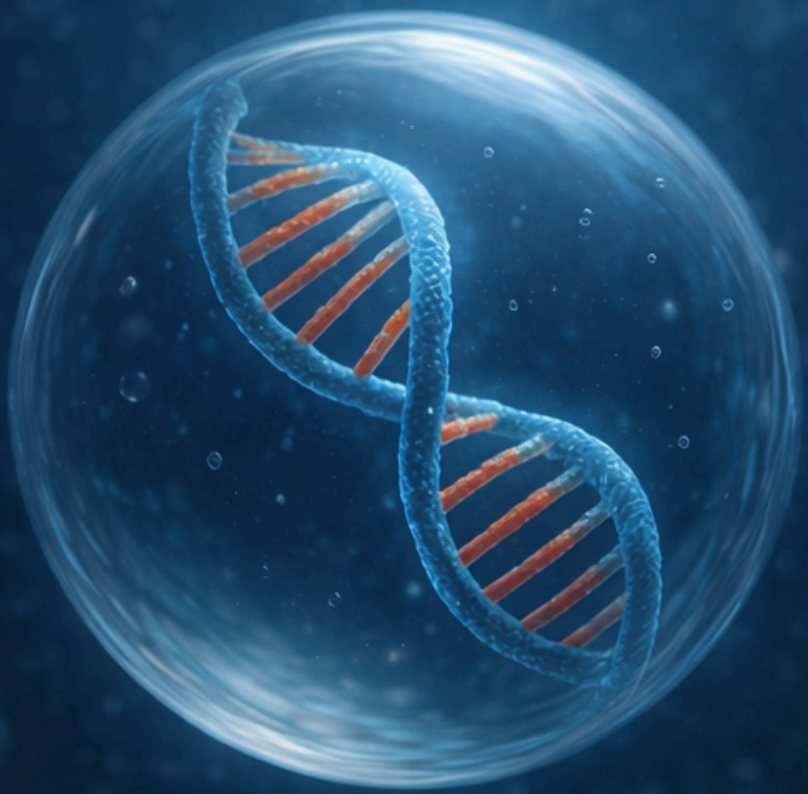
VISION & IMPACT BROCHURE

ExoVectory

Natural Nanocarriers for Delivery of Genetic Medicine



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THE FOUNDING STORY

“Reach the unreachable, Treat the untreatable.”

ExoVectory was founded by scientist Dr. Ir. Jeroen de Vrij, driven by his strong scientific interest in cell and gene therapy and his belief that these technologies can revolutionise treatment for patients. Jeroen’s passion for the field began in childhood, inspired by the television story Fantastic Voyage, about a shrunk-down crew travelling through the human bloodstream to save a life. As a teenager he was already writing about DNA, viral vectors, and gene therapies. While much of the world began to view gene therapy as too dangerous after several setbacks, including children developing leukemia from retroviral vectors, Jeroen kept following the field closely. The field recovered, first through lentiviral vectors, then through AAVs, each generation bringing gene therapy closer to its potential. But one problem persisted: every delivery vehicle was either too small or carried a toxicity that made it too dangerous to use.

Jeroen spent years in research, drawn particularly to the brain and to brain cancer, until one day he found himself standing in an operating room watching a viral vector being infused directly into a patient's brain. He held that patient's hand before and during the procedure, an experience he describes as the most unforgettable of his career and the moment the distance between the laboratory and the human being collapsed entirely. He left that operating room knowing that building the right delivery vehicle was not simply a scientific challenge. It was a moral obligation.

When his mother was diagnosed with multiple myeloma, there was only one drug available. It prolonged her life but could not save it. She died before T-cell therapy became available. Jeroen has never stopped thinking about the patients who run out of time while waiting for the right treatment to come, and his mission became to close that gap, by making precision medicine faster, better, and available to every patient who needs it, working not in isolation but in partnership with companies and, above all, with the hospitals where patients actually are.

Gene therapy now has extraordinary tools, including CRISPR, precision targeting, and a deepening understanding of the genome. What it has lacked is the right vehicle to carry those tools across the barriers that separate the laboratory from the cell that needs repairing. Exosomes are that vehicle, and Jeroen has spent his career arriving at exactly this point. He is building it now, not for the market, but for the patients, including the ones, like his mother, who could not wait.

