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Hope For Muscular Dystrophy Patients?

February 26, 2010 - 1:53 pm

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Matthew HerperBio | Email Matthew Herper is a senior editor at Forbes

Clinical trial results are due out soon that could give hope to some patients with Duchenne muscular dystrophy, a rare gene 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, mac 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 total Schoenebaum writes. If ataluren works in cystic fibrosis, where it is also being tested, that could mean another \$400 millior 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.

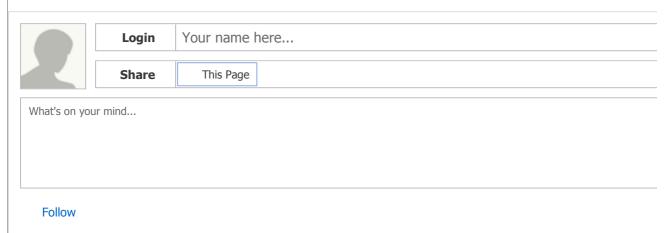
Muscular dystrophy has turned out to be an incredibly hard disease to treat. Wyeth, now part of Pfizer, quietly released nev gene that causes some animals to bulk out. Amgen and Acceleron, a privately held firm, are still working on myostatin bloc drug in preclinical development.

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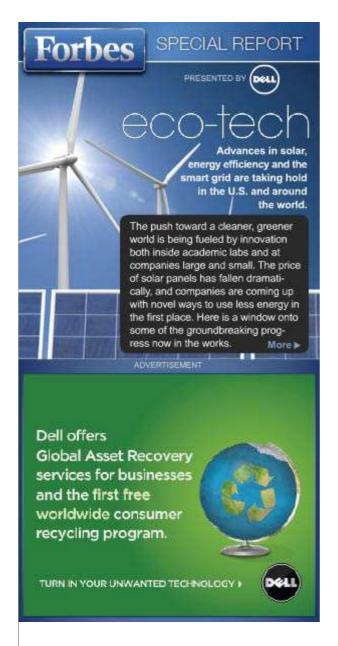
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