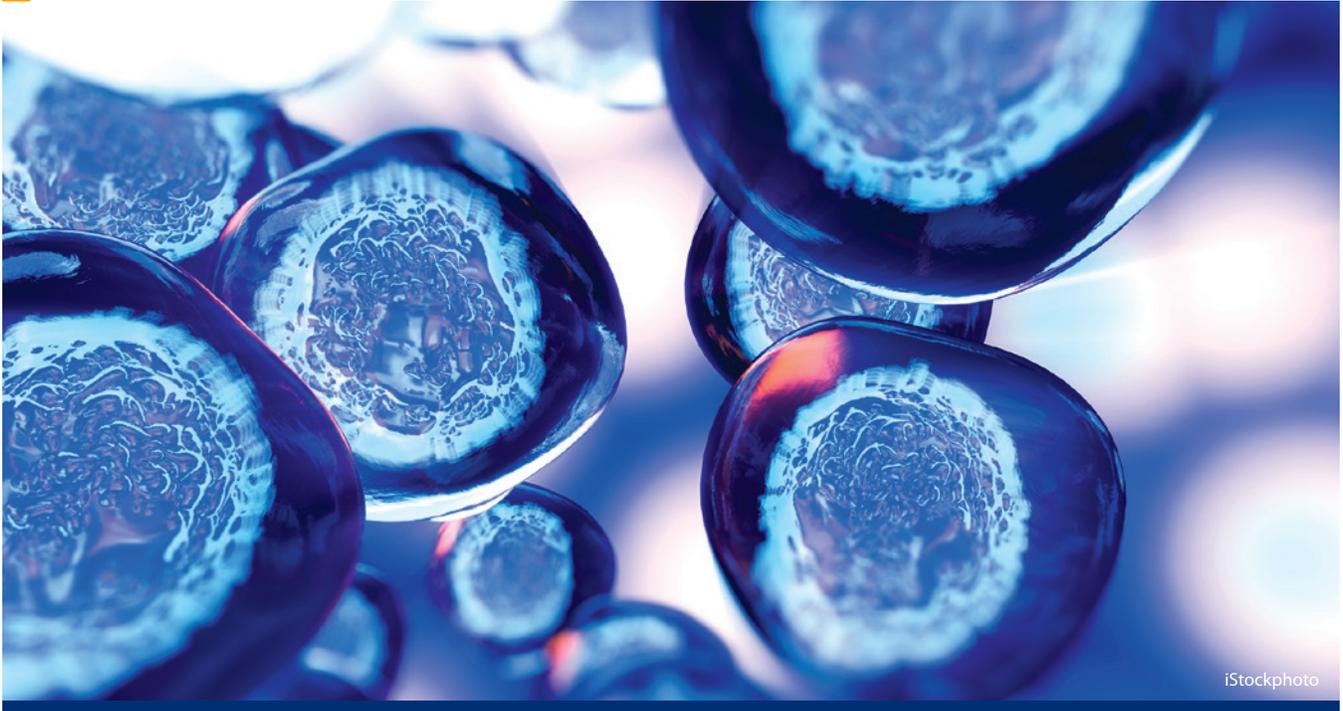


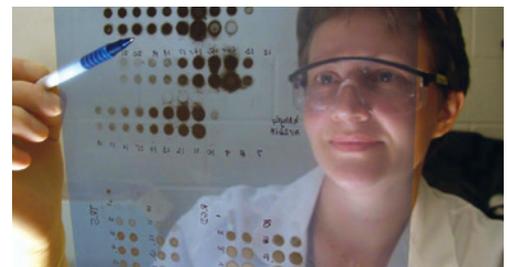
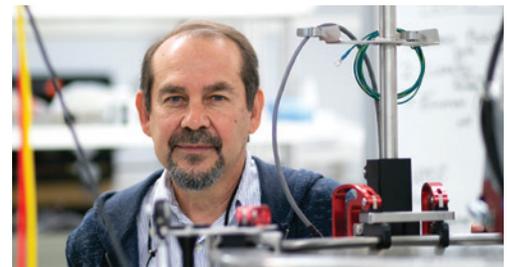
# North Carolina: A Flourishing Gene and Cell Therapy Hub



North Carolina has established itself as a leading hub of gene and cell therapy activity. As curative therapies emerge and bring new hope to patients with rare diseases and cancer, North Carolina's life sciences ecosystem provides a complete end-to-end solution to bring life-changing therapies from concept to market.

