What one boy’s ‘Lazarus-like’ recovery tells us about the future of bio drugs

Shortly after Cameron Harding’s one-month check-up, his mother, Alison, saw that her newborn seemed to stop moving. She’d unwrap him from a swaddle and his arms would flop to one side. He wouldn’t kick his legs or turn his head.

The diagnosis: spinal muscular atrophy.

Cameron’s case was severe enough he’d probably never even have a birthday.

But when he was seven weeks old, Cameron’s parents enrolled him in a clinical trial for an experimental drug. In videos shot two months later, he could move his head and reach for a toy. No child with his condition had ever made such a recovery before.

The drug Cameron received, Spinraza, was approved in the U.S. just before Christmas and may become the first blockbuster in a novel category of drugs called RNA therapeutics…

What drugs like Spinraza seek to do instead is use RNA to block, modify, or add to, the existing RNA messages in a cell.

“The dirty secret of RNA therapies is that most people are working on stuff where there is competition and there are other molecules,” [Rachel Meyers, previously a senior executive at Alnylam] says. “It’s those ones that don’t have a good alternative where you say, ‘Oh my gosh, we can really change the world, or someone’s life, with this.’

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: How a Boy’s Lazarus-like Revival Points to a New Generation of Drug

For more background on the Genetic Literacy Project, read GLP on Wikipedia.