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a health care blog

Hope For Muscular Dystrophy Patients?

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Clinical trial results are due out soon that could give hope to some patients with Duchenne muscular dystrophy, a rare gen wheelchairs by adolescence. Even the survivors wind up on ventilators and die in their 40s.

The hope comes from a new drug called ataluren, which could help 13% of the 13,000 boys with Duchenne. The drug, ma Plainfield, N.J.. The drug targets a particular type of genetic mishap, called a nonsense mutation, in which a single misspel muscle-making protein called dystrophin. Lack of this protein is what causes Duchenne.

Ataluren forces the body to keep reading the gene, despite the misspelling. The 174-patient trial that is expected soon test walk in six minutes, compared to placebo. (see:Stopping The Nonsense) Mark Schoenebaum, the biotechnology analyst a in the study has now been treated for 48 weeks, and that final results could come within two weeks. A PTC spokeswoman

Schoenebaum says that the results could be good news for PTC's partner, Genzyme, which plans to sell ataluren outside t drug. Assuming that half of them wind up taking it and that it costs $150,000 annually, this would mean $100 million in tota Schoenebaum writes. If ataluren works in cystic fibrosis, where it is also being tested, that could mean another $400 millio the drug aren't expect until next year.

Ataluren is interesting not just because it could help patients with rare diseases for which drugs are hard to come by, but al targets a particular kind of genetic flaw that can cause all manner of rare ailments. If it succeeds, it could be the first of a ne cause.

Muscular dystrophy has turned out to be an incredibly hard disease to treat. Wyeth, now part of Pfizer, quietly released ne gene that causes some animals to bulk out. Amgen and Acceleron, a privately held firm, are still working on myostatin bloc drug in preclinical development.

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- November 2009
- October 2009
- September 2009
- August 2009
- June 2009
- April 2009
- March 2009
- February 2009
- January 2009
- December 2008
- November 2008
- October 2008
- September 2008
- August 2008
- July 2008
- June 2008
- May 2008
- April 2008
- March 2008
- February 2008
- January 2008
- December 2007
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