



PepGen raises \$45M Series A

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RA Capital Management leads \$45M Series A financing for PepGen's next-generation oligonucleotide platform targeting Duchenne muscular dystrophy and other rare neuromuscular and cardiac diseases.

PepGen's proprietary cell-penetrating peptide technology promises enhanced safety, efficacy, and improved delivery to cardiac muscle; Oxford Sciences Innovation, The University of Oxford, and CureDuchenne Ventures also participated in the Series A round.

BOSTON, USA, and OXFORD, UK (9th December, 2020) – PepGen, a therapeutics company targeting severe neuromuscular diseases, including Duchenne muscular dystrophy (DMD), has closed a \$45 million Series A funding round led by RA Capital Management with participation from Oxford Sciences Innovation (OSI), the company's original seed investor. The University of Oxford and CureDuchenne, a leading patient advocacy group, also participated in the round.

PepGen will use the funding to advance into the clinic their next-generation cell-penetrating peptides conjugated to phosphorodiamidate morpholino oligomers (PPMOs) that are designed to correct genetic defects in diseases with high unmet medical need. PepGen's proprietary PPMO technology will dramatically enhance delivery of oligonucleotides to key tissues, while also improving safety compared with competing therapies.

"PepGen's cell-penetrating peptide (CPP) technology represents an important step change in that we have succeeded in optimizing cellular uptake whilst simultaneously engineering out the safety signals that historically impacted earlier attempts at this approach," said PepGen co-founder Matthew Wood, professor of neuroscience at the University of Oxford. "I am delighted to see our progress validated by support from top-tier investors, and look forward to seeing translation into the clinic."

One of PepGen's lead indications, DMD is a devastating genetic neuromuscular disorder affecting one in 3,500-5,000 male births around the world.

"Patients born with Duchenne muscular dystrophy lack dystrophin, a critical protein that confers structural integrity on muscle," said Caroline Godfrey, PhD, CEO and co-founder of PepGen. "PepGen is developing unique, proprietary cell-penetrating peptides that we expect will improve upon the efficacy of existing exon-skipping approaches to restore dystrophin expression in DMD patients."

A key benefit of PepGen's platform is its unique potential to reach all the tissues affected by DMD and other inherited neuromuscular and cardiovascular conditions. Notably, unlike other approaches, PepGen's drug candidates strongly distribute to cardiac tissue. Heart disease is a key cause of morbidity and mortality in these devastating conditions.

"A unique distinction of the PepGen peptides is that they can penetrate cardiac tissue, addressing a major and growing problem DMD patients face as they get older," said Ramin Farzaneh-Far, MD, venture partner at RA Capital Management, PepGen's executive chair, and a board-certified cardiologist. "With the recent approvals of treatments that generate small increases in dystrophin in skeletal muscle, patients may be ambulating and living longer, but this in turn is expected to shift the burden of morbidity and mortality towards an epidemic of heart disease, which is not adequately addressed by current DMD therapies."

Said Debra Miller, CEO of CureDuchenne Ventures, "PepGen, we believe, represents a potential breakthrough approach for which DMD patients and their caregivers have been hoping against hope. Virtually all Duchenne patients have cardiomyopathy, and cardiac failure is prevalent amongst this population, so therapies addressing this unmet need promise a major impact for the Duchenne community."

In addition to DMD, PepGen has a pipeline of drugs targeting rare neuromuscular and neurologic diseases, with a particular focus on those with severe cardiac manifestations as well as primary cardiac conditions.

"We consider ourselves lucky to be a part of this unique story. The ability of this technology to deliver life-saving drugs to all the organs and tissues affected by these devastating diseases offers real hope for total treatment of these conditions," said Joshua Resnick, MD, managing director at RA Capital Management and member of the PepGen board of directors.

PepGen's cell-penetrating peptide technology for oligonucleotide delivery was developed over more than a decade of research in the UK, and arose from collaborations between the neuroscience laboratory of Professor Wood at the University of Oxford and the peptide chemistry laboratory of Dr. Mike Gait at the MRC Laboratory of Molecular Biology in Cambridge.

