First Look: Duchenne Muscular Dystrophy and Related Dystrophinopathies: Developing Drugs for Treatment (FDA Guidance for Industry)

Provides guidance for consideration when developing a drug for treatment of dystrophin-related diseases.

Updated last **March 28, 2018**
for the 02/2018 guidance.

WHAT IT DOES

The Food and Drug Administration (FDA) released a guidance for industry on developing drugs for the treatment of various dystrophinopathies. These diseases result in muscle wasting and, in some patients, ultimately lead to respiratory and cardiac failure. Dystrophinopathies are among the most common of rare diseases and are thus heavily researched; pharmaceutical companies are increasingly creating potential therapies.

The guidance, while not legally binding, provides the FDA's current thinking on the topic, including recommendations on evaluating the safety and risk of potential therapeutics, constructing and carrying out trials to sufficiently demonstrate efficacy, and bypassing certain typical study measures or outcomes so as to offer a potential treatment in an expeditious manner.

RELEVANT SCIENCE

Dystrophinopathies are diseases that result from mutations in the dystrophin gene, including Duchenne muscular dystrophy (DMD), Becker muscular dystrophy, and DMD-associated dilated cardiomyopathy.

Many therapies are being developed for dystrophinopathies, including methods related to exon skipping and gene editing, as well as introducing a smaller version of the dystrophin protein that may restore function.

STATUS

The FDA, Department of Health and Human Services, Center for Drug Evaluation and Research, and Center for Biologics Evaluation and Research published this non-binding guidance for industry in February 2018.

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