The state's gene and cell therapy sector is built upon world-class academic and corporate research and the largest concentration of clinical research organizations in the world. Pilot- and commercial-scale manufacturing capabilities bring these therapies to life. Our pool of top talent rounds out North Carolina's strengths that fully support the growth of gene and cell therapy companies.

This wasn't an overnight success story. In 1993, The University of North Carolina School of Medicine, with grant help from the North Carolina Biotechnology Center, recruited gene therapy visionary Jude Samulski, Ph.D. Samulski pioneered the use of the harmless recombinant adeno-associated virus (AAV) as the premier delivery mechanism for gene therapy. This AAV technology is the foundation used by more than two-thirds of the gene therapy industry worldwide. It was brought to life in a North Carolina university, and to commercial reality by **Asklepios BioPharmaceutical (AskBio)**, a Research Triangle Park company co-founded by Samulski. And that's just the beginning of the North Carolina cell and gene therapy story.



(top) Jude Samulski, Ph.D. Photos courtesy of AskBio.  
(bottom) AskBio uses tiny genetic "trucks" to deliver DNA fixes for specific diseases.

# A Diverse, Established Cluster

From large pharmaceutical companies to university spinouts, North Carolina's gene and cell therapy cluster is broad and diverse. These companies are leaning on North Carolina's deep expertise in life sciences to transform research into commercially viable products that can be manufactured at scale. Below is a sample of companies in North Carolina that are making significant progress in the development and delivery of gene and cell therapy technologies. This list grows each month.

- **Abzena** is investing \$213 million to build a new biopharma manufacturing site, adding 325 employees in Sanford. The company will produce cell-based biologics for academic research labs and pharmaceutical companies.
- **Adverum Biotechnologies** is investing \$82 million in a manufacturing site in Durham that will employ 202 people. The site will produce AAV vector-based gene therapies targeting serious ocular and rare diseases.
- **AskBio**, a premiere gene therapy platform company whose founders pioneered AAV therapies, has been acquired by Bayer AG for up to \$4 billion.
- **Astellas Gene Therapies** is investing in a \$110 million facility and adding 209 jobs in Sanford. The company's gene therapies are based on the AAV vector and target serious rare genetic diseases.
- **Beam Therapeutics** is building an \$83 million biomanufacturing facility in Durham that will employ 200+ people. The company is developing precision genetic medicines through CRISPR technology known as base editing, aiming to create cures for serious diseases.



Abzena's new cGMP facility in North Carolina will focus on making mammalian biologics for Phase 3 clinical trials as well as commercial products.

*Since 2016, Pfizer has invested more than \$1.2 billion in expansions and acquisitions in North Carolina to secure its end-to-end capabilities in gene therapy including research, development and manufacturing.*



**August 2016**

*Acquires AskBio spinout Bamboo Therapeutics with an upfront payment of \$150 million and eligible payments up to \$495 million if milestones are met*

**February 2017**

*Provides \$4 million in funding for a multi-year gene therapy academic fellowship program managed by NCBIotech*

**August 2017**

*Announces a \$100 million investment in its Sanford gene therapy manufacturing operations*

**March 2019**

*Administers Duchenne muscular dystrophy therapy to first human patient at Duke University with successful results*

**August 2019**

*Announces an additional investment of \$500 million and 300 more jobs for its gene therapy site in Sanford*

