



## May 2006 BIOTECH SEMINAR

presented and sponsored by:  
**THE NORTH AMERICA TAIWANESE ENGINEERS' ASSOCIATION'S**  
**~SIG BIOTECH~**

### **PRESENTATIONS:**

*“RNA Interference (RNAi): A New Therapeutic Modality to Treat Disease”*

**DATE:** Friday, May 5<sup>th</sup>, 2006

**TIME:** 6:30 PM

**LOCATION:** Squire Sanders Law Firm  
600 Hansen Way, Palo Alto, CA 94304

**REGISTRATION:** FREE for NATEA members, \$5 for non-members, pizza/ soft drink  
**RSVP:** [ysyang@stanford.edu](mailto:ysyang@stanford.edu) or [slcheng1@yahoo.com](mailto:slcheng1@yahoo.com)

### **SPEAKER'S BIO:**

Linda B. Couto, Ph.D.

Dr. Couto joined Benitec in July 2004 as the Director of Preclinical Development and oversees all aspects of preclinical development related to Benitec's proprietary DNA-directed RNAi technology. This technology is being applied at Benitec to the treatment of both HCV and HIV and utilizes both viral and non-viral delivery systems. Prior to joining Benitec, she held a number of positions at Avigen from 1995-2003, most recently as Senior Director of Applied Research. At Avigen, she directed gene therapy research and development using Adeno-associated viral vectors to treat a number of inherited diseases, including hemophilia A and B. Dr. Couto was responsible for the preclinical translational research and provided the leadership that led to the successful filing of two INDs for hemophilia B gene therapy. From 1990-1995, Dr. Couto worked at Somatix as a research scientist in the area of *ex vivo* gene therapy. She holds a Ph.D. in molecular toxicology from the Massachusetts Institute of Technology.

### **ABSTRACT:**

RNA interference (RNAi) is a conserved biological response to double-stranded RNA that results in post-transcriptional gene silencing. It is mediated by short interfering RNAs (siRNAs) of 21-25 nucleotides which direct the sequence-specific cleavage of cognate mRNAs, and inhibit their translation. This powerful endogenous mechanism is being

exploited to develop new therapies against diseases that result from over-expression or aberrant expression of proteins, or from viral infections. Two approaches are being utilized to harness the RNAi pathway for the development of novel therapeutics. One involves the delivery of pre-synthesized short interfering RNA (siRNA) duplexes to cells either as naked oligonucleotides or in a complex with one of a number of formulations. The other involves delivery of viral vectors that express short hairpin RNAs (shRNAs), which are converted intracellularly to siRNAs. In both cases, siRNAs are loaded into the RNA induced silencing complex, known as RISC, which mediates the site specific cleavage of cognate RNAs. The choice of these approaches is guided by a variety of factors including the clinical indication and the target tissue. Multiple companies, including Benitec, are advancing RNAi-based therapies into the clinic for a diverse range of indications. Benitec is focused on the development of expressed shRNAs to treat two viral infections that represent major world-wide health problems; i.e., HCV and HIV. This presentation will summarize Benitec's programs in these areas, as well as current progress in the preclinical and clinical development of RNAi-based therapeutics in general.