

## DEPARTMENT OF HEALTH AND HUMAN SERVICES

### Food and Drug Administration

[Docket No. FDA–2009–N–0664]

### Improving Endpoints, Improving Care: Alpha-1 Antitrypsin Augmentation Therapy and Clinical Trials; Public Workshop

**AGENCY:** Food and Drug Administration, HHS.

**ACTION:** Notice of public workshop.

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The Food and Drug Administration (FDA) is announcing a public workshop entitled: Improving Endpoints, Improving Care: Alpha-1 Antitrypsin Augmentation Therapy and Clinical Trials. The purpose of the public workshop is to identify the most useful clinical trial endpoints and surrogate markers for Alpha-1 antitrypsin (AAT) augmentation therapy. FDA, Alpha-1 Foundation, and the Department of Health and Human Services, Office of Public Health and Science are convening this workshop to facilitate the design of future clinical trials intended to establish clinical efficacy of AAT products. The public workshop will feature presentations and panel discussions led by experts from academic institutions, government, and industry.

*Date and Time:* The public workshop will be held on March 23, 2009, from 8:30 a.m. to 5:30 p.m. and March 24, 2009, from 8:30 a.m. to 5 p.m.

*Location:* The public workshop will be held at the Lister Hill Center Auditorium, Bldg. 38A, National Institutes of Health, 8800 Rockville Pike, Bethesda, MD 20894.

*Contact Person:* Rhonda Dawson, Center for Biologics Evaluation and Research (HFM–302), Food and Drug Administration, 1401 Rockville Pike,

suite 200N, Rockville, MD 20852–1448, 301–827–6129, FAX: 301–827–2843, e-mail: [rhonda.dawson@fda.hhs.gov](mailto:rhonda.dawson@fda.hhs.gov).

*Registration:* Mail, fax, or e-mail your registration information (including name, title, firm name, address, telephone, and fax numbers) to the contact person by March 6, 2009. There is no registration fee for the public workshop. Early registration is recommended because seating is limited to 175 attendees. Registration on the day of the public workshop will be provided on a space available basis beginning at 7:30 a.m.

If you need special accommodations due to a disability, please contact Rhonda Dawson (see *Contact Person*) at least 7 days in advance.

**SUPPLEMENTARY INFORMATION:** AAT deficiency is a genetic condition that leads to decreased levels of alpha-1 antitrypsin in the blood and significantly increases the risk of serious lung disease in adults and liver disease in infants, children, and adults. Intravenous augmentation therapy with FDA-licensed, plasma-derived AAT products has become the standard of care for treatment in the subset of patients with AAT deficiency who have moderate pulmonary disease. Since the original product approvals, additional data collection and advances in understanding of AAT deficiency suggest the need to revisit and improve clinical trial efficacy endpoints.

The public workshop will facilitate scientific discussions to identify the most relevant and feasible, currently available and future clinical trial efficacy endpoints for AAT augmentation therapy and further evaluate its usefulness to a broader patient population. Topics to be discussed include: (1) AAT deficiency disease characteristics, progression and pulmonary pathophysiology; (2) patient selection for clinical trials; (3) current challenges to the development of endpoints for clinical trials; and (4) currently available

and future clinical trial endpoints, including functional markers of disease progression, and radiological and biochemical endpoints.

*Transcripts:* Transcripts of the public workshop may be requested in writing from the Freedom of Information Office (HFI-35), Food and Drug Administration, 5600 Fishers Lane, rm. 6-30, Rockville, MD 20857, approximately 15 working days after the public workshop at a cost of 10 cents per page. A transcript of the public workshop will be available on the Internet at <http://www.fda.gov/cber/minutes/workshop-min.htm>.

Dated: February 6, 2009.

**Jeffrey Shuren,**

*Associate Commissioner for Policy and Planning.*

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