# Muscular dystrophy ‘death sentence’ targeted by gene therapy trials

[T]hree U.S. teams say they are ready to try to treat Duchenne [Muscular Dystrophy] with gene therapy. The first study could begin as soon as next month at Nationwide Children’s Hospital in Columbus, Ohio… Two other tests on children…are slated to begin by year’s end and in the first half of 2018, respectively.

Each will test a “microdystrophin,” meaning a foreshortened copy of the dystrophin gene that’s small enough to fit inside a virus, which is required to shuttle the genetic information into a person’s cells…

Duchenne muscular dystrophy strikes one in every few thousand boys, but rarely affects girls. By their teens, most boys can’t walk anymore. They often die in their 20s and there are few treatment options.

Although the mini-genes won’t be perfect, doctors hope that boys who receive them will end up with a mild handicap…instead of a death sentence…

The earlier a boy is treated, doctors believe, the more muscle cells he’ll still have left and the greater the chance of success. And given the slow pace of drug development—and of gene therapy in particular—some parents see the new microgene trials as their best, and maybe only, chance before their boys are off their feet, in wheelchairs, and no longer eligible.

**The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post:** Doctors Plan Bold Test of Gene Therapy on Boys with Muscular Dystrophy