## Licensable Technologies

# Piperazine Nucleic Acid Analogs

### **Applications:**

- Anti-sense pharmaceuticals
- "Knock-out" studies

#### **Benefits:**

- Can be made in current solid-phase synthesizers
- Low toxicity
- Low aggregation
- High solubility
- High specificity
- Chiral fidelity

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#### Summary:

Los Alamos National Laboratory scientists have developed an advanced nucleic acid analog with a piperazine backbone (PzNA). PzNA may be used in antisense settings as an improvement over current peptide nucleic acids.

Like other nucleic acid analogs, PzNA will bind to specific DNA or RNA target sequences. PzNA has advantages over earlier nucleic acid analogs. Critically, PzNA should have lower aggregation problems than do peptide nucleic acids, and PzNA should be digestible by RNAse H. Finally, piperazine is known to be well-tolerated pharmaceutically, so PzNAs should be associated with low toxicity. For all these reasons, our scientists believe PzNA will form the next wave of antisense therapies.

In addition to its potential advantages as a pharmaceutical, PzNA is easily synthesized using standard solid-phase synthesizers. Thus, the capital costs for beginning PzNA synthesis are small or nonexistent.

Also, PzNA's sequence specificity should make it an excellent laboratory tool for detecting specific nucleotide sequences and inactivating genes in test organisms.

#### **Development Stage:**

We have synthesized PzNA and characterized it using NMR.

#### **Patent Status:**

US Patent 6,841,675 Piperazine-Based Nucleic Acid Analogs

#### **Licensing Status:**

Available for exclusive and non-exclusive licensing.

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