OPPORTUNITIES AND CHALLENGES IN THE MYELOMA DRUG DEVELOPMENT PROCESS

An MMRF Roundtable
July 23–24, 2008

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# Table of Contents

Welcome.................................................................................................................. 1
Agenda ...................................................................................................................... 2-4
Participant List ........................................................................................................ 5
Biographies
  Guest Speakers ....................................................................................................... 12
  Co-Chairs .............................................................................................................. 13
  Faculty ................................................................................................................... 17
  MMRF and MMRC Staff ....................................................................................... 19
Abstracts
  Orientation – Recent Approvals and Future Challenges in Myeloma Drug Development
    Kenneth C. Anderson, MD ................................................................................... 22
  Adaptive Designs for Cancer Trials
    Donald A. Berry, PhD .......................................................................................... 24
  FDA Overview and Perspectives – Lessons Learned in Oncology
    Ann T. Farrell, MD .................................................................................................. 25
Notes .......................................................................................................................... 26

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Welcome.

On behalf of the Multiple Myeloma Research Foundation (MMRF) it is our pleasure to welcome you to Opportunities and Challenges in the Myeloma Drug Development Process – An MMRF Roundtable. We are pleased to bring together key thought leaders from academia, industry, the National Cancer Institute, and the U.S. Food and Drug Administration, who are involved in this exciting field of cancer research.

The goal of today’s roundtable is to identify real and perceived obstacles to future myeloma drug approvals and develop solutions to overcome them. We appreciate the assistance of our distinguished co-chairs: Kenneth C. Anderson, MD, from the Dana-Farber Cancer Institute, Harvard Medical School; and Richard Pazdur, MD, FACP, from the Center for Drug Evaluation and Research at the FDA. We hope to generate open and stimulating discussion through the use of scenarios that will challenge our current thinking and increase our collective knowledge.

We would like to thank the following organizations for contributing valuable support for this event: Celgene Corporation, Eli Lilly and Company, Pfizer Inc., Merck & Co., Inc., Millennium Pharmaceuticals The Takeda Oncology Company, Centocor Ortho Biotech Services LLC, and Keryx Biopharmaceuticals, Inc.

Once again, thank you for joining us. We look forward to working together today and in future endeavors.

Most sincerely,

Kathy Giusti
Founder and CEO
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Louise M. Perkins, PhD
Chief Scientific Officer
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Susan L. Kelley, MD
Chief Medical Officer
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AGENDA

Wednesday, July 23

7:00 PM    Dinner & Guest Speakers

Translational Research Working Group – Process and Results
Lynn M. Matrisian, PhD
Vanderbilt University Medical Center

An Oncology Quandry: What to Do Between Phase I and Phase III?
Jeffrey D. Helterbrand, PhD
Genentech, Inc.
AGENDA (continued)

Thursday, July 24

7:00 AM  Breakfast and Registration

I. Overview

8:00 AM  Welcome and Introductions
Louise M. Perkins, PhD
Multiple Myeloma Research Foundation

8:10 AM  Orientation – Recent Approvals and Future Challenges in Myeloma Drug Development
Kenneth C. Anderson, MD
Dana-Farber Cancer Institute

8:30 AM  Adaptive Designs for Cancer Trials
Donald A. Berry, PhD
The University of Texas, MD Anderson Cancer Center

8:50 AM  FDA Overview and Perspectives – Lessons Learned In Oncology
Ann T. Farrell, MD
U.S. Food and Drug Administration

9:15 AM  Break
AGENDA (continued)

Thursday, July 24

II. Roundtable Working Sessions
Moderated by Susan L. Kelley, MD

9:30 AM  Session 1 – Drug Development and Approval Strategies: Accelerated Approval Opportunities

11:00 AM  Session 2 – Approaches to Combination Drug Therapy With New and Emerging Agents in Myeloma

12:30 PM  Working Lunch

II. Roundtable Working Sessions: (continued)

1:30 PM  Session 3 – Incorporation of Adaptive Trial Designs Into Myeloma Drug Development

3:00 PM  Discussion, Action Planning, and Wrap-up
         Louise M. Perkins, PhD

4:00 PM  Adjourn
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Jeffrey D. Helterbrand, PhD
Genentech, Inc.