THE PROBLEM

Cancer kills ten million people a year.
For some forms, there is still no answer.

Cancer is not one disease but hundreds, each caused by cells that begin growing uncontrollably, invading the surrounding tissue and spreading through the body. Nearly ten million people die every year because of cancer, and while medicine has made real progress with surgery, chemotherapy, and radiation, all of these approaches share the same fundamental limitation: they cannot reliably tell the difference between a cancer cell and a healthy one, and when cancer comes back, the options left for patients shrink dramatically. T-cell therapy works differently: instead of attacking the whole body to reach the cancer, it takes the body's own immune cells, teaches them to recognise the tumour, and sends them to destroy it, with results in certain cancers that have genuinely changed what medicine thought was possible. The challenge that remains is getting those engineered cells to where they are needed most, particularly inside the brain, where almost every treatment ever developed is blocked before it can reach its target.

10 million

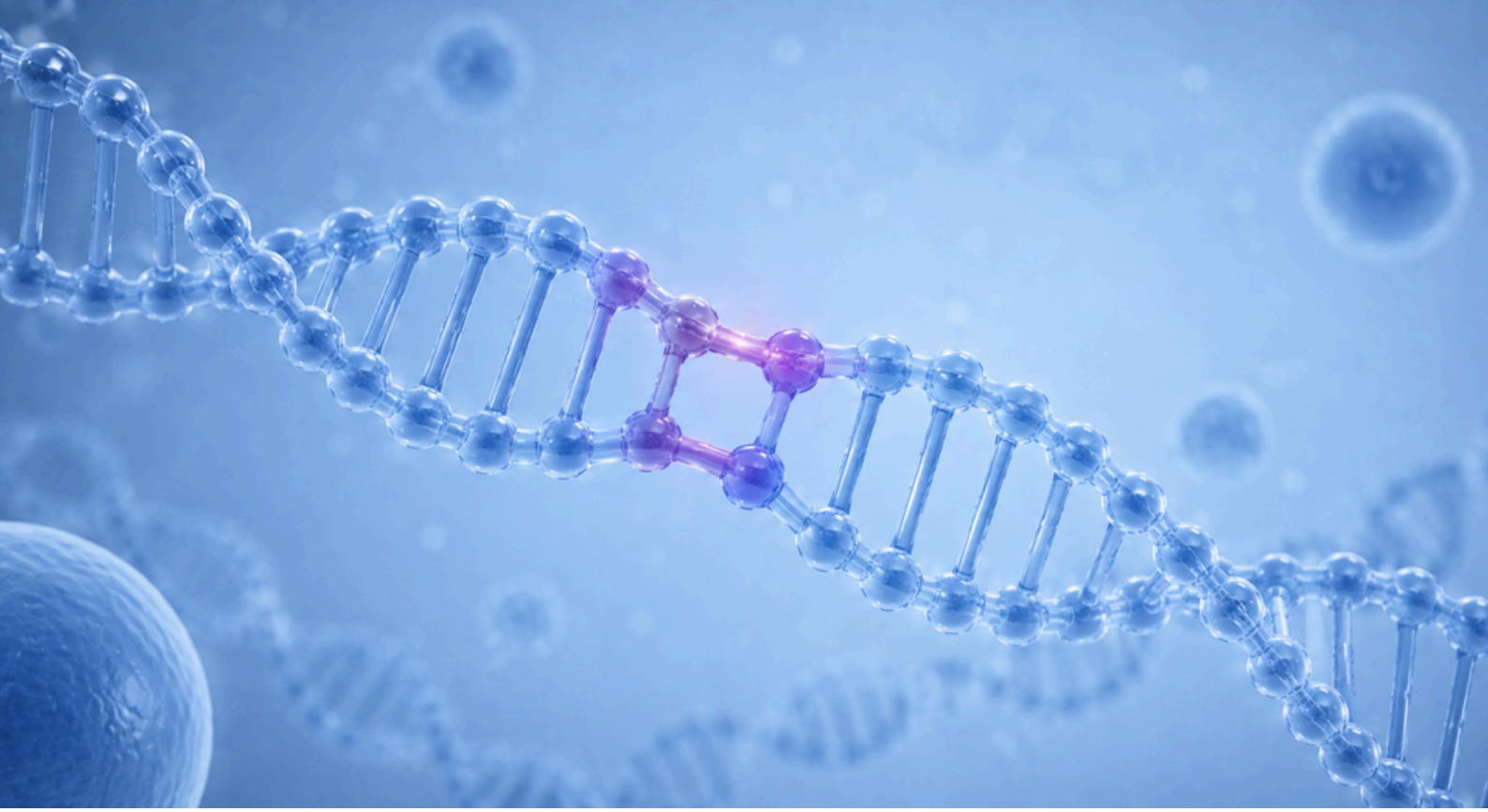
people die from cancer every year — one every three seconds.

