PNA-Based Gene Inhibitors (Yissum)
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A Novel Preparation Method of Cationic Building Blocks for Gene-Scilencing Molecules

Category	**Anti-gene and anti-sense drug development**
Development Stage	**Technology up-scale and enhancement**
Patent Status	**Patent Pending**
Market Size	**Market research firm Datamonitor (2005) has identified 99 companies developing DNA/RNA therapies, with 229 products in development.** **Biotech and pharmaceutical companies engaged in the R&D of anti-gene and anti-sense drugs include Isis Pharmaceuticals, Pfizer, Elan Corp., GeneVec, AVI Biopharma, Sanofi Aventis, GlaxoSmithKline, Targeted Genetics Corp., to name a few.**

Highlights

- **Cationic peptide nucleic acid (cPNA) conjugates were shown to penetrate cell membranes and blood brain barrier.**
- **These constructs may also serve as vehicles of drugs, allowing cell and BBB penetration.**
- **The proposed structures were shown to be able to escape early and late endosomic vesicles and lysosomal cell compartment, due to their positive charge**

Our Innovation

- **Cationic building blocks of peptide nucleic acid (PNA) were prepared.**
- **Several binding-enhancing substances were incorporate to the designed cationic sequences, resulting in fully charged sequences, or partially-charged PNA.**
- **The selective use of such binding-enhancing substances was shown to contribute significantly to the hybridization between the polymer and the targeted DNA.**
- **The group will also be able to produce a PNA-cassette that will allow for hybridization with particularly large DNA sequences.**

Development Milestones

- **Building block and enhancer and PNA production scale-up**
- **Establish cell membrane and BBB permeation in larger scales, in various biological systems**

The Opportunity

- **The proposed technology will be offered to anti-gene and anti-sense drug manufacturers. These drugs are mainly targeted at is non-responding cancer patients. According to the US National Institute of Cancer, it is estimated that 50% of cancer patients develop multidrug resistance to anticancer drugs.**

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