**October 2019**

*Invests \$19 million to renovate a 60,000-square-foot building in Durham into its Advanced Therapy Medicinal Facility*

## A Diverse, Established Cluster (continued)

- **Biogen** is investing \$200 million and adding 90 jobs to support the clinical manufacturing of its gene therapy pipeline.
- **Bluebird bio** opened its first wholly owned manufacturing facility in Durham, producing lentiviral vectors for its investigational gene and cell therapies targeting rare diseases and certain cancers.
- Shanghai-based cancer immunotherapy innovator **CARsgen** is investing \$157 million in Durham and employing 200 people to develop and manufacture its chimeric antigen receptor T cell cancer therapies.
- Paris-based **Collectis** is investing \$70 million and adding 200 jobs in its first North American manufacturing facility in Raleigh. The company is developing a new generation of cancer therapies.
- Based on research of North Carolina State University's Rodolphe Barrangou, Ph.D., **Locus Biosciences** signed an exclusive collaboration and license agreement worth up to \$818 million with **Janssen Pharmaceuticals**, a Johnson & Johnson company targeting infections of the respiratory tract and other organ systems.
- **Novartis Gene Therapies** is investing \$60 million more into its Durham County manufacturing facility and doubling its workforce to 400, anticipating final FDA operational approval in 2021.
- **Pfizer** has invested more than \$1 billion in acquisitions and expansions in North Carolina specific to its gene therapy capabilities, including AskBio spinout **Bamboo Therapeutics**.
- Duke spinout **Precision BioSciences** has entered into a gene editing research collaboration and exclusive license agreement with **Lilly** for up to six gene targets, with an initial focus on Duchenne muscular dystrophy.
- UNC spinout and AAV vector technology company **StrideBio** is partnering with **Sarepta** providing all R&D work up to the IND stage on four central nervous system targets.
- **Taysha Gene Therapies** is investing \$75 million and adding a 201-employee manufacturing site in Durham. The company is developing AAV vector-based gene therapies targeting diseases of the central nervous system.

Many of these companies are located in the Research Triangle, a centrally located region named for three world-renowned universities—University of North Carolina at Chapel Hill, Duke University and North Carolina State University—at its three vertices.



Rendering of Biogen's new RTP facility that will support gene therapy production for its clinical pipeline.



Collectis team announcing plans for the company's first North American manufacturing facility in Raleigh.



Locus Biosciences and Janssen Pharmaceuticals have an exclusive collaboration worth \$818 M to advance therapies for respiratory and other infections.



Astellas Gene Therapies and N.C. government leaders announced the company's plan to build a \$109 M production facility in Sanford.

# A Thriving Ecosystem

Gene and cell therapy companies succeed in part because of North Carolina's life sciences ecosystem. Emerging and established companies alike can benefit from the connectivity among North Carolina's specialized organizations, top-tier universities, and statewide medical experts.

North Carolina has plenty of expertise at the front end of the gene-and-cell-therapy pipeline. Top researchers in the field call Duke, NC State and UNC home.

At the **Duke Center for Advanced Genomic Technologies (CAGT)**, director and CRISPR expert Charlie Gersbach, Ph.D., leads integration of research across disciplines to understand more about human DNA and ultimately to identify new treatments for disease. Also under the CAGT umbrella, Aravind Asokan, Ph.D., is developing the next wave of AAV-derived therapies alongside Kris Wood, Ph.D., who works on designing new anticancer therapeutic strategies.

At **North Carolina State University**, Rodolphe Barrangou, Ph.D., works with CRISPR technology, using the Cas3 enzyme for its molecular scissors. This tool may be more useful for targeting a broader range of disease. The technology underpins local startup Locus Biosciences' treatments for antimicrobial resistance and a range of other applications, including agriculture.