5-7%

the five-year survival rate for glioblastoma (brain cancer), despite decades of research.

1 in 5

people will be diagnosed with cancer at some point in their lifetime.



THE PROBLEM

Genetic diseases are not rare.
The treatments are.

There are over 10,000 known genetic diseases, affecting an estimated 300 million people worldwide. The majority manifest in childhood, and many follow a progressive course, meaning that without effective treatment, patients experience a gradual deterioration in function over time. Despite decades of research and significant investment, approved therapies exist for fewer than 5% of these conditions. The gap between the number of diseases identified and the number for which medicine can offer a meaningful treatment remains one of the most significant challenges in modern healthcare.

For diseases affecting the heart and central nervous system, the obstacles have been particularly difficult to overcome. The heart has limited capacity for self-repair, and inherited mutations affecting cardiac muscle tend to cause irreversible structural changes over time. The brain, meanwhile, is protected by the blood-brain barrier, a highly selective biological membrane that blocks the vast majority of therapeutic molecules from reaching their target. These biological barriers have historically left patients with treatments that only slow the disease down. What they actually need is something that can get through, reach the problem, and fix it.

> 300 million

people worldwide are currently living with a genetic disease.

95%

of all known genetic diseases have no approved treatment of any kind.

1 in 3

children born with a genetic disease will not survive past the age of five.

BLOOD CANCER OF THE BRAIN • PCNSL

When the immune system turns on the brain, medicine struggles to follow.

Primary CNS lymphoma (PCNSL)

Primary CNS lymphoma is unlike most brain cancers. It does not grow from brain tissue itself, but begins in the immune system, as white blood cells called lymphocytes start multiplying uncontrollably and invade the brain, spinal cord, and eyes. It does not announce itself with a visible mass or obvious pain, but with subtle changes: a personality shift, a memory lapse, a moment of confusion that a loved one notices before the patient does. By the time it is found, it has almost always been growing for months, quietly rewriting the person from within.

From chemotherapy to T-cell therapy

Today, PCNSL is treated primarily with high-dose chemotherapy, a blunt instrument that cannot distinguish the cancer from the healthy brain tissue surrounding it, and that causes permanent cognitive damage in nearly half of all patients. Even when it works, half of those who respond will relapse within two years. Radiation, the other available option, carries the same risk of irreversible neurological harm. Because PCNSL originates in the immune system rather than the brain itself, it is uniquely suited to a different approach entirely: T-cell therapy, which engineers the body's own immune cells to recognise and destroy the lymphoma directly. The biology of this disease points toward exactly this treatment. The only thing that has prevented it from reaching patients is delivery — getting the engineered cells across the blood-brain barrier and to the tumour precisely and safely.

15-20%

of all PCNSL patients achieve long-term survival. For patients over 60, outcomes are significantly worse.

PATIENT STORY

For many PCNSL patients, the disease begins without warning with a gradual loss of vision, a moment of confusion, a symptom that is easy to dismiss as something minor. By the time a diagnosis arrives, the person who first noticed something was wrong is often unable to walk and struggling to understand what is happening to them. Treatment is long and demanding: rounds of chemotherapy, a stem cell transplant, and months spent in isolation while the body slowly rebuilds itself. Some patients come through it and recover fully, but many do not, and all of them deserve something better than what medicine currently has to offer.

Read a personal account of living with PCNSL → https://blog.dana-farber.org/insight/2021/05/stem-cell-transplant-sets-patient-with-rare-lymphoma-on-a-cancer-free-path/

GENETIC CNS DISEASES • BRAIN CANCER

Genetic CNS diseases and brain cancer share one brutal truth: medicine can't yet reach them.

