Beth Israel Deaconess Medical Center

Harvard Medical School Teaching Affiliate

Therapeutic

Novel Gene Therapy Target for Cancer & Proliferative Retinopathies

BIDMC #1264

➢ *Response Gene to Complement 32 (RGC-32) induction as a treatment for angiogenesis-related cancers and retinopathies*

Background

Use of anti-VEGF agents for the treatment of certain cancers and for the management of neovascular age-related macular degeneration has been a major breakthrough in these fields. However, shortcomings of the current drugs, such as short half-life, limited effectiveness in some patients and potential systemic side effects, continue to drive the search for new drug targets.

Investigators at BIDMC have identified RGC-32 as a novel therapeutic target for treatment of neovascular-related disorders. Induced by hypoxia inducible factor-1 (HIF-1) and VEGF, RGC-32 was surprisingly found to possess anti-angiogenic properties which are mediated by downregulation of fibroblast growth factor 2 and cyclin E.

Stage of Development

➢In-vitro and In-vivo proof of concept demonstrated where RGC-32 overexpression by adenoviral transfection:

(1) reduced the proliferation & migration of cultured human endothelial cells;
(2) destabilized vascular structure formation *in vitro;* and
(3) reduced growth of blood vessels correlating with reduced tumor size in a xenograft rat tumor model & in Matrigel assays

Publication

An et al., *Circulation* (2009) 120: 617-627.

Commercialization

Market: Cancer/Retinopathy
Indication: Angiogenesis-related
- *Solid tumors such as breast cancer*
- *Diabetic retinopathy*
- *Macular Degeneration*

Product: Gene Therapy
Use: Therapeutic to overexpress RGC-32

Patent / Licensing Status

- US Patent pending
- Flexible licensing options available

Lead Inventor

Jian Li, MD, PhD

Competitive Advantage

✓ Proof-of-principle of the efficacy of gene therapy for the treatment of eye diseases has recently been demonstrated in animal models and humans.
Trends in Molecular Medicine (2008) 15: 23-31.

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