

## Revolutionary Trojan Horse Type Platform that can Deliver Large Molecules across the Blood-Brain Barrier to Treat Severe Neurological Diseases



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“By generating a ‘Trojan horse’ out of our Trojan Horse antibody and the missing enzyme, we have created a next generation of ERT that treats both, the somatic disease burden and also crosses the BBB to address the cognitive disease burden. Why is this so important? As I said, now for the first time we are able to add years to the life, but also life to the years of our patients.”- Dr. Mathias Schmidt, Ph.D.

**CEOCFO: Dr. Schmidt, what is the focus for ArmaGen today?**

**Dr. Schmidt:** ArmaGen has developed a platform to bring biotherapeutics across the blood-brain barrier (BBB). Let me briefly tell you why this is important. Recombinant biotherapeutics, which were invented almost thirty years ago, have revolutionized the way we treat serious and life-threatening diseases. However, the last white spot in the landscape of human organs that is not addressed by biologics is the brain. The reason for that is that nature invented the BBB to prevent large molecules, including pathogens or toxic molecules, to cross the BBB and wreak havoc on the brain. What was a great invention by Mother Nature, is the most significant impediment towards exploiting biologics to address diseases with very high unmet medical needs like Alzheimer’s, Parkinson’s disease, multiple sclerosis, and other serious diseases affecting the brain.

**CEOCFO: What has ArmaGen developed?**

**Dr. Schmidt:** ArmaGen has developed a Trojan Horse platform that can bring large molecules across the BBB. We know that there are certain receptors at the BBB that can transcytose larger molecules into the brain that are needed for physiological functions. One of those molecules is insulin. We all know that insulin is exclusively made in the pancreas, but has an important physiological role in the brain. The way that this is solved is that this molecule is bound by the insulin receptor and transcytosed from the blood circulation into the brain. ArmaGen is exploiting this very mechanism by having developed an antibody against the insulin receptor. By fusing a payload with therapeutic effector function to the antibody, we generate a therapeutic molecule that can travel across the BBB very much like insulin. Like a Trojan Horse, this fusion protein then brings the therapeutic payload into the brain.

**CEOCFO: Does it ever worry you that you can trick the brain? How can you accomplish this? Why are we able to trick the body in different ways that are helpful?**

**Dr. Schmidt:** This platform and technology sounds intuitive. However, the entire pharmaceutical industry and researchers have struggled for decades to trick the human body and to trick the brain to bring large molecules across the BBB. Our Founder and Chief Scientific Officer, Dr. William (Bill) Pardridge, M.D., has basically dedicated his entire scientific career to come up with a platform solution that is applicable to almost any kind of central nervous system (CNS) disease. Importantly, it is also safe enough for human application and efficacious enough to bring a sufficient amount of the therapy across the BBB.

**CEOCFO: What are you working on today?**

**Dr. Schmidt:** Although the platform is broadly applicable, ArmaGen’s focus is in a disease area that is called Lysosomal Storage Diseases (LSD’s): To explain this very briefly, the lysosome is the trash can of the cell that degrades and recycles