Drug that ‘mutes’ defective genes that cause nerve disorders effective in trials

A crucial, late-stage clinical trial showed that the [nerve disorder drug patisiran] works—and that it’s safe. And now the biotech company behind it, Alnylam, is poised to bring this first-of-its-kind therapy to market. The news has thrilled both patients and scientists, who have been working for decades on the technology to mute misbehaving genes, known as RNA interference, or RNAi.

…

Most drugs work by targeting ill-formed or malfunctioning proteins and trying to excise them from the body. RNAi, by contrast, goes after the genetic source of the faulty protein production and shuts that system down. When it works, it can ease symptoms in patients with no other options.

Alnylam’s lead RNAi drug patisiran, aimed at treating a rare nerve disorder called familial amyloid polyneuropathy, is projected to ultimately exceed $1 billion in worldwide sales at its peak, which is expected in 2023.

…

“If we can expand the role of RNAi to other organ systems beyond the liver, the likelihood that RNAi could overtake antibodies in terms of importance for diseases of man, animals, and even plants, is certainly there,” said Dr. Geert Cauwenbergh, president and CEO of RXi Pharmaceuticals. “It’ll just take work, like anything else.”

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Experimental Drug That Mutes Defective Genes Raises New Hopes