Jeffrey D. Helterbrand, PhD, graduated from St. Olaf College with undergraduate degrees in mathematics and economics, and received his PhD in statistics from Iowa State.

In 1993, Dr. Helterbrand joined Eli Lilly & Company as a statistician focusing on programs in cardiovascular and critical care medicine. He joined Genentech in 2002, initially focusing on programs within oncology and hematology. Dr. Helterbrand is currently the head of biostatistics and epidemiology at Genentech.

Lynn M. Matrisian, PhD
Vanderbilt University Medical Center

Lynn M. Matrisian, PhD, is Chair of the Department of Cancer Biology and Ingram Distinguished Professor of Cancer Research at the Vanderbilt Ingram Cancer Center. She received her BS in medical technology from Bloomsburg University in Pennsylvania, and her PhD in molecular biology from the University of Arizona in Tucson. She was appointed to the faculty of Vanderbilt University in Nashville, TN following postdoctoral training in the Laboratory of Eukaryotic Molecular Genetics in Strasbourg, France.

Dr. Matrisian is past president of the American Association of Cancer Research and was recently awarded the Paget-Ewing award from the Metastasis Research Society for her contributions to understanding how cancer spreads. Research in her laboratory revolves around the molecular mechanisms underlying tumor progression and metastasis, with emphasis on the biology of matrix-degrading proteinases. She currently works half time at the National Cancer Institute of the National Institutes of Health outside Washington, DC, on an initiative to accelerate the translation of laboratory discoveries into better ways to detect, diagnose, treat, and prevent cancer.
Biographies

Co-Chairs

Kenneth C. Anderson, MD
Dana-Farber Cancer Institute
Kraft Family Professor of Medicine
Harvard Medical School

Kenneth C. Anderson, MD, graduated from Johns Hopkins Medical School, trained in internal medicine at Johns Hopkins Hospital, and completed hematology, medical oncology, and tumor immunology training at the Dana-Farber Cancer Institute. He is the Kraft Family Professor of Medicine at Harvard Medical School; and serves as Chief of the Division of Hematologic Neoplasia, Director of the Jerome Lipper Multiple Myeloma Center, and Vice Chair of the Joint Program in Transfusion Medicine at Dana-Farber Cancer Institute. Dr. Anderson serves as chair of the National Comprehensive Cancer Network Multiple Myeloma Clinical Practice Guidelines Committee. He is a Principal Investigator for the Cancer and Leukemia Group B. He serves on the Board of Scientific Advisors of the International Myeloma Foundation; on the Scientific Advisory Board of the Multiple Myeloma Research Foundation; on the Board of Directors and Steering Committee of the Multiple Myeloma Research Consortium. Dr. Anderson has published more than 350 original articles, 250 chapters, and has edited multiple textbooks on both multiple myeloma and on transfusion medicine. He is a Doris Duke Distinguished Clinical Research Scientist and has had long term RO-1, PO-1, and SPORE NIH funding.

His numerous awards include the 2001 Charles C. Lund Award of the American Red Cross Blood Services, the 2003 Waldenström's award for research in plasma cell dyscrasias, the 2004 Johnson & Johnson Focused Giving Award for Setting New Directions in Science and Technology, the 2005 Robert A. Kyle Lifetime Achievement Award, the 2007 American Association for Cancer Research Joseph H. Burchenal Award for Clinical Research, the 2007 ROFEH Distinguished Service Award for providing compassionate patient care internationally, and a 2007 Champion in Advocacy Award from the American Society of Hematology. He was named Editor in Chief of Clinical Cancer Research in 2007. In 2008, he received the Dameshek Prize from the American Society of Hematology.

Over the last 2 decades, Dr. Anderson has focused his translational research studies on B-cell malignancies, especially multiple myeloma. Highlights of his contributions to science and medicine include: discovery of the first plasma cell reactive monoclonal antibodies; development of an immunophenotyping model for diagnosis and treatment of B-cell malignancies; pioneering novel methods to improve safety and efficacy of autografting and allografting in myeloma; characterizing the signaling cascades whereby cytokines mediate myeloma cell growth, survival, and drug resistance in the bone marrow microenvironment; using oncogenomics and developing in vitro and in vivo models to both identify novel targets and validate therapies targeting the myeloma cell and its bone marrow milieu; translating these preclinical studies to the bedside in derived phase I–III clinical trials; and establishing a new treatment paradigm using novel therapies targeting the tumor cell, tumor-host bone marrow interaction, and bone marrow microenvironment to overcome drug resistance and improve patient outcome in myeloma.

