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# MYELOMA FOCUS

Newsletter of the

# MMRF

## MMRF Chicago Event Raises Over \$1 Million

The MMRF was blown over in the windy city by the incredible support of Chicagoans and special guests who turned out March 4, 2003 at the Four Seasons Hotel, Chicago. That evening, over \$1 million was raised in support of myeloma research, surpassing everyone's expectations.



Bob Costas and Kathy Giusti

The evening's highlights included the presentation of the **Ann Landers Research Fund Award** to Northwestern University researcher Seema Singhal, MD, Director of the Myeloma Program at Northwestern's Feinberg School of Medicine. Dr. Singhal was selected for her investigation into novel therapies, including her exploration of gene function in myeloma. The two-year grant is funded by a \$100,000 contribution from the Chicago Tribune Foundation and a matching \$100,000 gift from Ann Landers' daughter, Margo Howard and her husband Dr. Ronald Weintraub. Scott Smith, CEO, Tribune Co., along with Margo Howard, presented the award to Dr. Singhal.

In addition, Kathy Giusti presented NBC Sportscaster Bob Costas, with the **MMRF Public Awareness Award** for his extraordinary work on behalf of cancer patients and his support of the MMRF. The Bob Costas Cancer Center at Cardinal Glennon Children's Hospital, in his hometown of St. Louis, MO, is a comprehensive program dedicated to the diagnosis and treatment of childhood cancers and other blood and immune disorders.

Olympic gold medalist and speed skating champion Dan Jansen served as the evening's master of ceremonies, and the event was chaired by Lester Knight, CEO of RoundTable Healthcare Partners. Lester's close friend, colleague and myeloma patient



Event Chairman Lester Knight and his wife Becky



Bob DeBaun, inspired the support of many business and healthcare leaders in the Chicago area. Nine dinner chairs helped to bring over 600 attendees to a very successful evening marked by the raising of more than \$1 million toward accelerating the search for a cure.

cont. page 3

(L) MMRF Board Member Dana LaForge, (R) Dan Jansen with wife Karen

# Welcome Letter

**D**ear Friends,

One evening shortly after he was diagnosed, Robert (Bob) DeBaun called for information and advice. I was happy to give him as much information and personal support as I could. Little did I know then just how many people in Chicago, his hometown, would want to do the same.

I am continually amazed at how much of an impact one person can make. Bob made an impact in the city of Chicago long before his diagnosis with multiple myeloma, and the city responded in a swelling of support on March 4, 2003 at the MMRF Chicago Awards Dinner when over \$1 million was raised in support of myeloma research. It was a true testament to Bob, to the people of Chicago and to the profound difference a single person can make.

First among those people was Lester Knight, Bob's close friend, co-founder of RoundTable Healthcare Partners, and chair of the evening. As the event chairman, his goal was simple: "to raise as much awareness and money for multiple myeloma research as possible." With help from Dan Jansen,



Bob and Jane DeBaun with their son Blake and daughter Liz

Bob Costas, the Chicago Tribune and Margo Howard, the evening surpassed all of our expectations, and was further proof of just how valuable the gift of friendship and the bonds formed around one patient can be. It showed that there is no better gift a friend can give than their heartfelt support.

On behalf of the MMRF, it is my honor to thank Bob and all those who have supported him in friendship, in life and in the search for a cure.

*Kathy Giusti*

## Families Funding Research

**W**hen a family member was diagnosed with multiple myeloma, Christine Cardillo, an experienced event planner, knew that organizing a fundraiser in support of research would be one of the best ways to put her love and her talents to use for her family and for patients everywhere. After learning more about the foundation, she knew that the MMRF is leading the charge in support of myeloma research. "I was so impressed by the work they were doing and was truly inspired by Kathy Giusti's efforts and drive."

This contagious enthusiasm helped turn Christine's desire to help into the big success of The First Annual **Friends Together Gala**, held March 1, 2003 at the

Mystic Marriott in Groton, CT. With help from her sister and several close friends and supporters, the event raised \$50,000 and showed that bringing friends together can make a big difference.

Mel Goldstein, friend, myeloma patient and New Haven, CT, Channel 8 Meteorologist, received the MMRF Public Awareness Award. Known as "Dr. Mel" to hundreds of thousands of televi-

sion viewers, radio listeners and newspaper readers, he has been an important spokesperson on behalf of myeloma patients.

