U.S. Food and Drug Administration

Vaccines, Blood & Biologics

Public Workshop on Cell and Gene Therapy Clinical Trials in Pediatric Populations

November 2, 2010
8:00 AM-5:30 PM

Workshop Goals and Objectives

The FDA will convene a one-day workshop to facilitate an exchange of information about best practices in conducting cel and gene therapy clinical trials in pediatric populations. Cellular and gene therapies are the subject of great interest as novel products that potentially may improve the lives of patients by restoring lost function and modifying the nature and course of diseases. However, these therapies are not without risks: indeed, the novelty of these products contributes to their real and potential risks. Conducting clinical trials with novel products in pediatric patients requires special scrutiny to ensure that the rights of subjects are protected and that potential risks and benefits are appropriately balanced.

The purpose of the workshop is to gather information from Institutional Review Boards (IRBs), gene and cellular therapy clinical researchers, and other stakeholders regarding best practices related to cell and gene therapy clinical trials in pediatric populations, as well as challenges and considerations in the review of these clinical trials.

The workshop will include presentations on cell and gene therapy clinical trials in pediatric populations. The workshop will include panel discussions regarding best practices related to cell and gene therapy clinical trials in pediatric populations including those related to: (1) evaluating these novel therapeutic products prior to initiating pediatric clinical studies; (2) identifying and minimizing risks associated with the administration of cell and gene therapy products in pediatric populations; (3) obtaining informed consent and assent; and (4) conducting continuing review, of cell and gene therapy products in pediatric populations. The workshop also will include panel discussions addressing the challenges an considerations in the review of cell and gene therapy clinical trials in pediatric populations and the role of institutional review boards.

Workshop Location

Bethesda North Marriott Hotel and Conference Center
5701 Marinelli Rd
North Bethesda, MD 20852

Registration

There is no registration fee for the public workshop. Early registration is recommended because seating is limited. Registration on the day of the public workshop will be provided on a space available basis beginning at 7:30 a.m. Email mail or fax your registration information (including name, title, firm name, address, telephone and fax numbers) to the contact person by October 1, 2010.
If you need special accommodations due to a disability, please contact Bernadette Kawaley (see Contact Person) at leas 7 days in advance.

Contact Person

Bernadette Kawaley
Center for Biologics Evaluation and Research (HFM-43)
Food and Drug Administration,
1401 Rockville Pike, Suite 200N, Rockville, MD 20852-1448
Phone (301) 827-2000, Fax (301) 827-3079; email: CBERTraining@fda.hhs.gov (Subject line: Pediatrics Ethics Workshop).

Federal Register

- FR Notice: Cell and Gene Therapy Clinical Trials in Pediatric Populations; Public Workshop (PDF - 51KB)[1]

Agenda

- Agenda for the Public Workshop on Cell and Gene Therapy Clinical Trials in Pediatric Populations[2]

Links on this page:

1. http://www.fda.gov/downloads/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/UCM224740.pdf
2. http://www.fda.gov/BiologicsBloodVaccines/NewsEvents/WorkshopsMeetingsConferences/ucm225184.htm