Dr. Anderson’s team led both preclinical and clinical trials of the novel proteasome inhibitor bortezomib, as well as the immunomodulatory drug lenalidomide, culminating in the rapid FDA approval of these agents for treatment of myeloma. His paradigm for identifying and validating targets in the tumor cell and its milieu has already provided novel therapies which have transformed myeloma therapy, and offers great promise to improve patient outcomes in hematologic malignancies and solid tumors as well.
Biographies

Co-Chairs

Susan L. Kelley, MD
Multiple Myeloma Research Foundation
Multiple Myeloma Research Consortium

Susan L. Kelley, MD, is Chief Medical Officer of the Multiple Myeloma Research Foundation (MMRF) and the Multiple Myeloma Research Consortium (MMRC), where she provides senior medical leadership for the clinical development activities supported by the MMRC.

Dr. Kelley has more than 20 years of experience in oncology clinical research and drug development within the pharmaceutical industry. Dr. Kelley most recently served as Vice President of Global Clinical Development for Oncology at Bayer Healthcare Pharmaceuticals, where she was responsible for the management of the oncology drug development portfolio and for the supervision of Bayer’s oncology physicians and clinical project managers. While at Bayer, she oversaw the successful advancement of Nexavar® (sorafenib) through the FDA approval process. Prior to her position at Bayer, Dr. Kelley held several leadership positions in Bristol-Myers Squibb’s (BMS) Oncology Therapeutic Area. While at BMS, she participated in the development and registration of several new drug products, including Vumon® (teniposide), Taxol® (paclitaxel), and Videx® (didanosine); she also supervised clinical development programs for a cancer vaccine for melanoma and numerous phase I and II clinical trials.

Dr. Kelley received her medical degree from Duke University School of Medicine. She completed certification in internal medicine at the University of Colorado followed by medical oncology training at the Dana-Farber Cancer Institute in Boston.
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Richard Pazdur, MD, FACP, is presently the Director of the Office of Oncology Drug Products in the Center for Drug Evaluation and Research of the U.S. Food and Drug Administration (FDA). This Office was formed in 2005 to consolidate the review of drugs and therapeutic biologics for the diagnosis, treatment, and prevention of cancer, as well as the review of drugs and therapeutic biologics for hematologic diseases and for medical imaging. Dr. Pazdur’s position facilitates coordination of oncology activities across all FDA Centers and ensures an ongoing outreach and collaboration between the FDA, the National Cancer Institute, and other cancer-related organizations within and outside of the government. Dr. Pazdur was the Director of the Division of Oncology Drug Products from September 1999 to May 2005.

Prior to joining the FDA, Dr. Pazdur was Professor of Medicine at The University of Texas, MD Anderson Cancer Center in Houston, TX. Dr. Pazdur was on the faculty of the MD Anderson Cancer Center from 1988 to 1999. During his tenure at the MD Anderson Cancer Center, Dr. Pazdur held administrative positions of Assistant Vice President for Academic Affairs, Associate Director of Clinical Trials Administration (Division of Medicine), and Director of Educational Programs (Division of Medicine).

Dr. Pazdur’s main research interests are in clinical trial design and drug development of anticancer agents in advanced colorectal cancer. He has performed numerous phase I, II, III, and adjuvant therapy trials in this disease. Dr. Pazdur has published over 300 articles, book chapters, and abstracts. Dr. Pazdur was on the faculty of Wayne State University in Detroit, MI, from 1982 to 1988. He received his undergraduate degree from Northwestern University in Evanston, IL, his MD degree from Loyola Stritch School of Medicine in Maywood, IL, and completed clinical training at Rush-Presbyterian St. Luke’s Medical Center in Chicago, IL, and the University of Chicago Hospitals and Clinics.
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Co-Chairs

Louise M. Perkins, PhD
Multiple Myeloma Research Foundation

Louise M. Perkins, PhD, is the Chief Scientific Officer at the MMRF, where she is responsible for the strategic development and execution of the MMRF’s research agenda. Dr. Perkins brings more than 16 years of pharmaceutical research experience from 2 major companies to the MMRF.