Genetic CNS disorders

Approximately 40% of all known genetic disorders affect the central nervous system. What makes genetic CNS disorders uniquely cruel is the combination of what they attack and what medicine can offer in response. Huntington's disease causes nerve cells in parts of the brain to gradually break down and die, producing uncontrollable movements, cognitive deterioration, and personality changes. There is no treatment that can stop or reverse it. For ALS, Huntington's, Parkinson and many other neurological diseases, there is neither a cure nor a preventative treatment available — only symptomatic management. The genetic error is known, the damage it causes is understood, and yet medicine cannot reach inside the brain to correct it.

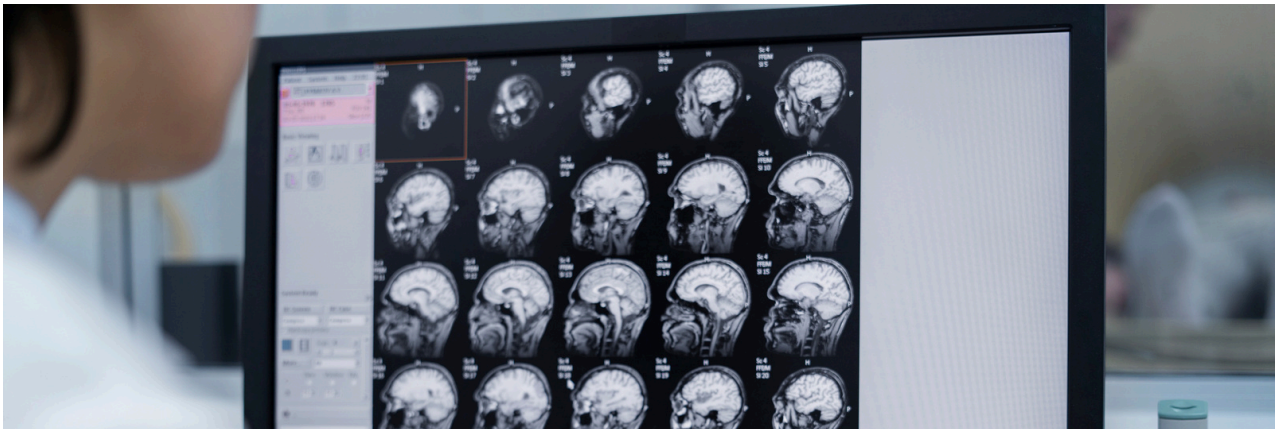
Glioblastoma

Glioblastoma is the most aggressive form of glioma, brain cancer originating in glial cells. Unlike PCNSL, which originates in the immune system, glioblastoma is a cancer born from the brain's own tissue, and that distinction makes it one of the hardest tumours in existence to treat. It does not form a clean, removable mass, but infiltrates surrounding tissue, spreading along neural pathways no surgeon can safely reach, and it recurs in almost every case. With surgery, radiation, and chemotherapy combined, median survival is 15 months — a figure that has not changed in thirty years. When the cancer returns, there is no standard second-line treatment and no targeted approach capable of reaching it precisely enough to matter.

PATIENT STORY

Glioblastoma does not give families time to prepare. The disease announces itself as a crisis: a seizure in the night, a scan that reveals a tumour that was not there months before, and a diagnosis that offers little hope. The most aggressive subtype of glioma moves faster than any available treatment can follow, and within a year or two of the first symptoms the patient is most often gone, leaving behind children still in school and a youngest sibling who will grow up with photographs instead of memories.

Read a personal account of a family living with glioblastoma → https://braintumor.org/news/a-teenagers-story-of-resilience-after-fathers-glioblastoma-diagnosis/



GENETIC CARDIAC DISEASES

The most common genetic heart disease affects 1 in 500 people. There is still no cure.

Genetic cardiac diseases are inherited conditions written into a person's DNA from birth. The heart muscle grows too thick, or too weak, or fails to repair itself. For decades, patients may have no idea anything is wrong. Then they collapse on a sports field, or lose a parent to sudden cardiac arrest at 40, or watch their child stop walking by age twelve.