Honored with the MMRF Spirit of Hope Award, was friend Fran Delaney, a 35-year veteran of the US Postal Service. Fran's successful stem cell treatments have helped him to be virtually symptom free and as Christine put it, "such an inspiration to my family."



(L-R) Arlene and Dr. Mel Goldstein, Kathy and Fran Delaney, Kelly Goulet, Dr. John Foley, Marie Kenny, Christine and Mario Cardillo

Christine is an inspiration to us all by having brought friends together, and sparking enough enthusiasm to have them looking forward to next year.



# MMRF Laugh For The Cure

## Raises Over \$70,000

The MMRF held its first **MMRF Laugh for the Cure** Event on Thursday, April 10, at Stand Up NY Comedy Club in New York City. Sponsored by Pepsi and co-chaired by Jo Cavallo and Sue Korn. More than 200 guests enjoyed an evening of laughter with performances from some of New York's finest up and coming comedians. All event proceeds will support one of the MMRF's 2003 Senior Research Awards in honor of the event. If you are interested in conducting a **MMRF Laugh for the Cure** in your city please contact Scott Santarella at [santarellas@themmrf.org](mailto:santarellas@themmrf.org).



Laugh Committee (L-R) Cindi Stern, Laurie Goldschein, Sondra Goldschein, Joan McLachlin, Jo Cavallo, Scott Santarella, Sue Korn, Heidi Sandreuter, David Stern and Steve Goldschein. Missing from the photo: Carol Goldschein



The MMRF Executive Director, Scott Santarella presents MMRF's Plaque of Recognition to Steve Goldschein for his family's support of the MMRF's research efforts

## The MMRF Chicago Dinner (cont.)



The MMRF's guests, Joe Hogan, CEO of GE Medical Systems and his wife Lisa with the MMRF Public Awareness Award winner, Bob Costas.



(L-R) Dean and Kimberly Assink, Dr. Ken Anderson, Dr. John Lust and Kate and Bill Schuett. The Assinks and Schuett have been long-time supporters of the MMRF Research Awards and were given the honors of making the evenings presentations to Dr. Anderson of Dana-Farber and to Dr. Lust of the Mayo Clinic.

# MMRF RACE FOR RESEARCH 5K Walk/Run

## Second Annual San Francisco Race for Research Raises Over \$200,000

The second annual **MMRF Race for Research** - San Francisco, held on April 13th at Crissy Field, was another enormous success. Over 750 walkers and runners braved the storms and rain to come out in support of the MMRF. In addition to raising a great deal of awareness and having a great time, event participants and sponsors raised more than \$200,000 for myeloma research.

A very special thank you goes out to our flagship sponsors, Genentech, Inc. and Wilson Meany Sullivan, as well as to our event chairmen, Dr. Mark Ahn, Genentech, Vice President, Hematology and MMRF Board Member, William Wilson III.



Walkers and runners brave the weather as they gather to get ready to raise funds for research.

Visit the MMRF's website at [www.multiplemyeloma.org](http://www.multiplemyeloma.org) for upcoming Race for Research 5K Walk/Run event dates:  
July 19, 2003 ☞ Seattle  
September 14, 2003 ☞ Chicago  
October 5, 2003 ☞ Atlanta  
November 16, 2003 ☞ Washington, DC

### Please Pardon Our Error

In the Winter 2002 edition of Myeloma Focus, Alice and Jeffrey Oberlander (Platinum Level donors) were mistakenly listed as Van Der Moolen Specialists USA. Also, Mr. and Mrs. Alvin Dischler (Platinum Level donors) were mistakenly omitted. We apologize for our error and thank you for your support.



NBA Legends and Honorary Starters, Al Attles and Nate Thurmond enjoy the race with Committee Chairs, Beth Compton and Ron Anker

The MMRF would also like to thank the following special companies and individuals who made the event the success that it was: Bay Area Multiple Myeloma Support Group, the Compton Family, the Heydlauff Family, Ron Anker, Jack Aiello, Netta Banks, John Newton, Mary Azevedo, Renee Adams, Theresa Nicoletto, Susan Hopkins, TEAM DAN, the Briggs Family and KNBR Radio.

