



News Articles, FDA Update, Disabilities, Pharmacology

FDA reviews ethical question in muscular dystrophy clinical trial

by from the Food and Drug Administration Office of Pediatric Therapeutics and Division of Pediatric and Maternal Health

The Food and Drug Administration (FDA) has determined that a two-year study using a totally implantable central venous access device (TICVAD) to administer an investigational drug or placebo to children with Duchenne muscular dystrophy (DMD) may proceed.

An institutional review board (IRB) asked the FDA to determine if use of the TICVAD for infusion of placebo would be acceptable after a subject's parent complained about problems with the use of peripheral intravenous lines.

The randomized, double-blind, placebo-controlled study will proceed under criteria established by Additional Safeguards for Children in Clinical Investigations (21 CFR part 50 subpart D). Under these regulations, placement of a TICVAD for children in the active treatment arm would be ethically acceptable since they are offered a prospect of direct clinical benefit. A "direct clinical benefit" may improve the health or well-being of the individual child enrolled in the research *and* results from the intervention being studied (and not from other clinical interventions included in the protocol).

Since patients randomized to placebo do not have a prospect of direct clinical benefit as they receive no active treatment, the IRB sought formal review by the FDA Pediatric Advisory Committee (PAC), including an opportunity for public comment. The PAC voted unanimously on May 18 to recommend use of a TICVAD at the discretion of the parent or guardian in consultation with the investigator and consulting surgeon. In addition, adequately informed and voluntary parent or guardian permission and child assent must be obtained. The FDA determination was issued May 25.

The FDA determined that a TICVAD should be used over other forms of central venous access, unless contraindicated, due to its lower incidence of infection, thrombosis and other complications.

Resource

- [The meeting materials and FDA determination letter are posted on the FDA website.](#)