Hypertrophic cardiomyopathy (HCM)

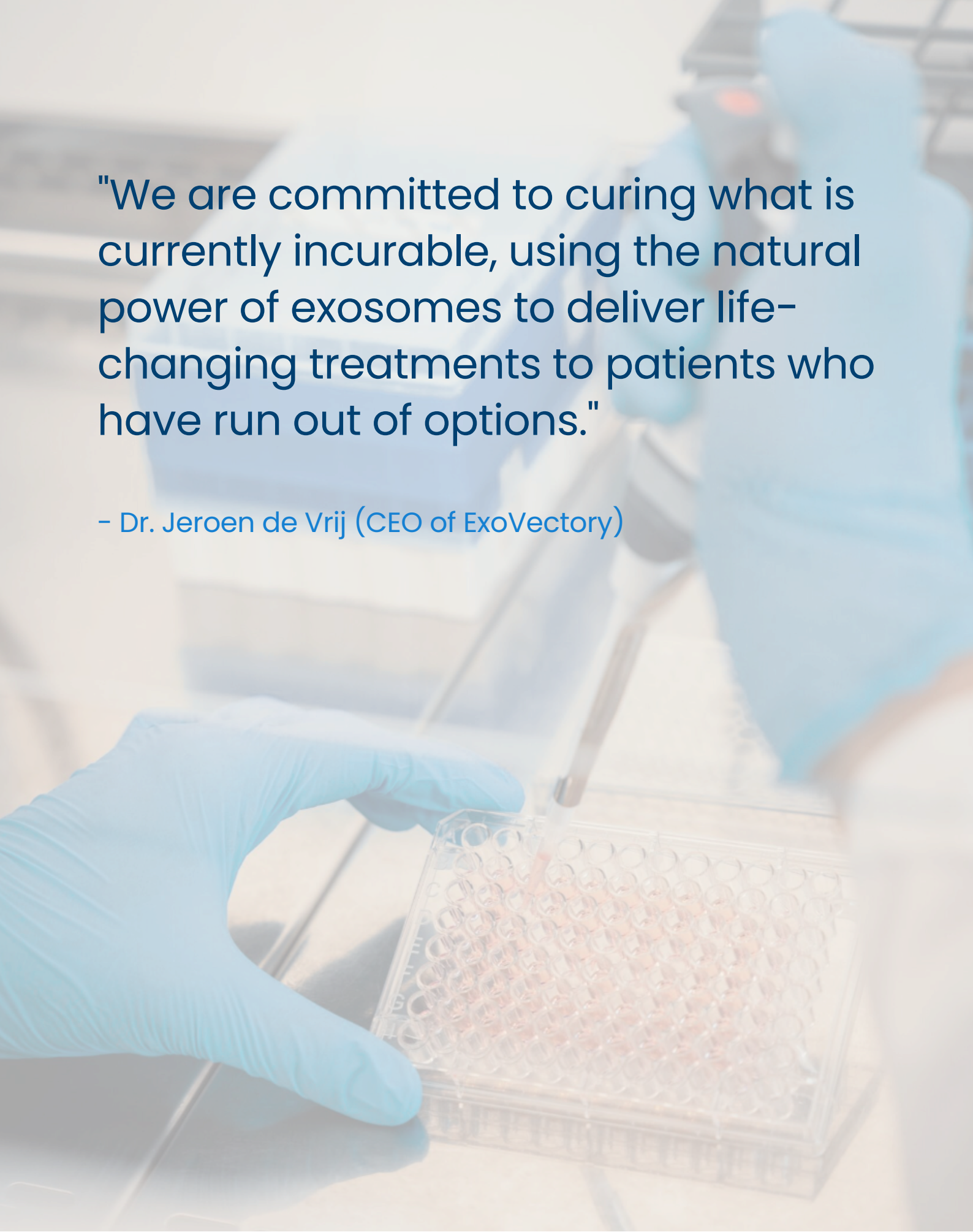
HCM is the most common genetic heart condition and the leading cause of sudden cardiac death in young athletes. It causes the heart muscle to become thicker and stiff, making it harder for the heart to pump properly. Most patients live their whole lives undiagnosed, until their heart stops without warning. Current treatments manage the symptoms but do not correct the underlying genetic defect.

Phospholamban cardiomyopathy (PLN)

Phospholamban cardiomyopathy is a rare inherited condition caused by a mutation in a gene that controls how the heart muscle contracts. The mutation disrupts calcium movement in heart muscle cells, causing the heart to gradually lose its ability to pump properly, leading to dangerous irregular rhythms and progressive heart failure. There is no established treatment other than standard heart failure therapy or heart transplantation.