And AAV pioneer Jude Samulski, Ph.D., helped to create the **Gene Therapy Center at the University of North Carolina at Chapel Hill** in 1993. Two core facilities—the **UNC Vector Core** and **Human Applications Laboratories**—facilitate the progression and translation of gene therapy research from the laboratory bench into Phase 1 clinical trials to treat human disease.<sup>1</sup>

Companies can tap research and clinical expertise at three NCI-designated Comprehensive Cancer Centers: **Duke Cancer Institute**, **UNC Lineberger Comprehensive Cancer Center**, and **Wake Forest Baptist Comprehensive Cancer Center**. More than 700 clinicians and researchers focus on cancer genetics and genomics, cellular therapies, clinical research, and cancer prevention and control, among others.

Three top-tier research universities give Research Triangle its name



Grifols | Plasma fractionation



Biogen | Multiple product support



Novo Nordisk | API facility



<sup>1</sup>UNC Medical School, Gene Therapy Center, 2021. <https://www.med.unc.edu/genetherapy/about-us/>

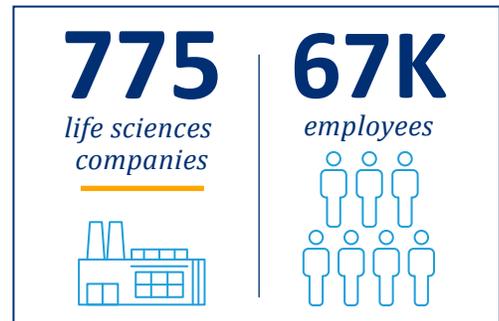
## A Thriving Ecosystem (continued)

These many avenues of research yield therapies that go into pre-clinical and clinical testing, where North Carolina once again owns the landscape. More than 150 contract service organizations call North Carolina home including industry founders **IQVIA**, **PPD**, and **LabCorp**. Along with innovative leaders **Rho**, **Syneos Health** and dozens more, these companies manage more than 4,600 active clinical trials.

Production of these potentially lifesaving therapies happens in a cluster of 775 companies, 131 of which are dedicated to production and manufacturing. Of the 67,000 total life sciences employees state-wide, more than 30,000 skilled workers manufacture pharmaceuticals, monoclonal antibodies, cell and gene therapies, industrial enzymes, and vaccines. Compared to other U.S. life sciences hubs, North Carolina has the most biological product manufacturing employees.<sup>2</sup>

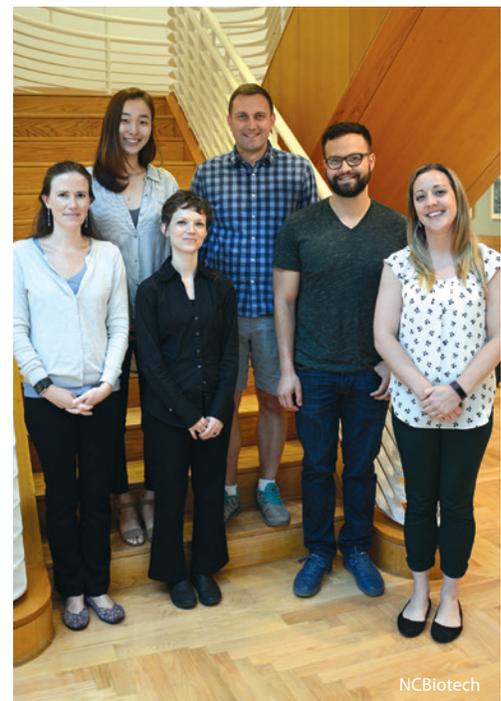
Research, development, and manufacturing require a broad spectrum of support companies. North Carolina has nearly 2,500 firms that handle staffing, IT, construction, facility design, intellectual property and much more. The state-funded North Carolina Biotechnology Center tailors its programs to meet the needs of the evolving industry, from startups to multinational companies. One initiative, the **North Carolina Precision Health Collaborative**, brings together leaders in research, healthcare, insurance, investment, policy and information technology to accelerate activities that nurture research, equip providers, and engage industry in this sector.