Prior to joining the MMRF, she was the Director of Cancer Research at Bayer Pharmaceuticals in West Haven, CT, where she contributed to advancing novel targeted therapies toward clinical study, including Nexavar® and other innovative signal transduction inhibitors. While at Bayer, she also served as the Director of Research Licensing and was responsible for oncology licensing activities in support of cancer research programs. Prior to joining Bayer, she led a cancer research group at the Schering-Plough Research Institute in Kenilworth, NJ. In this role, she participated in several early-stage research programs including novel target-finding research using human genomics data.

Dr. Perkins graduated from the University of Michigan with a PhD and MS in biological chemistry and conducted postdoctoral studies at Princeton University in the Department of Molecular Biology. She earned her BS in zoology from the University of North Carolina at Chapel Hill.
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Faculty

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Donald A. Berry, PhD, is an international expert in the field of biostatistics. He holds the Frank T. McGraw Memorial Chair for Cancer Research at The University of Texas, MD Anderson Cancer Center, where he is Head of the Division of Quantitative Sciences and Chair of the Department of Biostatistics. His primary interest is the prevention and treatment of breast cancer. He serves as the faculty statistician on the Breast Cancer Committee of the Cancer and Leukemia Group B (CALGB), a national oncology group. In this role, he designs and supervises the conduct and analysis of clinical trials in breast cancer.

A native of Massachusetts, Dr. Berry received his PhD in statistics from Yale University, and previously served on the faculty at the University of Minnesota and at Duke University, where he held the Edger Thompson Professorship in the College of Arts and Sciences.

The author of more than 200 published articles and several books on the use of biostatistics in medical research, Dr. Berry has been the principal investigator for numerous medical research programs funded by the National Institutes of Health and the National Science Foundation. He was also the principal investigator of an NCI project CISNET: Cancer Intervention and Surveillance Network. This project focused on statistical modeling to assess the relative contribution of screening mammography, tamoxifen, and chemotherapy to the drop in breast cancer mortality observed in the United States since 1990. Another focus of Dr. Berry’s statistical research is designing clinical trials that utilize patients more efficiently and that treat patients in the trials more effectively.

Dr. Berry is a statistics editor for the Journal of the National Cancer Institute and associate editor for Breast Cancer Research and Treatment and also for Clinical Cancer Research. He is a fellow of the American Statistical Association and of the Institute of Mathematical Statistics.
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Faculty

Ann T. Farrell, MD
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Ann T. Farrell, MD, is a Deputy Director in the Division of Drug Oncology Products in the Center for Drug Evaluation and Research (CDER) at the U.S. Food and Drug Administration (FDA). She currently serves as a co-chair for the chemoprevention subcommittee of the IntraAgency Oncology Task Force. Prior to her FDA career, Dr. Farrell was an Assistant Professor of Medicine in the Division of Hematology/Oncology at the University of Massachusetts in Worcester, MA. Dr. Farrell received her medical degree from Rush Medical College in Chicago, IL.

She completed her internship and residency at a Yale-affiliated program, the Hospital of St. Raphael in New Haven, CT. Dr. Farrell completed her hematology/oncology fellowship at the University of Massachusetts. She is board certified/eligible in internal medicine, hematology, and medical oncology. She is a member of the American Society of Hematology and the American Society of Clinical Oncology. She has coauthored publications including journal articles and book chapters.


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Kathy Giusti
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Kathy Giusti is the Founder and Chief Executive Officer of the Multiple Myeloma Research Foundation (MMRF) and the Multiple Myeloma Research Consortium (MMRC). In 1998, following her diagnosis with multiple myeloma, she founded the MMRF to fund innovative myeloma research and drug discovery. Having raised more than $100 million to date, the MMRF is the world’s number one funder of myeloma research.