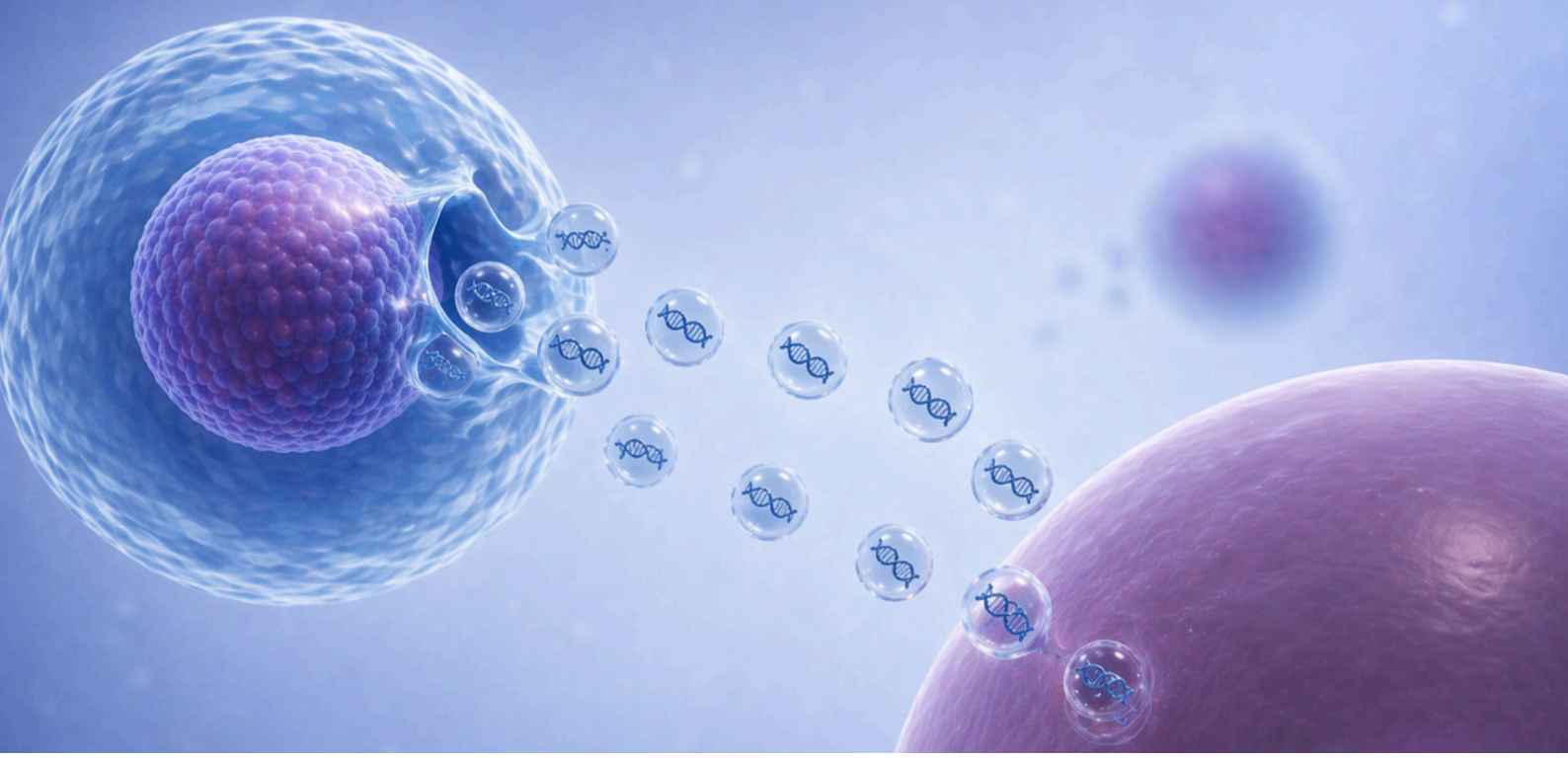
Duchenne muscular dystrophy (DMD)

Duchenne is primarily known as a muscle-wasting disease, but its most lethal dimension is cardiac. Because the heart muscle lacks dystrophin entirely, all boys born with Duchenne develop cardiomyopathy, with cardiac failure being the leading cause of death in these patients. Current interventions have extended life expectancy into the twenties, but Duchenne still remains a 100% fatal condition with no approved cardiac therapy.



