

# Cutting edge

*Life sciences are gaining ground on big health concerns.*

In the life-sciences arena, researchers are constantly working on high-tech solutions to complex problems that can run the gamut from addiction medicine to gene therapy that treats heretofore incurable diseases. In this special section, you will read about advances in medicine and other health issues taking place all over North Carolina. A collaboration of patients, physicians, health care providers, technologists, scientists, engineers, public-health officials and others is working toward solving the nationwide epidemic of opioid addiction using digital health. A technology company is working on nutritional advice, medication choice and lifestyle suggestions through the study of genomes and microbiomes. In Pitt County in eastern North Carolina, partners are collaborating on pharmaceutical education and workforce development for new and existing pharma companies. And a dedicated researcher is exploring a genetic solution for diseases such as Duchenne muscular dystrophy, which strikes young boys. His gene-therapy research company was acquired by Pfizer, a major drug company. The North Carolina Biotechnology Center has brought together academia, technology, pharma, research institutions, health insurance, health care providers and others to stimulate growth in the precision health sector. That work is generating excitement in the industry but more important, hope for children and adults who suffer from debilitating diseases.

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# LOOKING FOR A CURE

Gene therapy is the current frontier for researchers and doctors who aim to discover treatments for rare and debilitating diseases.

As a young researcher in the early 1980s, Jude Samulski made a name for himself in the world of gene therapy when he worked with adeno-associated virus as a vector to efficiently transport therapeutic genes into a patient to treat a select variety of devastating diseases.

“Think about vectors as FedEx trucks delivering a genetic payload that will cure diseases,” he says.

His work focuses on gene therapy to correct the genetic mutation that causes Duchenne muscular dystrophy, a fatal muscular disease affecting mostly males. Boys who are born with this genetic mutation may seem healthy at birth, but their muscles begin wasting away when they are toddlers. They continue to regress until they can no longer walk. As the disease progresses, it affects the muscles that control breathing and the heart, eventually causing death.

Samulski, director of the UNC School of Medicine Gene Therapy Center, formed Bamboo Therapeutics Inc. in 2014 to further his research. The company is a spinoff from Asklepios BioPharmaceutical Inc., launched with funding from angel investors, foundations and the parents of children suffering from neuromuscular diseases. The North Carolina Biotechnology Center provided more than \$700,000 to support research and development.

“I started Bamboo because parents were knocking on my door and asking, ‘Can you save my children?’” Samulski says. “These parents are desperate. If a child is born with a genetic mutation that causes a debilitating disease and is never able to walk and has to spend the rest of his life in a wheelchair, it’s heartbreaking.”

Gene therapy is one area of precision medicine the N.C. Biotechnology Center supports. But NCBiotech is taking a view beyond precision medicine and biotechnology innovation to broader opportunities for disease prevention by addressing population health and public health, according to Sarah Imhof, senior director of the center’s precision-health initiative.

“We have built an interdisciplinary approach and formed the North Carolina Precision Health Collaborative (NCPHC) to bring together academia, technology, pharma, nonprofit research institutions, the health insurance sector and health care providers to stimulate growth in the precision-health sector,” she says.

According to Kenneth Tindall, NCBiotech’s senior vice president of science and business development, the effort is an ecosystem.

“That’s really the strength of our collaborative here,” he says. “Rather than silo the efforts of individual researchers, health care providers, pharmaceutical manufacturers and others, the collaborative can do things that no organization would do alone.”

The NCPHC is at work on a pilot project to screen North Carolina residents for conditions that have a good chance of being prevented or stopped, focusing on diseases such as breast and ovarian cancer. The cooperative also is working with the National Institutes of Health “All of Us” program to educate health care professionals about the value and opportunities of precision medicine. The idea is to collect information about hereditary medical conditions, factoring in an individual’s genetic makeup, environment and lifestyle choices.

“Through the precision-health initiatives, we focus closely on actionable genetic mutations so we know exactly what we could do with that information when we study hereditary diseases like cancer, heart disease, high cholesterol and others,” Imhof says. ■

— Teri Saylor is a freelance writer from Raleigh.

## BAMBOO’S BIG DEAL

Jude Samulski has devoted his life’s work to gene therapy, motivated by parents of children with devastating diseases such as muscular dystrophy, cystic fibrosis, hemophilia and others. Samulski, director of the Gene Therapy Center at UNC Chapel Hill, focuses his work on technology that uses recombinant adeno-associated virus (AAV) as a vector to carry normal genes into the cells of patients who have faulty genes that cause disease.

In 2014, Samulski formed Bamboo Therapeutics, a biotechnology company that uses AAV in therapies for four severe diseases that cause degeneration and deterioration in nerves and muscles. Pfizer, a major drug company, embarked on gene therapy four years ago, with a goal of finding potential solutions to 7,000 rare monogenic diseases, says Robert Smith, Pfizer’s senior vice president for business development and alliance management in Philadelphia.