As an extension of the MMRF’s mission, she founded the MMRC in 2004 to enable leading research institutions to work within the industry to speed the discovery and development of effective new treatments. Comprising 15 leading research institutions, the MMRC is widely recognized for breaking down barriers in tissue banking, data management, genomics, and clinical trials, and is considered an optimal research model to accelerate the development of new therapies.

Ms. Giusti received her MBA in general management from Harvard Business School and graduated from the University of Vermont magna cum laude with a BS in biological sciences. She began her career in 1980 with Merck & Co., and later joined the Gillette Company. In 1993, she joined G.D. Searle & Co., where she last served as Executive Director of Searle’s worldwide arthritis franchise.

Ms. Giusti received the 1998 Healthcare Businesswomen’s Association’s Woman of the Year Award, the 2001 Harvard Business School Entrepreneurial Award, the 2002 McCarty Cancer Foundation Humanitarian Award, the 2002 Joseph Michaela Award from the Weill Medical College of Cornell University, the 2005 Harvard Business School Award for Courage and Valor, and the 2006 Partners in Progress Award from the American Society of Clinical Oncology. She has served on the Institute of Medicine’s National Cancer Policy Board and the Cancer Leadership Council. Today, she continues to serve on the National Cancer Advisory Board, an appointment she received in 2003 from President Bush, and is a member of the Board of Directors for IMS Health as well as the Health Research Alliance. Most recently, her efforts to advance cancer research and drug development have been featured in The Wall Street Journal, “NBC Nightly News,” “CBS Evening News,” and The New Yorker.

She lives in New Canaan, CT, with her husband, Paul, and her children, Nicole, who is 13, and David, who is 11.
Biographies

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Multiple Myeloma Research Foundation

Joan Levy, PhD, is the Associate Director of Research at the MMRF, where she is responsible for the planning and implementation of its research agenda. Dr. Levy has more than 20 years of experience in oncology and osteoporosis research in both pharmaceutical and academic settings.

Before joining the MMRF, Dr. Levy was Technical Director at The Binding Site, supporting use of Freelite™, a diagnostic assay for multiple myeloma. In addition, she worked at Bayer Pharmaceuticals Corporation in West Haven, CT, for 9 years. In that capacity, she was engaged in target identification, compound validation, and project leadership in the fields of cancer and osteoporosis research. She has contributed to the delivery of novel drug candidates for clinical studies in each of these indications.

Dr. Levy earned her PhD at the University of Vermont and then completed 2 post-doctoral fellowships at the Rockefeller University and later at the State University of New York at Stonybrook. Her 2 academic appointments were at New York University Medical Center and Yale University, studying various aspects of cell signaling pathways involved in the pathogenesis of cancer and development of osteoporosis.

Anne Quinn Young, MPH
Multiple Myeloma Research Foundation

Anne Quinn Young is the Program Director at the MMRF, where she oversees all communications, as well as educational programming and outreach to healthcare professionals and patients. Anne currently serves as the MMRF’s representative on the Cancer Leadership Council (CLC) and as principal investigator on a major grant from the Centers for Disease Control and Prevention (CDC).

Prior to joining the MMRF, Anne was a consultant in the healthcare practice of Datamonitor, a global market research and business intelligence company. Anne previously worked in healthcare public relations at Burson-Marsteller and Chandler Chicco Agency, following a post-graduate internship at the Department of Justice, Antitrust Division.

Anne has a Master of Public Health from the Mailman School of Public Health of Columbia University and graduated cum laude from Dartmouth College with a BA in government.
Biographies

MMRF and MMRC Staff

**Sandra M. Wear, RN**
Multiple Myeloma Research Consortium

Sandra M. Wear, RN, is the Associate Director of Operations of the MMRC where she is responsible for the execution of preclinical and clinical projects within the consortium. She brings more than 27 years of experience to the MMRC as both an oncology nurse and a senior manager and has vast experience from both an industry and a contract research organization perspective.

She most recently served as Vice President of Regional Monitoring Operations at Endpoint Research in Mississauga, Ontario, Canada, and Senior Manager of Clinical Process and Training and Trials Management at MannKind Biopharmaceutical Corporation in Danbury, CT. Before this, she served as Associate Director of Clinical Operations at Purdue Pharma, L.P., in Stamford, CT; Senior Project Manager at Quintiles Transnational Corporation in Mountain View, CA; and Clinical Research Manager at Pharmacia & Upjohn, Inc., in Don Mills, Ontario, Canada.

