Duchenne Clinical Trials and Drug Development: Today and in the Future

Webinar Questions from Participants

1. How quickly will Tamoxifen be available for Duchenne after Europe? What are risks to doctors in the US prescribing Tamoxifen off-label?
   We cannot say how quickly Tamoxifen will be available in the US for Duchenne. Physicians in the US may prescribe medications for an individual patient off-label as part of the practice of medicine. This is an accepted practice, provided appropriate information on the benefits and risks have been explained to the patient. Whether insurance will cover the cost is another matter.

2. If a company validates microdystrophin expression as a biomarker to functional benefit using research grade manufacturing material, a path for accelerated approval be possible with commercial material and level of microdystrophin only (without functional benefit)?
   The validation of a biomarker as described could potentially be used to support accelerated approval.

3. How does the FDA support clinical research about non-prescription drugs or supplements for Duchenne?
   FDA is a regulatory agency that funds a very limited amount of clinical research, generally on very specific regulatory topics. NIH and others fund clinical research.

4. How does the FDA prioritize research for life-threatening conditions like Duchenne to ensure drugs can reach patients as quickly as possible to extend life or prevent death?
   FDA is a regulatory agency that funds a very limited amount of clinical research, generally on very specific regulatory topics. NIH and others fund clinical research. FDA does, however, prioritize the review of products for serious diseases like Duchenne.

5. Is there a way to transplant the "good" chromosome from the mother/carrier to take the place of or override the defective chromosome?
   There are gene therapy approaches under investigation that can accomplish something similar in an individual. However, these are not being used to correct the defect so that is no longer inherited from one generation to the next. That is not permitted in the US at this time.

6. Does the FDA ever allow wide distribution of a drug after Phase 2 for patients willing to take the risk?
   Yes, it frequently does as part of expanded access programs. Please see the FDA's expanded access site:
   https://www.fda.gov/news-events/public-health-focus/expanded-access

7. What is the turnover of FDA members? For instance does the same team follow a clinical trial all the way through or do they keep switching throughout the duration of the study?
   Although some of the reviewers come and go, others stay working with a product from when it first comes in to the agency for early investigation through to its regulatory approval.

8. When the FDA is considering the risk/benefit profile of a drug, how is the "risk tolerance" of the patient group factored in? For example, patients with Duchenne may accept a higher degree of risk for negative side effects vs other patient populations.
   FDA routinely considers the seriousness of disease as it looks at the balance of risks and benefits prior to approval. Exactly, as noted, some with serious diseases may be more willing to accept side effects than those with more minor conditions.

9. Are the bio markers set by the FDA or the drug companies? Will there ever be any new biomarkers that would make it easier to include more patients?
   Biomarkers are developed by academic investigators, industry, and sometimes in conjunction with FDA. This is an area of heavy focus in the field and at the agency, and we certainly expect better biomarkers to be developed over the next years that will facilitate more efficient product development.

Watch webinar at cureduchenne.org/cure/webinars/

1400 Quail Street, Suite 110, Newport Beach, CA 92660 | (949) 872-2552 | info@cureduchenne.org | Federal Tax ID #: 501(c)(3) 20-0299958