In January 2016, Pfizer made an equity investment in Bamboo of \$43 million, and later that year, paid \$150 million to buy the company and integrate it into Pfizer’s rare-disease unit, according to Smith. The unit is located at Pfizer’s manufacturing facility in Sanford.

This therapy is anticipated to slow or stop progression of monogenic diseases and is predicted to be transformational for patients who are already receiving treatment through clinical trials.

“Pfizer treated the first patient with muscular dystrophy on March 22,” Samulski says. “This was a real milestone.” During the first trial, Pfizer will treat a dozen boys between the ages of 5 and 12 while they are still ambulatory, so doctors can monitor the treatment’s ability to slow the disease, Smith says. He hopes to start seeing results in two years.

# 'THE SCIENCE IS ENDLESS'

Technology is in place to use microbiomes and genetics to help people make better choices in nutrition, health and lifestyle.

Technology is enabling scientists to take giant steps in understanding the role genetics plays in human health. Not only are they able to focus on individual needs, they can use precision-health methodologies to select medicines, provide nutritional advice and make lifestyle suggestions based on our genetics, and even our microbiomes.

In North Carolina, efforts are underway to study genomes and microbiomes to determine how to grow healthy food and help people learn precisely what types of foods satisfy their needs.

Metabolon Inc., an 18-year-old technology firm near Research Triangle Park in Durham, specializes in metabolomics, the study of small molecules that result from the metabolic processes in our bodies and are interwoven in our genetic makeup. When our biological systems are disrupted by disease, genetic mutations or environmental factors, our metabolic profile often changes.

Metabolomics can be used either alone or in conjunction with genome study to create a more complete understanding of health.

Using its trademarked Precision Metabolomics technology to identify biomarkers of wellness and disease, Metabolon can measure individual microbiomes to determine if disruption has occurred and examine other factors, such as genetics, environment, diet and lifestyle to make adjustments to bring the body back into balance.

"For example, we might take a blood sample that is shipped to us. We extract it for a metabolomics analysis using our software and methods we've developed and deliver data and a consulting interpretation," says Mike Milburn, Metabolon's chief scientific officer.

At the UNC Nutrition Research Institute in Kannapolis, Martin Kohlmeier is using genetics to target precise individual nutrition needs.

"Using our technology, we can measure precisely how many calories a person needs to consume, or how many



Rohan Hastie is the chief business officer at Metabolon, a firm that uses metabolomics technology to promote wellness and to diagnose, treat and monitor disease.

calories they burn per day within a range of 60 or 70 calories," he says.

Dedicated to helping the public benefit from advancements in genetics and nutrition, Kohlmeier is forging pathways to enable people to help themselves by submitting their own biological samples for testing and using the results to design a healthy lifestyle.

"The science is endless," he says. "There are 3 billion DNA spellings in our genes, and that makes a big haystack. We are always making amazing discoveries and enjoy bringing genetics to a practical level that consumers can understand and use."

The BioAg Alliance, an innovative partnership between Novozymes, a global biotechnology company with its North American headquarters in Franklinton, and agricultural giant Monsanto, has resulted in development of a biological seed treatment to help plants build healthy root systems to produce a higher yield.

"The goal of this product was to make plants healthier and more resistant to disease," says Ejner Bech Jensen, vice president of bioag research and applications at Novozymes. "Agricultural growth is becoming harder and harder, due to extreme weather conditions and less land for farming due to development. If microbes can help grow even 10% more food, it would make a major impact on food supply for future generations." ■

— Teri Saylor is a freelance writer from Raleigh.

# DIGITAL SOLUTIONS

The future of health care lies in the development of technology that uses digital devices and software to transform outcomes.



Leyan Phillips, left, and Michael Levy are co-founders of Bluedoor, a collaborative network of key players in health care that has joined with Digital Health Impact + Transformation to affect quality of care.

The opioid epidemic has hit populations from small cities and rural areas hard, spurred, in part, by the lack of access to alternative treatments for chronic pain from injuries, surgeries and illness. This epidemic has turned deadly in recent years and today, opioids obtained through prescriptions and illicit activity have grown into the main driver of drug overdose deaths, according to the Centers for Disease Control and Prevention.

The CDC reported that opioids were involved in 42,249 deaths nationwide in 2016, five times more than in 1999. North Carolina saw significant overdose death rates between 2015 and 2016, and a 31% increase in emergency-room visits due to opioid overdoses.