She received her nursing degree from Mohawk Nursing College in Hamilton, Ontario, Canada.
Abstracts

Orientation – Recent Approvals and Future Challenges in Myeloma Drug Development

Kenneth C. Anderson, MD
Dana-Farber Cancer Institute
Kraft Family Professor of Medicine
Harvard Medical School

Only a minority of preclinical drug candidates translate from the bench to the bedside and U.S. Food and Drug Administration (FDA) approval at high cost and long intervals. In myeloma, however, bortezomib, thalidomide, and dexamethasone; lenalidomide and dexamethasone; and bortezomib with pegylated liposomal doxorubicin have all been FDA approved since 2006. These approvals reflect a partnership between academia, pharmaceutical companies, the FDA, the National Cancer Institute, and advocacy groups that has markedly fast forwarded this process.

Previous workshops on drug development in myeloma have focused upon the importance of preclinical models, biomarkers, response criteria, partnerships, and correlative science; phase I–III clinical trial design; and appropriate clinical trial design in the context of different stages of myeloma. In the setting of this remarkable progress, myeloma remains an attractive area for pursuing approval of novel targeted therapies due to the effective use of oncogenomics to define novel targets in the tumor cell; and (2) the established paradigm of drug development illustrated by thalidomide, lenalidomide, and bortezomib, all of which rapidly moved from preclinical evidence of efficacy against tumor cells in the bone marrow milieu to derived phase I–III clinical trials and approval. Moreover, the recent approval of bortezomib as first-line therapy confirms the ability in myeloma to rapidly extend approval from advanced to newly diagnosed disease.

Although relapsed/refractory myeloma represents an unmet medical need, patients will now have received novel therapies including bortezomib and immunomodulatory drugs, and it is therefore unlikely that any single agent will overcome resistance. Therefore, future directions will not only include the evaluation of novel single agents, such as the proteasome inhibitors NPI-0052 and carfilzomib, but also combination clinical trials directed to enhance efficacy, overcome drug resistance, and minimize attendant toxicity. Although this presents new challenges, preclinical studies in myeloma can inform the design of clinical trials.
Abstracts

Orientation – Recent Approvals and Future Challenges in Myeloma Drug Development

Kenneth C. Anderson, MD
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Already the combination of bortezomib and pegylated liposomal doxorubicin has been approved. Phase III clinical trials are ongoing which compare the combinations of bortezomib with a heat shock protein 90 inhibitor; the Akt inhibitor perifosine; or the histone deacetylase inhibitor vorinostat versus bortezomib alone. These trials are all predicated upon promise in preclinical studies. Most exciting, preclinical studies informed the design of clinical trials of the bortezomib and lenalidomide combination, which has achieved unprecedented extent and frequency of response in patients with relapsed/refractory myeloma, and most recently in patients with newly diagnosed disease. The major challenge is to rationally design and rapidly perform high quality effective combination trials to expedite FDA approval. Combination therapies have proven to be curative in childhood acute lymphocytic leukemia, Hodgkin lymphoma, and testicular cancer, and the stage is now set to rapidly identify effective combination therapies in myeloma as well.

References
Abstracts

Adaptive Designs for Cancer Trials

Donald A. Berry, PhD
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Adaptive designs are being increasingly used in drug and medical device trials. An adaptive design is one in which the course of the trial is determined by the results observed so far in the trial. Examples of adaptations include early stopping, extending accruing when the answer is not yet clear, varying randomization proportions to maximize information (such as information about dose effects) or to maximize effective treatment of patients in the trial, dropping treatment arms seamlessly from one trial phase to another, and focusing on responding subsets of patients. I will describe the use of adaptive designs in medical research, with emphasis on cancer drug development, and provide some examples. One example is a biomarker-driven trial—more a "process"—designed to determine which patients respond to which treatment regimens, including combinations of experimental agents.
Abstracts

FDA Overview and Perspectives – Lessons Learned in Oncology

Ann T. Farrell, MD
U.S. Food and Drug Administration
Notes