

A gene therapy pioneer: the Jude Samulski story

"I won't be able to pay you, because I'll probably never be able to raise money, and people think this is sort of ludicrous. But if we're successful, we'll change the world."

With those words, a scientist named Jude Samulski planted the seed for what has become one of the world's most successful gene therapy companies that is, in fact, helping to change the practice of medicine.

Jude co-founded AskBio in 2001 with life science attorney Sheila Mikhail and Xiao Xiao, PhD, another pioneer and world-leading scientist in the exciting field of gene therapy. The foundational discoveries and advancements of Dr. Samulski and other longtime associates laid the groundwork for gene therapy today. His vision has led to the development of advanced gene therapy and AAV technology that has helped create a future in which treating genetic disease is possible.

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– R. Jude Samulski, PhD, 2001

AAV gene therapy comes to life

After earning a Bachelor of Science degree in microbiology from Clemson University, Jude began his doctoral studies in molecular biology in 1978 at the University of Florida in Gainesville. The potential for how a virus could be used to safely deliver corrected genes into the body captured his attention and over the next four years, it was the focus for his doctoral studies. His discovery of how to clone the adeno-associated virus (AAV) was a feat that had never been achieved, and his research was highlighted in his dissertation published in 1982. This breakthrough set the course for gene therapy and has contributed to approved and investigational therapies for the treatment of previously untreatable diseases.

Possibilities emerge

AskBio is founded

Revolutionizing genetic medicine was in his genes

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Jude's curious nature and scientific expertise was only accelerated as he completed his postdoctoral research fellowships at the State University of New York at Stonybrook and Princeton University and when he joined the faculty at the University of Pittsburgh in the Department of Biological Sciences. In 1993, he was recruited by the University of North Carolina at Chapel Hill as director of the School of Medicine's Gene Therapy Center. During the next 25 years, Jude spearheaded countless groundbreaking studies and scientific discoveries to advance gene therapy technology.

He became a full professor at UNC and continued as the director of the Gene Therapy Center until 2016. Along with others under his tutelage, his work in AAV development processes, therapeutic design and genetic technology applications evolved into a vast compilation of intellectual property and accomplishments now vital to the industry and patients with a high unmet medical need.

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When Jude and Sheila founded AskBio with Dr. Xiao in 2001, they achieved tremendous successes but also faced a number of challenges. At the time, gene therapy was still considered an unreachable feat and an unworthy investment by many in the financial community. But even working without compensation for more than a decade, Jude, Sheila and Xiao were never deterred, and their belief in the power of gene therapy was unwavering. The look in the eyes of children, adults and their families who were desperately searching for answers and clinging to hope was all the motivation they needed.

Jude's vision was always much bigger than any one disease and early on, he saw the significant access issues that remain today. Over 40 years of his tireless work to create effective gene therapeutics was also focused on how to overcome clinical process and manufacturing obstacles.

"If I could find a way to give away gene therapy to every patient in need across the world to correct a genetic defect they did not ask for, I would. But, even if we had an FDA-approved therapy for every genetic disease, we can't make enough genetic medicine to serve all those in need. That has to change," Jude once remarked.

The pathway for AskBio to secure funding in the early years was littered with roadblocks. Raising sufficient capital to follow Jude's vision prompted him and his colleagues to pursue creative fundraising strategies such as bake sales held by families whose children suffered from muscular dystrophy and believed in AskBio's potential.

In 2004, the Muscular Dystrophy Association (MDA) agreed to invest \$1.6 million in Jude's vision and AskBio. The investment was the largest and first ever the group had made to a commercial entity and was the catalyst for early Duchenne muscular dystrophy (DMD) research through a Pfizer/AskBio divestiture and license agreement. The MDA investment and limited angel funding kept the vision alive, and as the first decade of the 2000's came to a close, the world was beginning to wake up to the real possibilities of gene therapy.

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From 2010 through 2018, Jude and his team at AskBio made momentous advances in gene therapy. If it was not for his early determination and unwavering belief in genetic medicine over the years, it is possible genetic medicine would not be as far along as it is today.

In 2010, one of the little-known yet most prolific discoveries and advancements in gene therapy came to fruition. Jude and Josh Grieger, PhD former research assistant who served as AskBio's chief technology officer, brought to life Pro10™, a cell line for generating AAV therapeutics. Pro10 was first to use human embryonic kidney (HEK) cells in serum-free suspension media to develop and produce AAV vectors at scales and yields currently among the highest in the industry. At the time, few in the field of AAV gene therapy were focused on the importance of manufacturing.

Along with his partners, Jude also created two AskBio subsidiaries – Bamboo Therapeutics, which focused on Duchenne muscular dystrophy, and Chatham Therapeutics, which focused on genetic solutions for hemophilia. Years of genetic research were validated when two of the largest pharma and bioscience

companies in the world came calling. Pfizer acquired Bamboo and Baxter (Takeda) acquired Chatham. These essential transactions created the capital needed to advance an end-to-end gene therapy platform that no one else in the industry could foresee.

Now armed with Pro10, unrivaled knowledge for AAV therapeutic design, unique development processes, and a new source of self-generated funding, Jude and AskBio were ready to write the next chapter of a story only beginning to unfold. AskBio's pipeline of therapeutics, innovative capsid design, self-complementary AAV technology and clinical processes were sowing the seeds for a dynamic organization on the forefront of AAV therapeutics and technology. Gene therapy and biopharmaceutical companies, both large and small, along with banks and venture capital firms around the globe now recognized AskBio's undeniable leadership and value to the future of AAV therapeutics.

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2019 was a game-changing year for Jude and AskBio. In April, two investment firms [TPG Capital](#) and [Vida Ventures](#), invested \$225 million in the company, and AskBio's founders contributed an additional \$10 million. This investment provided the capital necessary to accelerate its vast portfolio of technology, promising clinical research, high-yield manufacturing and groundbreaking research collaborations.

AskBio formed strategic partnerships with leading biotechnology players including Selecta Biosciences, Editas Medicine and others to help solve challenges such as the avoidance of neutralizing antibodies to enable redosing and *in vivo* delivery of genetic medicine. Acquisitions included the nanotechnology assets from Rovermed BioSciences and gene control technology from Synpromics, Ltd. The Synpromics office in Edinburgh, Scotland is now home to AskBio's European operations.

Also in 2019, the second AAV gene therapy gained FDA approval – neither of which would be possible without Jude's groundbreaking research. Biotechnology companies and universities around the globe are expanding their gene therapy research portfolios based on many of Jude's discoveries and advancements. He sees everyone in the industry playing important roles in a world where any genetic disease can be treated or cured.

The vision, scientific leadership and history-making advances by one of gene therapy's most prolific figures, R. Jude Samulski, PhD, helped drive the future of AskBio and its prominent place in gene therapy. The advanced manufacturing capabilities, synthetic promoter and DNA technology, unique library of chimeric capsids, robust IP portfolio and promising gene therapeutics are a powerful testament to Jude's incredible body of work.



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