

## Silencing Cells

### A new therapy shuts down the genetic process that causes eye disease

By Kevin Kelleher | January 2005

In the seven years since scientists discovered RNA interference, or RNAi—a way of hijacking a cell's defense mechanism to silence defective genes—the technology has been hailed as a potential treatment for everything from cancer to coronary heart disease. Now scientists are putting it to the test in humans.

This past November, Acuity Pharmaceuticals in Philadelphia began testing an RNAi-based therapy in 12 patients suffering from age-related macular degeneration, the leading cause of blindness in people over 55. The treatment, which involves an injection of engineered RNA strands into the patient's eyes, is a simple, if not painless, process [see below]. The strands are designed to intercept faulty instructions sent by sick genes inside eye cells, thereby halting the deterioration of the macula. Although scientists are still unsure about potential side effects, given the alternative of almost certain blindness, patients feel that it's worth the risk.