A second program specific to gene therapy is the **Pfizer-NCBiotech Distinguished Postdoctoral Fellowship Program in Gene Therapy**. The program supports the scientific and professional development of postdoctoral fellows interested in establishing careers in gene therapy.



**North Carolina:**  
**#1 in biological product  
manufacturing employees  
in the U.S.<sup>2</sup>**

Inaugural class of Pfizer-NCBiotech Postdoctoral Fellows  
in Gene Therapy



<sup>2</sup>U.S. Bureau of Labor Statistics, Quarterly Census of Employment and Wages for NAICS code 325414

# Talent to Drive Creativity and Commercialization

Companies from Astellas to Taysha choose North Carolina time and again for its robust talent pipeline. A workforce of 67,000 grows each year, fueled by 4,900 biological and biomedical sciences graduates. The community college system, working closely with industry, runs certificate and associate degree programs tailored specifically to industry needs. The system is providing customized training for cell- and gene-therapy facilities including Beam Therapeutics, bluebird bio, Novartis Gene Therapies, and more.

These training partners work together in the NCBioImpact consortium to focus on bio and pharma manufacturing training that aligns with industry needs. The partnership created a certificate program for process technicians in 2000, and the partners continue to monitor industry growth and training needs, now including gene therapy. The training partners include:

## NC State's Biomanufacturing Training and Education Center (BTEC).

Undergraduates, graduate students, and working professionals including FDA inspectors train at BTEC for hands-on learning with the latest biomanufacturing technologies. This facility includes a cGMP pilot plant and industry-standard equipment that helps prepare students for on-the-job, real-world application of the technologies. BTEC's range of courses recently expanded to include production of AAV vectors.

In Durham, **North Carolina Central University's Biomanufacturing Research Institute and Technology Enterprise (BRITE)** offers undergraduate and graduate degree training. Students move through curricula designed with industry input to ensure that graduates learn up-to-date training techniques. BRITE has a strong focus on research and drug discovery.



NCSU/BTEC

BTEC was first to offer graduate degrees in biomanufacturing in the U.S.

**NC BioNetwork's flagship course BioWork** teaches foundational skills needed to enter the biomanufacturing workforce in a process technician role.

Ten community colleges across the state offer the BioWork certificate program, a 136-hour course that teaches the science and bioprocessing techniques required for entry-level positions. Designed for people with a wide range of educational backgrounds, the course provides thorough comprehension of current Good Manufacturing Practices, current chemistry for process manufacturing, process flows, fermentation, cell growth and more. Visit [ncbionetwork.org/biowork](http://ncbionetwork.org/biowork) for specific details.



NC BioNetwork

NC BioNetwork prepares students for entry-level positions in biomanufacturing.



NC BioNetwork

NC BioNetwork develops custom training programs for companies growing their talent pipeline.

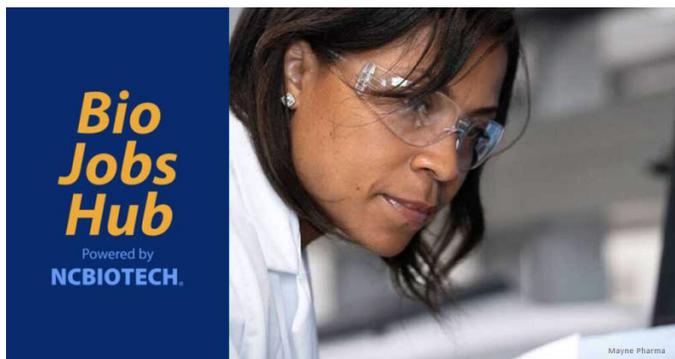
## Talent to Drive Creativity and Commercialization (continued)



Industry professionals have helped to shape the curricula at BRITE, preparing students for careers in research and drug discovery.

