEALTH SCIENCE
VALLEY FORGE TOURISM & CONVENTION BOARD
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WITHUMSMITH+BROWN
WUXI APPTEC, INC
YOUR GENERAL COUNSEL LLC

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