Advancing Drug Development in Myelodysplastic Syndromes (MDS)

Organized by the U.S. Food & Drug Administration (FDA) and National Cancer Institute (NCI)

May 16th to 17th, 2023

Meeting objectives: In order to support the goal of expediting the development of drugs and biological products for the treatment of MDS, the FDA Oncology Center of Excellence (OCE) in coordination with the NCI, is convening a public meeting to advance the discussion around optimal design of clinical trials for patients with MDS. Specifically, the objectives for this event are to: 1) discuss key eligibility considerations and risk stratification on MDS clinical trials; 2) discuss optimal definitions of MDS clinical trial endpoints; and 3) present available evidence to support new endpoints for clinical trials in MDS.

DAY 1

12:30 p.m. **Welcome and Introductions**

Kelly Norsworthy, U.S. Food and Drug Administration

Steven Pavletic, National Cancer Institute

12:45 p.m. **Session I: Risk Stratification and eligibility for MDS clinical trials**

Co-Chairs:

Guillermo Garcia-Manero, MD Anderson Cancer Center

James Foran, Mayo Clinic Cancer Center

Panelists:

Katherine R. Calvo, National Institutes of Health Clinical Center

Lea Cunningham, National Cancer Institute

Peter Greenberg, Stanford University

Robert Hasserjian, Massachusetts General Hospital, Harvard Medical School

Nina Kim, U.S. Food and Drug Administration

Alain Mina, National Cancer Institute

Aziz Nazha, Incyte

Elli Papaemmanuil, Memorial Sloan Kettering Cancer Center

Anupam Verma, Inova Schar Cancer Institute

2:00 p.m. **Session II: Response Criteria**

Co-Chairs:

Jacqueline Garcia, Dana Farber Cancer Institute

Amer Zeidan, Yale University

Panelists:
Uma Borate, The Ohio State University
Guillermo Garcia-Manero, MD Anderson Cancer Center
Emily Jen, U.S. Food and Drug Administration
Noa Holtzman, National Cancer Institute
Richard Little, National Cancer Institute
Kelly Norsworthy, U.S. Food and Drug Administration
Steven Pavletic, National Cancer Institute
Bart Scott, Fred Hutchinson Cancer Center, University of Washington
David Steensma, formerly Novartis

3:15 p.m. **Break**

3:30 p.m. **Session III: Time-to-event Endpoints**

Co-Chairs:
Amy DeZern, Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University
Rami Komrokji, Moffitt Cancer Center

Panelists:
Maria Diez-Campelo, Universidad de Salamanca
Lori Ehrlich, U.S. Food and Drug Administration
Liz Garrett-Mayer, American Society of Clinical Oncology
Steven Gore, National Cancer Institute
Emma Groarke, National Heart, Lung, and Blood Institute
Alain Mina, National Cancer Institute
Valeria Santini, University of Florence
Jonathon Vallejo, U.S. Food and Drug Administration
Erica Warlick, Syros Pharmaceuticals
Amer Zeidan, Yale University

4:45 p.m. **Closing Remarks**
Alain Mina, National Cancer Institute
Lori Ehrlich, U.S. Food and Drug Administration

5:00 p.m. **Adjournment**

DAY 2

12:30 p.m. **Welcome and Recap of Day 1**
Lori Ehrlich, U.S. Food and Drug Administration
Peter Aplan, National Cancer Institute

12:45 p.m. **Session IV: Transfusion Endpoints**

Co-Chairs:
Mikkael Sekeres, Sylvester Cancer Center, University of Miami
Bart Scott, Fred Hutchinson Cancer Center, University of Washington

Panelists:
Yasmin Abaza, Northwestern University
Rafael Bejar, Aptose Biosciences
Jacqueline Garcia, Dana Farber Cancer Institute
Uwe Platzbecker, University Hospital in Leipzig, Germany
David Swoboda, Cancer Center of South Florida, Tampa General Hospital
Tanya Wroblewski, U.S. Food and Drug Administration

2:00 p.m. **Session V: Functional assessments and integration into clinical trial endpoints**

Co-Chairs:
Rena Buckstein, Odette Cancer Centre, Toronto
Sara Tinsley-Vance, Moffitt Cancer Center

Panelists:
Andrew Artz, City of Hope Cancer Center
Victoria Barghout, Viver Health
Vishal Bhatnagar, U.S. Food and Drug Administration
Tracey Iraca, MDS Foundation
Tito Mendoza, National Cancer Institute
Shannon McCurdy, University of Pennsylvania
Lisa Pleyer, Paracelsus Medical University, Austria
Fabio Efficace, Italian Group for Adult Hematologic Diseases (GIMEMA)

3:15 p.m. **Break**

3:30 p.m. **Session VI: Biomarker development and minimal residual disease**

Co-Chairs:
Kathy McGraw, National Cancer Institute
Amit Verma, Albert Einstein Cancer Center

Panelists:
Omar Abdel-Wahab, Memorial Sloan Kettering Cancer Center
Peter Aplan, National Cancer Institute
Eric J Duncavage, Washington University
Pamela Ebrahimi, U.S. Food and Drug Administration

Maria "Ken" Figueroa, Sylvester Cancer Center, University of Miami
Sylvie D. Freeman, University of Birmingham
Jarek Maciejewski, Cleveland Clinic
Thomas Prebet, Bristol Myers Squibb
Eduard Schulz, National Cancer Institute
Dan Starczynowski, Cincinnati Children's Hospital

4:45 p.m. **Closing Remarks**
Steven Pavletic, National Cancer Institute
Kelly Norsworthy, U.S. Food and Drug Administration

5:00 p.m. **Adjournment**