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## The Science Business

a health care blog

### 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 that causes wheelchair use 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, made by PTC, is based in Plainfield, N.J.. The drug targets a particular type of genetic mishap, called a nonsense mutation, in which a single misspelled letter in the 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 to test whether patients can walk in six minutes, compared to placebo. (see: [Stopping The Nonsense](#)) Mark Schoenebaum, the biotechnology analyst at PTC 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 the U.S. drug. Assuming that half of them wind up taking it and that it costs \$150,000 annually, this would mean \$100 million in total sales, Schoenebaum writes. If ataluren works in cystic fibrosis, where it is also being tested, that could mean another \$400 million in sales. The drug isn't expected 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 also because it 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 new class of drugs.

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

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