



A Strategic Framework for Novel Drug Development in Multiple Myeloma
A Multiple Myeloma Research Foundation Roundtable

Washington, DC
June 15-16, 2005

Co-Chairpersons

Kenneth C. Anderson, MD
Alison Hannah, MD
Richard Pazdur, MD

WEDNESDAY

6:30 PM **Welcome Dinner**

FDA Critical Pathway Initiative/Lessons Learned that Can Aid in the Development of Myeloma Drugs - *Rick Pazdur*

THURSDAY

7:00 AM **Continental Breakfast**

8:00 **Welcome, Roundtable Objectives, & Agenda Review** - *Co-chairs*

8:05 **Summary of Key Points: "Development of Effective New Treatments for Multiple Myeloma" Publication** - *Ken Anderson and Ann Farrell*

- Preclinical/Safety Summary
- Established Response Criteria
- Accepted Surrogate Endpoints
- Bortezomib Development
- Accelerated Review Utilizing Surrogate Endpoints
- Rationale behind This Follow-up Roundtable



Session I – Strategic Framework for Novel Drug Development

8:15 **Preliminary Model Presented & Discussed** - *Ken Anderson*

Session II – Strategy for Clinical Trial Development - *Session Chairs: Rick Pazdur and Ann Farrell*

8:30 **Clinical Development Models Explored**

- Bortezomib Development Strategy - *Beth Trehu*
- Lenalidomide Development Strategy - *Bob Knight*

8:45 **Unmet Medical Need in Myeloma –A Changing Landscape** - *Rick Pazdur and Ann Farrell*

9:00 **Discussion**

Session III –Phase 1 Development - *Session Chair: Ken Anderson*

9:30 **Study Design**

- Differences for Small Molecule/MABs - *Michele Bronson*
- Trial Populations (myeloma vs. all comers)
- Is the Target Being Hit? (utilizing biomarkers to determine drug efficacy)
- Combination of Investigational Agent and an Approved Compound
- Combination of Several Investigational Agents

10:30 **Break**

10:45 **Discussion**

Session IV– Phase 2 Development - *Session Chairs: Alison Hannah and Julian Adams*

11:15 **Study Design**

- Combination or Single Agent Design (timing)
- Selected vs. Unselected Populations
- Comparative Trials (comparing drugs, doses, schedules)
- Window of Opportunity Trials
- The Impact of Accelerated Review on Trial Design

12:15 PM **Working Lunch—Discussion** (discussion to encompass 30 mins of lunch)

Session V – Phase 3 Development - *Session Chairs: Bob Knight, Bob DeLap, and Paul Richardson*

1:15 **Study Design Determination** *Bob Knight/Bob DeLap*

- Combination or Single Agent Design
- Process for Determining the Final Trial Design

Establishing a Control Group for Different Study Populations - *Paul Richardson*

Specific Evidence of Clinical Benefit

- “Threshold of Credibility” (AML vs. MM)
- Degree of PFS or TTP Prolongation Needed
- Magnitude of Response
- “Clinical Benefit” = Myeloma-specific Quality of Life Instrument

2:15 **Discussion**



Session VI – Roundtable Recommendations: A Strategic Framework for Novel Drug Development in Multiple Myeloma

Moderated by Alison Hannah/Ken Anderson

3:00 **Modifications to the Preliminary Strategic Framework**

4:00 **Adjournment**

"The views expressed do not necessarily represent the views or findings of the U.S. Food and Drug Administration or the United States."