In North Carolina, a new partnership is at work building an ecosystem to bring together patients, health care providers, technologists, scientists, engineers, public-health officials and others to develop innovative solutions to this complex problem. Led by Digital Health Impact + Transformation, a social-benefits corporation founded in 2017, this partnership is exploring digital initiatives to address North Carolina's ongoing opioid abuse epidemic.

"At DHIT, we believe there are tremendous opportunities for digital health technologies to assist and complement community efforts to alleviate health problems like the opioid crisis," says Chairman Don Turner.

Digital health is a rapidly growing area of innovation at the intersection of human health, digital devices and software. Digital devices can serve as bridges between consumers or patients who need health assistance and health care providers and researchers who can use the data generated to create solutions for individual patients or the public at large through research.

Michael Levy, a DHIT co-founder and CEO of Bluedoor, a collaborative network of health care providers and experts;

software and hardware developers; entrepreneurs; and strategists, says digital health care technology will lead to improving the quality of patient care instead of relying on quantity of care as in years past.

"As you know, health care is an extraordinary commercial reality," Levy says. "It's just a matter of how you align incentives to best drive impact into the ecosystem as defined by reducing costs, increasing access and enhancing experience."

Bluedoor and DHIT have joined forces to address supply and demand, give consumers better access to health care, lower costs and improve patient outcomes. They have identified the opioid crisis as an area where they can make a strong impact.

In March, DHIT and its partners published a white paper on "The State of the Opioid Crisis in North Carolina," which examined a variety of digital health strategies that could bring solutions to people across the spectrum, even in rural areas where there is less access to traditional health care providers.

Broadband access is beginning to reach more remote places, increasing opportunities for technology to make a difference.

"The problem has a wide range of scenarios and stages, stemming from chronic pain to addiction, and it wrecks lives," Turner says. "Here's the reality: We know there is not just a simple solution to quickly end the problem." ■

— Teri Saylor is a freelance writer from Raleigh.

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# HIGH-TECH TRAINING

The pharmaceutical manufacturing sector in eastern North Carolina gets an important boost from an innovative, collaborative effort.



The North Carolina Pharmaceutical Services Network (NCPSN) is the newest innovation for Pitt County and eastern North Carolina, bringing together a variety of partners in a one-of-a-kind collaboration: A manufacturing lab that offers pharmaceutical education, workforce development and training services for new and existing pharma companies in the region and across the state.

The network's evolution began in 2012 but built a head of steam last year. Thanks to a \$1.7 million grant from the Rocky Mount-based Golden LEAF Foundation, more than \$300,000 from the Pitt County Development Commission, \$100,000 from the Pitt County Committee of 100 and \$40,000 from the N.C. Biotechnology Center, the network has been able to convert a section of Greenville's Technology Enterprise Center into a state-of-the-art educational facility.

The training facility has conducted 11 training sessions since opening its doors last January and has already trained more than 120 employees of Mayne Pharma and Thermo Fisher Scientific in how to manufacture oral solid dosage pharmaceuticals, or pills.

"As Thermo Fisher Scientific grows and expands, attracting key talent is important to us," says Michelle Logan, business unit director. "Our employees get hands-on experience with small-scale equipment and are able to learn on it before tackling larger-scale batch equipment. This is driving talent."

The Pitt County Economic Development Commission saw an opportunity to serve existing industry and develop a recruitment tool when Thermo Fisher (formerly Patheon) and Mayne Pharma announced expansion plans, according to executive director Wanda Yuhas.

Workers at Thermo Fisher Scientific and Mayne Pharma can train in a manufacturing lab developed by the North Carolina Pharmaceuticals Network in Pitt County.

The pharmaceutical manufacturing sector already supports nearly 2,000 employees in Pitt County, and that number swells to 10,000 across the state's eastern region, according to Yuhas. "In five years, our region has generated close to \$5 billion in investment, with more to come. The network makes a direct correlation between people and their skills to companies and their needs," she says.

The center offers a two-and-a-half-day hands-on training program every two weeks, in a small group setting, according to Mark Phillips, director of the North Carolina Biotechnology Center's eastern region who helped establish the partnership.

"At this point, the facility is focusing on training employees of Thermo Fisher Scientific and Mayne Pharma, but we expect it to branch out to new hires and eventually be used as a way to introduce a new workforce to this type of manufacturing process," he says. He added the training center would be an ideal resource for retired military personnel who live in the area.

The center serves brand new employees, as well as 20-year veterans of the pharmaceutical manufacturing industry.

"Customized training is catching on," says Mike Renn, who is the principal instructor. "We see our efforts in workforce development and education as a tool to strengthen the pharmaceutical industry in the eastern region of the state. It's also an economic-development tool. We have a nice training facility, and pharmaceutical companies are already starting to take notice." ■

— Teri Saylor is a freelance writer from Raleigh.



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