



Clinical manufacturing facility at Nationwide Children's Hospital in Columbus, Ohio.

Ohio

Home to a Thriving Gene Therapy Ecosystem

In the past two decades, Ohio has developed a rare environment that supports, encourages and drives cutting-edge gene therapy research and commercialization. Today, that ecosystem, anchored by Nationwide Children's Hospital and The Ohio State University, is among the nation's leaders in generating startups, attracting investment and generating global industry attention.

World-class researchers, high-tech facilities and a growing investor base have created centers of excellence in Columbus, as well as in Cleveland's well-known healthcare sector and Cincinnati's entrepreneurial community. Fueled by a talent pipeline and research initiatives at nearby universities such as The Ohio State University, University of Cincinnati and Case Western Reserve University, these healthcare hubs are performing valuable research and generating companies with a common goal: to develop treatments that serve patients.

Established as an affiliate company of Nationwide Children's Hospital and its Research Institute, Andelyn Biosciences is a product of Nationwide's success in clinical manufacturing and gene therapy.

Andelyn Biosciences will become a Phase 3 compliant Good Manufacturing Practices (cGMP) clinical manufacturing facility producing materials for phase 1, 2, and 3 gene therapy clinical studies for biotechnology and pharmaceutical industries around the world.

It will be central Ohio's first commercial-scale Good Manufacturing Practices (cGMP) clinical manufacturing facility devoted to gene therapies.



The Power of a Working Ecosystem

Ohio's gene therapy ecosystem depends upon world-class researchers, visionary entrepreneurs and knowledgeable investors. The results: startups, progress toward product commercialization and, ultimately, improved patient care.



Formed in 2017 based on research at Nationwide Children's Hospital, clinical-stage gene therapy focused on Limb-Girdle Muscular Dystrophies (LGMDs).



Acquired Myonex for \$165 million. Sarepta plans to build the Gene Therapy Center of Excellence in Columbus.

"We're making a very significant commitment and investment in Columbus. We like to believe with the work that Nationwide Children's Hospital has already done, coupled with our commitment, there is a real chance Columbus, Ohio, could become the most important place in the world for gene therapy development."

Doug Ingram, president and CEO, Sarepta



Founded in 2012 through a discovery by scientists at Nationwide Children's Hospital, Milo is clinical-stage gene therapies focused on increasing muscle strength and improving the lives of patients with muscular dystrophy.

"There's a rare combination of resources in Ohio that make it a really attractive destination for gene therapy companies. Entrepreneurs and young biotechs can tap into clinical expertise at The Research Institute at Nationwide Children's Hospital, and their resources like the GMP facility and in-house regulatory group. In addition, forming a company in Ohio, where there is more risk tolerance and great early stage funding partners, gives you a chance to excel as an entrepreneur much quicker."

Al Hawkins, founder and CEO, Milo Biotechnology



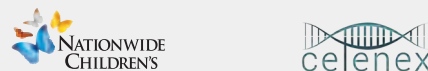
Technology, invented by researchers at Nationwide Children's Hospital, led to the development of AveXis, a clinical-stage gene therapy company focused on Spinal Muscular Atrophy (SMA). *(exited to Chicago)*



Purchased AveXis in 2018 for \$8.7 billion.

"In neurological disease, it is rare to go from gene defect to therapy so directly, and the fact that this has happened here in one place is perhaps even rarer."

John Kissel, M.D., chair of Neurology at Ohio State Wexner Medical Center and director of the SMA Clinic at Nationwide Children's



Started at Nationwide Children's Hospital, Celenex develops treatment for rare, fatal neurological conditions.



Acquired Celenex in September 2018 for \$100 million upfront and up to \$350 million in milestone payments.

"The groundbreaking work of Drs. Brian Kaspar and Kathrin Meyer at Nationwide Children's Hospital, along with collaborator, Arthur Burghes, Ph.D., professor at The Ohio State University, on these programs has led to remarkably strong and consistent pre-clinical results and now, in CLN6 Batten disease, encouraging early results in children. This is science and biotechnology at its best."

John F. Crowley, chairman and chief executive officer, Amicus Therapeutics



Formed in early 2013 based on technology developed at Nationwide Children's Hospital, Abeona focuses on gene and cell therapy for rare diseases such as Epidermolysis Bullosa, Sanfilippo Syndrome and Batten Disease.

Ohio's first fully functional clinical and commercial manufacturing facility for producing Abeona's cell and gene therapies.



New biopharmaceutical company focused on developing innovative gene therapies for hematological conditions.



Roivant and Cincinnati Children's Hospital partner to launch Aruvant Sciences.

"An important goal of our research enterprise is to facilitate the translation of promising discoveries from bench to bedside, and the closing of this deal represents a significant step in ensuring this program has the potential to impact patients here in Cincinnati and around the world."

Dr. Peggy Hostetter, chief medical officer of Cincinnati Children's and director of the Cincinnati Children's Research Foundation

To learn about the partnerships and resources available in Ohio, visit JobsOhio.com or reach out to our team:



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