Minimal Residual Disease as a Surrogate Endpoint in Hematologic Cancer Trials  
Hotel Monaco • Washington, DC  
September 7, 2016

Meeting objectives: In order to support the goal of expediting the development and review of innovative new medicines intended to address unmet medical needs for serious or life-threatening diseases (FDASIA 901(a)(c)), the Duke-Margolis Center for Health Policy, under a cooperative agreement with the U.S. Food and Drug Administration, is convening a public meeting to advance the discussion around the validation and use of minimal residual disease (MRD) as a surrogate endpoint in clinical trials for hematologic malignancies. Specifically, the objectives for this event are to: 1) discuss the regulatory background for use of MRD as a surrogate endpoint for regulatory decisions; 2) discuss the statistical basis for demonstrating and validating surrogacy; and 3) present the evidence available to support new surrogate endpoints for clinical trials in new treatments of multiple myeloma, chronic lymphocytic leukemia, acute lymphoblastic leukemia, and acute myeloid leukemia.

8:30 a.m.  Registration

9:00 a.m.  Welcome and Introductions  
Mark McClellan, Duke-Margolis Center for Health Policy  
Greg Daniel, Duke-Margolis Center for Health Policy

9:15 a.m.  Surrogate Endpoints in Regulatory Decision-Making: The FDA Perspective  
Vishal Bhatnagar, U.S. Food and Drug Administration

9:25 a.m.  In Vitro Diagnostics in Hematologic Cancer  
Jennifer Dickey, U.S. Food and Drug Administration

9:45 a.m.  Major Biostatistical Considerations for Demonstrating Surrogacy  
Daniel Sargent, Mayo Clinic

10:30 a.m.  Break

10:45 a.m.  Session I: MRD as a Surrogate Endpoint in Multiple Myeloma

Presentation:  
Ola Landgren, Memorial Sloan Kettering Cancer Center

Moderator:  
Mark McClellan, Duke-Margolis Center for Health Policy

Panelists:  
Nicole Gormley, U.S. Food and Drug Administration  
Nancy Valente, Genentech  
Daniel Auclair, Multiple Myeloma Research Foundation  
Richard Little, National Cancer Institute  
Antje Hoering, Cancer Research and Biostatistics
11:45 a.m. Session II: MRD as a Surrogate Endpoint in Chronic Lymphocytic Leukemia (CLL)

Presentation: John Byrd, The Ohio State University

Moderator: Greg Daniel, Duke-Margolis Center for Health Policy

Panelists: Angelo de Claro, U.S. Food and Drug Administration
Rod Humerrickhouse, AbbVie
Sumithra Mandrekar, Mayo Clinic
Carol Preston, Patient Power
Aaron Logan, University of California, San Francisco

12:45 p.m. Lunch

1:45 p.m. Session III: MRD as a Surrogate Endpoint in Acute Lymphoblastic Leukemia (ALL)

Presentation: Jerald Radich, Fred Hutchinson Cancer Center

Moderator: Mark McClellan, Duke-Margolis Center for Health Policy

Panelists: Ashley Ward, U.S. Food and Drug Administration
Janet Franklin, Amgen
Donald Berry, M.D. Anderson
Wendy Stock, University of Chicago

2:45 p.m. Session IV: MRD as a Surrogate Endpoint in Acute Myeloid Leukemia (AML)

Presentation: Christopher Hourigan, National Heart, Lung, and Blood Institute

Moderator: David Rizzieri, Duke Cancer Institute

Panel Discussion: Donna Przepiorka, U.S. Food and Drug Administration
Sharon McBain, Janssen
Amy Burd, Leukemia and Lymphoma Society
Lisa McShane, National Cancer Institute
Gail Roboz, Weill Medical College of Cornell University

3:45 p.m. Closing Remarks
Mark McClellan, Duke-Margolis Center for Health Policy

4:00 p.m. Adjournment

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