Gene therapy likely best shot at curing brain diseases

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

The concept of gene therapy is straightforward, beautiful even: Fix an illness caused by a faulty gene by replacing or supplementing a healthy new copy of the gene. In other words, use genes as medicine. For example, one of the earliest human tests of gene therapy was in 1990, when two young girls with severe combined immunodeficiency (SCID) received repeated infusions of their own white blood cells modified to fix a defective gene. It worked—with continued treatments, both children showed signs of a restored immune system and went on to lead normal lives.

Microbiologist Guangping Gao first met a Canavan patient in 1993, at a party celebrating his discovery of the gene and mutations that caused the disorder. A family wheeled over their 6-year-old with Canavan disease to the young scientist, who realized that the boy had donated tissue to his research project. “I’d held his DNA in my hands. I had found what was wrong with his DNA,” recalls Gao, then a Ph.D. student. Yet he had no way to fix it.

Gene therapy is well-suited for treating inherited brain diseases. First, most drugs can’t get through the brain’s formidable blood-brain barrier, but something small, like a virus with a healthy gene tucked inside, can do it. Additionally, the brain is a closed compartment, so the risks of gene therapy are minimized—other parts of the body, the liver or the lungs, say, are undisturbed.

Read full, original post: Gene therapy might be the best, and perhaps only, chance at curing brain diseases