

North Carolina: a burgeoning gene therapy hub

\$1 of **\$9**

*NIH dollars for rare disease
flow through North Carolina*



5X

*growth of global
gene therapy market
by 2026*

1800+



*clinical
trials for
gene
therapies*

Sources: GEN, May 2018; Grandview Research Inc., Global Gene Therapy Market Size, Analysis, Industry Report 2018-2026

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An industry-leading cluster, first-of-its-kind manufacturing, and expertise in training, regulatory, policy, and clinical trials make North Carolina the place to locate or scale your company.

Gene therapy products are rapidly making their way into the market. They give new hope to patients with rare diseases and cancers, and also present new challenges to production, distribution and reimbursement, as compared to other therapies.

Several of the leading players in this space are now calling on North Carolina's deep expertise in life science and biomanufacturing. Its central location, leading university research and industry-focused training programs accelerate these novel treatments to market. Consider:

- **bluebird bio's** 125,000-square-foot facility in Durham, which will manufacture lentiviral vector for use as gene therapies targeting rare diseases and certain cancers.
- a new RTP facility under construction for **AveXis**, a Novartis company. AveXis will manufacture its treatment for spinal muscular atrophy here.
- **Pfizer** invested \$100 million to add AAV gene therapy production capability to its Sanford facility.

Pfizer's expansion followed its acquisition of **Bamboo Therapeutics**, a University of North Carolina at Chapel Hill spinout headed by AAV pioneer Jude Samulski, Ph.D. Bamboo is just one of several startups pushing the boundaries of **gene therapy** technologies:

- Also using AAV vector technologies, UNC spinout **StrideBio** recently closed a \$15.7 million investment to continue development of its therapies for rare diseases.
- **Locus Biosciences**, based on the research of North Carolina State University's Rodolphe Barrangou, is using CRISPR-Cas3 to create precision antimicrobial treatments targeted to drug-resistant bacteria and the microbiome.
- In Durham, Duke spinout **Precision BioSciences** recently inked a deal with Gilead to develop hepatitis B treatments. The potential \$445 million collaboration is one of many human health applications of Precision's ARCUS gene-editing platform.

An ecosystem of expertise to transform your technology

Nucleus of Clinical Research

More than 1,800 gene therapy clinical trials have been conducted to support the development of these therapeutic platforms. North Carolina, as the birthplace of CROs, has expertise in trial design, patient recruitment, data management, and regulatory requirements. Find what you need from industry founders **IQVIA** and **PPD**, or innovative leaders **Rho** and **Syneos Health**, or **LabCorp** and any of the 120 other contract research organizations that call North Carolina home. Partner with one of our three NCI-designated comprehensive cancer centers.

With positive clinical results, nearly 200 manufacturing consulting firms are available to guide you through scaling production. With the state's emerging cluster, these firms are familiar with cGMP, commissioning and validation, as well as the changes in manufacturing methods for gene therapy vectors and cell therapy treatments.

Talent Pipeline

North Carolina's biopharma manufacturing community is more than 125 companies strong with 26,500 trained workers. A unique and long-standing partnership between industry and training partners—called **NCBioImpact**—continues to propel this highly skilled workforce. The three training partners deliver hands-on training on industry-standard equipment. Standard courses and custom-designed training are both available. (More at ncbioimpact.org)

A dozen or more North Carolina companies and universities are active members of the **National Institute for Innovation in Manufacturing Biopharmaceuticals**. The Golden LEAF Biomanufacturing Training and Education Center at NC State is developing a hands-on training course to teach viral vector manufacturing with the support of a NIIMBL grant. In addition to this biomanufacturing-focused training, North Carolina's universities award 4,800 biomedical and biological science degrees each year, and 4,200 engineering degrees.

A Partner for Growth

As a private nonprofit funded by the state, **NCBiotech** serves as the coordinator for these many resources to grow North Carolina's life science community. As part of their expansion plans, AveXis, bluebird and Pfizer all worked with NCBiotech to create solutions for their training and workforce development needs. Emerging companies located in the state can take advantage of company funding programs, designed to leverage investments in a company's growth phase. NCBiotech also coordinates the **North Carolina Precision Health Collaborative**. This partnership of researchers, companies, investors, payers, medical providers, and policy makers is forging common ground and a path forward for the many technologies and players who will contribute to the success of personalized health care.

North Carolina's biomanufacturing prowess grew from a value proposition: low business costs and skilled talent. We continually evolve our training programs to anticipate industry needs. Today, we are leading development and production of the first wave of gene and cell therapies. Ask NCBiotech how to make our success yours.

North Carolina Biotechnology Center

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