"We are committed to curing what is currently incurable, using the natural power of exosomes to deliver life-changing treatments to patients who have run out of options."

- Dr. Jeroen de Vrij (CEO of ExoVectory)



OUR TECHNOLOGY

Exosomes: the body's own delivery system, now carrying treatments that couldn't get through.

What exosomes are

Exosomes are tiny particles that every cell in the body produces naturally. Their purpose is to carry biological instructions from one cell to another, coordinating repair and function across the entire body. Exosomes are generally well tolerated by the immune system and show strong potential to cross the blood-brain barrier, opening possibilities for reaching tissues that most existing delivery systems struggle to access.

Why existing approaches fall short

Gene therapy has been promising cures for decades. What has held it back is delivery. The two dominant systems used today — lipid nanoparticles and viral vectors — both carry certain limitations. Lipid nanoparticles are synthetic and can trigger immune responses. They also normally cannot reach the brain and carry limited genetic payloads. Viral vectors can provoke dangerous immune reactions and carry risks of unintended genetic effects.

EXOVECTORY'S EXOSOME PLATFORM

ExoVectory has developed a proprietary platform that loads exosomes with large therapeutic DNA, delivering complete genetic instructions to the precise cells that need them. The ability to carry large DNA sequences means that a single platform can address a broad range of genetic diseases, rather than requiring a separate mutation-specific approach for each condition. The platform is also versatile in how it is applied, supporting both ex vivo applications, where cells are engineered outside the body before reinfusion, and in vivo delivery, where the therapeutic payload is administered directly into the body and travels to its target. The platform combines large payload capacity, natural biocompatibility, and precise targeting in a single delivery system, making it uniquely suited to the diseases that current medicine has been unable to reach.

APPLICATIONS AND DEVELOPMENT

PCNSL, CNS disorders and cardiomyopathy - diseases where current medicine falls short.

ExoVectory is committed to treating the diseases that medicine has not yet been able to cure, giving patients a future that their diagnosis once made unthinkable. We are focused on two therapeutic approaches: T-cell therapy for cancer, and gene therapy for diseases of the brain and heart. Both are made possible by our exosome platform — the delivery system that gets treatment to places nothing else can reach. The diseases we have chosen (PCNSL, CNS disorders, and cardiomyopathy) all share the same problem: existing treatments cannot get to them. Ours has the potential to, and that is why we chose them.

EX VIVO

PCNSL

We take a patient's own immune cells, engineer them using exosomes to recognise and destroy the lymphoma, and send them back into the patient to kill the tumour inside the brain.