With a seed investment of £4.5 million from OSI, PepGen was spun out in 2018 to commercialize the cell-penetrating peptide platform. The company was additionally supported by a prestigious £1.6 million grant from Innovate UK, the UK's innovation agency.

"Delivery is a challenge many innovative therapeutic platforms face; we're proud of the team's achievements to date as PepGen's CPP technology opens up a broad set of opportunities in gene therapy and gene modulation," said Uciane Scarlett, PhD, OSI's investment principal. Christopher Ashton, PhD, advisor to OSI, will continue to serve on PepGen's board of directors.

PepGen's new corporate headquarters will be in Boston, and its research hub will continue in the UK.

About RA Capital Management

RA Capital Management is a multi-stage investment manager dedicated to evidence-based investing in public and private healthcare and life science companies that are developing drugs, medical devices, and diagnostics. The flexibility of its strategy allows RA Capital Management to provide seed funding to startups and to lead private, IPO, and follow-on financings for its portfolio companies, allowing management teams to drive value creation from inception through commercialization.

At RA Capital's core is its TechAtlas research division, a scientifically trained team that maps out competitive landscapes to put data into context, identify breakthroughs, and originate conviction in new ideas. TechAtlas provides market intelligence, technical diligence, and other resources to both our internal Investment Team and our portfolio companies. The team's understanding of industry best practices is derived from an extensive collection of case studies documenting the impact of clinical trial design, partnership structures, and public market dynamics.

About Oxford Sciences Innovation

Oxford Sciences Innovation (OSI) is a leading science and technology business. OSI ensures Oxford's world-leading science moves out of the laboratory and onto the global stage. In partnership with the University of Oxford, OSI creates fundamental technology companies, built on science. We match scientists with experienced entrepreneurs and patient capital to turn idea to impact, discovery to company. OSI invests in Life Sciences, Deep Tech, Healthtech, AI and Software to create companies taking on challenges like diagnosis and treatment of disease and cancer, hyper resolution microscopy, renewable energy, drones, nuclear fusion and quantum computing. Founded in 2015, we've raised over \$800M of evergreen capital, building on Oxford's renowned research legacy, to create a leading science and technology ecosystem and home for entrepreneurs.

OSI backs companies from their inception and invest for the long-term, helping them to build their businesses by finding senior management talent, entrepreneurs, expert advisors and global investors to realise their vision. OSI reinvests any returns back into the Oxford ecosystem and the next generation of scientists and technologies to create even more companies capable of tackling more of the world's most important problems.

About the University of Oxford

Oxford University has been placed number 1 in the Times Higher Education World University Rankings for the fifth year running, and at the heart of this success is our ground-breaking research and innovation. Oxford is world-famous for research excellence and home to some of the most talented people from across the globe. Our work helps the lives of millions, solving real-world problems through a huge network of partnerships and collaborations. The breadth and interdisciplinary nature of our research sparks imaginative and inventive insights and solutions. Through its research commercialization arm, Oxford University Innovation, Oxford is the leading university patent filer in the UK and is ranked first in the UK for university spinouts, having created more than 200 new companies since 1988. Over a third of these companies have been created in the past three years.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, lethal, inherited neuromuscular disease that occurs in approximately one in every 3,500-5,000 male births globally. It is caused by a mutation in the gene that encodes instructions for dystrophin, a key structural protein. Symptoms of DMD usually appear in infants and toddlers. Affected children may experience developmental delays such as difficulty in walking, climbing stairs or standing from a sitting position. As DMD progresses, muscle weakness in the lower extremities spreads to the arms, neck and other areas. Most patients require full-time use of a wheelchair in their early teens, and then progressively lose the ability to independently perform activities of daily living. Eventually, patients have difficulty in breathing due to respiratory muscle dysfunction, which ultimately may require ventilatory support. Cardiac muscle dysfunction frequently leads to heart failure and death. The condition is universally fatal, and patients usually succumb to the disease in their twenties.