Pediatric Subcommittee

of the

Anti-Infective Drugs Advisory Committee

Center for Drug Evaluation and Research, Food and Drug Administration

Hyatt Regency Bethesda, One Bethesda Metro Center, Bethesda,MD

September 11, 2000

AGENDA

Morning Session - Ethical Issues

8:00 a.m. **Call to Order/Introductions**
P. Joan Chesney, M.D., Chair, Pediatric Advisory Subcommittee

Conflict of Interest Statement
Jayne E. Peterson, R.Ph., J.D., Subcommittee Executive Secretary

8:05 a.m. **Welcome and Review of Meeting Agenda/Background Information and Overview**
Dianne Murphy, M.D., Associate Director of Pediatrics, Center for Drug Evaluation and Research (CDER), FDA

8:15 a.m. **Presentations/Discussion:**
Part 1: The Ethics of Placebo-Controlled Clinical Trials in Children

Open Public Hearing
(******30 minutes allocated unless public participation does not last that long.)

8:45 a.m. **Overview of Placebo-Control Trial Design: Benefits and Difficulties**
Robert Temple, M.D., Director, Office of Medical Policy, CDER

9:10 a.m. **International Perspective on Pediatric Placebo-Controlled Trials**
Dr. Barbara van Zwieten-Boot
Efficacy Coordinator, Medicines Evaluation Board, NL
Vice Chair of the Efficacy Working Party, CPMP

9:30 a.m. **Questions from the Subcommittee**

9:40 a.m. **Ethical Concerns in Pediatric Placebo-Controlled Trials**
Charles Weijer, M.D., Ph.D., Bioethicist and Asst. Prof. of Medicine, Dalhousie University, Halifax, Nova Scotia

10:00 a.m. **Ethical Concerns in Pediatric Placebo-Controlled Trials from the European Experience**
Prof. Francis P. Crawley, Chairman, Ethics Working Party, European Forum for Good Clinical Practice & Member, Ethics Working Group, Confederation of European Specialists in Paediatrics

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AGENDA (cont.)

10:20 a.m. **Questions from the Subcommittee**

10:30 a.m. **Break**

10:45 a.m. **Use of Data and Safety Monitoring Boards (DSMB) and their Role in Pediatric Clinical Trials**
Susan Ellenberg, Ph.D., Director, Office of Biostatistics and Epidemiology, Center for Biologics Evaluation and Research (CBER), FDA

11:05 a.m. **Subcommittee Questions to Speakers**

11:10 a.m. **Subcommittee Discussion of Case Studies/Introductory Remarks**
Dianne Murphy, M.D.

Example A: Pediatric placebo-controlled <u>Add-on</u> clinical trial design
Presentation of Example: Debra Birenbaum, M.D., Medical Officer, Division of Pulmonary and Allergy Drug Products
Assumptions are the following:

- The patient has the disease/condition
- The patient is receiving the standard of care therapy
- The disease/condition is stable but with less than optimal control of signs/symptoms and/or exacerbations
- Examples to be given: asthma, seizures

Question: Is there a situation, population, disease/condition where this type of placebo-controlled study would not be appropriate? (see attached)

11:45 a.m. **Lunch**

12:45 p.m. **Example B: Pediatric placebo-controlled trial design when there is <u>No Approved Therapy</u>**
Presentation of Example: Rosemary Roberts, M.D., Medical Officer, Pediatric Team
Assumptions are the following:

- The patient has a chronic disease/condition and requires long term treatment
- Approved therapy in adults for the condition, but no approved therapy in the pediatric population
- Examples to be given: depression
- Question: Is there a situation, population, disease/condition where this type of placebo-controlled study would not be appropriate? (see attached)

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AGENDA (cont.)

1:45 p.m. **Example C: Pediatric placebo-controlled clinical trial design including a <u>Withdrawal Phase</u> when there is only one or limited effective therapy**
Presentation of Example: Steven Hirschfield, M.D., Ph.D, Medical Officer, Division of Oncology Drug Products
Assumptions are the following:

- The patient has a chronic disease/condition and requires long term treatment
- The disease/condition is considered stable but patient continues to have intermittent exacerbations on present therapy
- There are limited therapeutic options available
- Examples to be given: asthma, hypertension

Question: In what situation, population, disease/condition do you see this type of placebo-controlled study as appropriate and when is it not? (see attached)

2:45 p.m. **Break**

Afternoon Session – Pediatric Psychotropic Drug Use Issues

3:00 p.m. **Part 2: A Proposed Approach to the Development of Psychotropic Drug Therapies for Pediatrics/Introduction**
Dianne Murphy, M.D.

Conflict of Interest Statement
Jayne E. Peterson, R.Ph., J.D., Subcommittee Executive Secretary

3:05 p.m. **Open Public Hearing** (**30 minutes allocated unless public participation does not last that long.)

3:35 p.m. **Current Regulatory Issues in Pediatric Psychopharmacology**
Thomas Laughren, M.D., Team Leader, Psychiatric Drug Products, Division of Neuropharmacological Drug Products, CDER, FDA

3:50 p.m. **Pediatric Psychopharmacology: A Clinical Perspective**
Richard Malone, M.D., Eastern Pennsyl. Psychiatric Institute, Philadelphia, PA

4:05 p.m. **Pediatric Psychopharmacology: A Research Perspective**
Mark Riddle, M.D., Johns Hopkins Medical Institutions, Baltimore, MD

4:20 p.m. **NIMH Perspective on Pediatric Psychopharmacology**
Benedetto Vitiello, M.D., Chief, CATPIRB, DISR, NIMH, Bethesda, MD

4:35 p.m. **Subcommittee Discussion of Questions/Issues**

5:25 p.m. **Closing Remarks**

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