**NCBioNetwork** is the group of community colleges that provide certificate and degree training for biopharma manufacturing jobs. All 58 campuses support scientific degrees and custom training for this sector. In 2020, the network directly supported 81 companies with 264 courses.

The **North Carolina Pharmaceutical Services Network** was created as companies in Eastern North Carolina expanded. The partnership between Pitt Community College and East Carolina University provides GMP/GLP courses, analytical services training, and a pilot-type manufacturing environment to develop company-specific skills.



The Bio Jobs Hub educates non-scientists on job opportunities and training in North Carolina's growing biopharma manufacturing industry. Visit [biojobshub.com](http://biojobshub.com) for details.

### **Workforce Initiatives**

*NCBiotech champions workforce development initiatives to continue to fill the state's talent pipeline.*

*NCBiotech is a strong partner in workforce development. Its programs engage veterans, transitioning military members, and their spouses, linking them to training geared toward their experience. Another program enlists graduating high school seniors who meet specific math and science requirements in a weeklong training program that, upon completion, qualifies them for interviews with Thermo Fisher. Other programs work in underserved areas to increase diversity in the workforce pipeline, and to attract nontraditional workers to these jobs.*



NCBiotech's Veterans Outreach Program has created pathways for those transitioning from the military to springboard to a new career in biopharma manufacturing.



Thermo Fisher hired Diana De Leon after she completed the Pharma K12 Workforce Training that provides high school seniors in Pitt County with an introduction to drug manufacturing.

# Exceptional Business Climate and Quality of Life

North Carolina has an advantageous business climate and offers an **exceptional quality of life** to its residents.

Compared to other life sciences hubs, North Carolina's **cost of doing business is lower**. N.C. boasts the lowest corporate income tax rate at 2.5%. Utility costs are below the national average, and infrastructure and water are abundant here. In biomanufacturing specifically, labor and operating costs in North Carolina's Research Triangle Region are the lowest of all major U.S. hubs. In fact, it is 24% less expensive to operate in North Carolina than the Boston and San Francisco areas.<sup>3</sup>

Targeted, performance-based incentives are provided through the North Carolina Department of Commerce for companies expanding in the state.<sup>4</sup> The commerce department works closely with the Economic Development Partnership of North Carolina to support business growth in the state.<sup>5</sup>

North Carolina's **prime mid-Atlantic location** provides easy access to millions of people. The state's infrastructure includes four major international airports, the largest consolidated railroad system in the country, and the second-largest highway system with more than 90,000 miles of road.

As a **culturally rich state**, North Carolina has something for everyone. The iconic Blue Ridge Mountains. Hundreds of miles of coastline dotted with lighthouses. Bustling cities. The moderate climate includes mild winters, warm summers and enjoyable spring and autumn seasons. Arts and culture, professional and nationally ranked sports teams and a welcoming cost of living round out a first-rate business climate, making North Carolina the optimal destination for businesses.

[Learn more at \*\*ncbiotech.org/genetherapy\*\*](https://ncbiotech.org/genetherapy)

[Contact NCBiotech for information on workforce, competitive landscape, and resources to accelerate growth.](#)

[Contact EDPNC for a no-cost consultation on site identification and state incentives.](#)

*Site Selector ranked North Carolina #1 for business climate (2020)*



*Boyd Company report confirms North Carolina's lower biomanufacturing operating costs*



*North Carolina has mountains, cities, and beaches to enjoy*



<sup>3</sup>The Boyd Company Inc. "Comparative Biomanufacturing Industry Operating Costs." 2019

<sup>4</sup>[nccommerce.com/grants-incentives/competitive-incentives](https://nccommerce.com/grants-incentives/competitive-incentives)

<sup>5</sup>[edpnc.com/why-north-carolina/incentives/](https://edpnc.com/why-north-carolina/incentives/)

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