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CRISPRi EPIGENOME MODIFICATION TO TREAT MUSCULOSKELETAL DISEASE

THERAPEUTICS

CRISPR-based gene therapy to treat inflammation due to degenerative disc disease, a major contributor to spinal disc tissue damage and debilitating back pain.

TECHNOLOGY TYPE

CRISPR-Based Gene Editing
Gene Therapy
Gene Silencing
Stem Cells

STAGE OF DEVELOPMENT

- Alteration of inflammatory response demonstrated in cell models.

- Ongoing animal studies.

IP PROTECTION

Nationalized PCT Pending in the United States and Europe

RNA-Guided Transcriptional Regulation and Methods of Using the Same for the Treatment of Back Pain
WO2016205688A3

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Roberta Hunt
Technology Manager
roberta.hunt@tvc.utah.edu
801-587-0519

TECHNOLOGY SUMMARY

Musculoskeletal diseases are a leading cause of disability worldwide. Current treatments for osteoarthritis and low back pain (LBP), however, are largely palliative and fail to prevent disease progression. Stem cell delivery treatment to the intervertebral disc in clinical trials may work, albeit on a short-term basis as cells succumb to inflammatory responses.

The proposed technology is an innovative CRISPR-based approach that temporarily silences specific pro-inflammatory genes to regenerate the disc to full functionality. This approach promotes cell survival, stem cell differentiation, and immunomodulation under inflammatory conditions. The epigenome editing vector package can be locally injected, or autologous cells can be modified and delivered to replace the lost disc tissue. Studies with dorsal root ganglion demonstrate inhibition of degenerative intervertebral disc neuron activity and preservation of non-pathologic activity.

FEATURES AND BENEFITS

- Provides long term protection against inflammation in musculoskeletal diseases.
- Slows the progression of disc degeneration
- Reduces follow-on surgeries and other treatments by stopping tissue damage.

RECENT PUBLICATIONS

Stover, J.D., Farhang, N., Berrett, K.C., Gertz, J., Lawrence, B., Bowles, R.D. (2017). CRISPR epigenome editing of AKAP150 in DRG neurons abolishes degenerative IVD-induced neuronal activity. *Molecular Therapy*. *25*(9): 2014-2027. doi: 10.1016/j.ymthe.2017.06.010.

Farhang, N., Brunger, J.M., Stover, J.D., Thakore, Gersbach, C.A., Setton, L.A., Bowles, R.D. (2017). CRISPR-Based epigenome editing of cytokine receptors for the promotion of cell survival. *Tissue Engineering 23*(15-16): 738-749. doi: 10.1089/ten.TEA.2016.0441.

INVENTOR PROFILE

Robert Bowles, Ph.D., Assistant Professor – Biomedical Engineering

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