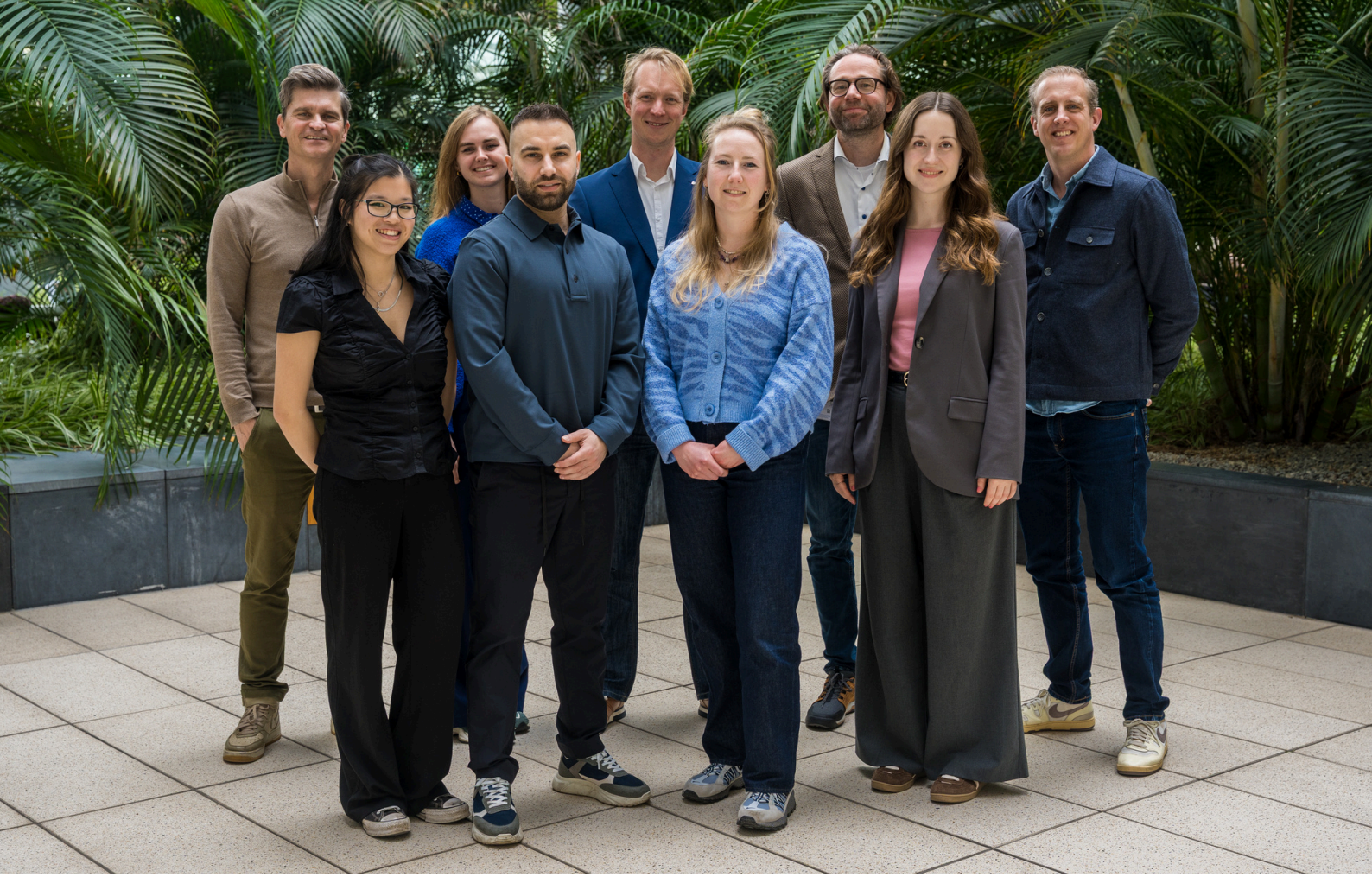
IN VIVO

CNS

Exosomes cross the blood-brain barrier naturally, allowing us to deliver therapeutic DNA directly to brain and tumour cells inside the brain, reaching targets that surgery and drugs cannot reach.

Heart

Using exosomes as carriers, we deliver gene sequences to the heart muscle to induce repair and regeneration of the heart tssue.



OUR TEAM

Building a new class of medicine requires more than good science. It requires the right people, the right advisors, and the right partners.

ExoVectory is built by passionate individuals who believe exosomes can change the way we treat diseases. Our team brings together specialists in exosome biology, gene therapy, production and business development, supported by a network of well-known scientific advisors and experienced biotech entrepreneurs.

Our advisors include Dr. Bram Bout, Dr. Audrey Sporrij, and Dr. Inez de Greef, who bring deep expertise in drug development, commercial strategy, and biotech entrepreneurship. On the scientific side, Prof. Mirjam Heemskerk and Dr. Koen Breyne contribute world-class knowledge in cellular immunotherapy, neuro-oncology, and exosome-based therapeutics. We are also proud to collaborate with and be supported by respected institutions and organisations including Erasmus MC, LUMC, and Harvard Medical School, as well as funding bodies NWO, Health Holland, and Eurostars, and industry partners Thermo Fisher, Getinge, and Support Casper.

Beyond the core team and advisors, ExoVectory works with a trusted network of partners across legal, patent, regulatory, financial, and strategic functions, including Axon Science Lawyers, VO Den Haag, 3D-PharmXchange, Kaaphoorn, and FFUND. Each partner has been chosen not just for their expertise, but for their commitment to the mission ExoVectory is pursuing.

Erasmus MC · Leids Universitair Medisch Centrum · Harvard Medical School · NWO · NL Health~Holland · eurostars · Support Casper · ThermoFisher Scientific · Getinge

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