

[371] CRISPR: A NOVEL GENE THERAPY TO PROTECT THE HEART FROM ACUTE ISCHEMIC DAMAGE

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- **Investment Rational**
Ischemic Heart diseases (IHD) are the second most cause of death. CRISPR & STEM THERAPEUTICS LTD is developing a method for gene therapy for acute Ischemic diseases and IHD. We are developing a quick procedure that integrates stem cell and gene therapy of CRISPR-mediated gene-KO that should increase the protective effect of the implanted cells for treating acute ischemia.
- **Business Strategy**
This is the first time that such a "stem cell implant" is composed of a technological combination of gene therapy by CRISPR technology and tissue engineering for implantation in ischemic heart tissue. Cell-based therapies based on CAR T technology have been licensed as very-high-value added life-saving products and have laid the groundwork for the development and licensure of cell-based therapies.
- **Core Technology**
We are developing a market-disruptive cell therapy procedure based on ex-vivo CRISPR-mediated KO gene of fresh autologous SCs, which is significantly cardio-protective for ischemic heart from damage. Our innovative solution offers a very speedy protocol for ex-vivo treatment of fresh SCs that will be implanted within a few hours. CAR T will be the conceptual of the product.

Product Profile/Pipeline

Autologous "stem cell implant" based on CRISPR-modified Gene-KO SCs could be used for further cell therapy of ischemic diseases such as spinal cord injury, Ischemic stroke, Multiple Sclerosis and Amyotrophic Lateral Sclerosis, and Cerebral Palsy Patients.

- Partners: Pharmaceutical company
- Biotechnology company
- Medical device company – commercial
- Venture capital
- Incubator
- Contract Manufacturing Company

What's Next?

By 2021 Clinical trials will be conducted for one year during the phase two project and their evaluation will be performed at three different hospitals. After successful validation, we will work with a medical marketing company to commercialize the procedure project. The company projects will be entering the commercial market within four years after project completion.