We would also like to acknowledge our many wonderful sponsors who played an integral role in our financial success: Miller Ream, Webcor Builders, Farella Braun & Martell, Embarcadero Capital Partners, Seiler and Company, Robert Mondavi Wineries, The CAC Group, Mike Podell, Dennis Wong, Betsy & Mark Gates, Capital Guardian Trust and Dr. Clyde Burch. Special kudos also go out to Nate Thurmond and Al Attles, two legendary National Basketball Association (NBA) figures who came out to serve as our honorary starters.

A wonderful collaborative effort from a dedicated and hard working community. Thank you all!!



Scott Santarella, William Wilson and Nate Thurmond

# NYC MMRF AWARDS DINNER

## Ortho Biotech and Dr. Sundar Jagannath To Be Honored At NYC Fundraiser

**C**ompassion and Caring will be the theme as the MMRF returns to New York City for its third annual summer fundraiser, an evening honoring the people that are making some of the most heartfelt differences in the lives of myeloma patients.

The event will take place at New York's St. Regis Hotel on Monday, June 9th and will again be hosted by longtime friends of the MMRF, and compassionate advocates for all patients, Mel Stottlemire, Pitching Coach of the NY Yankees, and his wife Jean. Geraldine Ferraro, Dick Grasso, CEO of the NYSE and Ed Randall, Host of New Century Talking Baseball, will serve as the evening's Honorary Chairs.

Ann Curry, NBC anchor, and longtime MMRF friend, will serve as the evening's special guest. As a former MMRF honoree and a daughter who experienced her mother's battle with cancer, Ann has demonstrated time and again her relentless compassion and dedication to the cause of helping patients.



Ann Curry

Carol Webb, Group Chairman of Johnson & Johnson, will accept the **MMRF Corporate Leadership Award** on behalf of Ortho Biotech, a subsidiary of Johnson & Johnson. The MMRF is honoring Ortho Biotech for their leadership in supporting cancer education and research; their ongoing commitment to improving the quality of life of cancer patients worldwide; and their dedication to helping families cope with the emotional and physical challenges of caring for a loved one with cancer through their Strength for Caring Program. Also, Dr. Sundar Jagannath, Chief of the Multiple Myeloma Center at St. Vincent's Cancer Center, will be honored for his role as a champion of novel therapies for myeloma patients and his compassionate care of so many patients and families.

Whether in their own personal battles with cancer or in their commitment to help so many others, each of these people has demonstrated their compassion and caring in extraordinary ways. We look forward to joining them in New York to honor their remarkable efforts.

It's not too late to get tickets and sponsorship information. Please contact Jenny McMahon at 203-972-1250 or [mcmahonj@themmrf.org](mailto:mcmahonj@themmrf.org)



Carol Webb,  
Group Chairman of  
Johnson & Johnson

Dr. Sundar Jagannath,  
Chief of the Multiple  
Myeloma Center at St.  
Vincent's Cancer Center



### Combined Federal Campaign Approval for the MMRF

**The Multiple Myeloma Research Foundation – No. 1048** – is approved by the Combined Federal Campaign (CFC) for the annual fundraising drive conducted by federal employees in their workplace. If you are a federal employee or have a friend or family member who works for the US



Government, you can support the MMRF by making a donation through the CFC.

# THE MMRF GRANTS \$300,000 IN FELLOWS AWARDS

The MMRF is pleased to announce the **2003 MMRF Fellows Award Winners**. These award winners were selected from among a large group of grant applicants in a highly competitive environment and emerged from the MMRF's stringent review process. These junior researchers will receive \$50,000 to conduct cutting edge research that helps us to better understand the disease and to develop novel therapies. The MMRF remains committed to its objective of funding the most promising research, and these grant awards are part of our \$5 million commitment toward funding the most promising research this year.



**David Dingli, MD**  
Mayo Clinic

**Preclinical toxicology and pharmacology of MV-NIS, a replicating measles virus for myeloma gene therapy**

Our laboratory has developed a virus that can preferentially infect myeloma cells and induce the expression of a normal thyroid protein that allows the cells to concentrate iodide isotopes. Myeloma tumors that are infected with the virus concentrate radio-iodine and are completely eradicated. We want to perform animal studies to evaluate the potential toxicity of this virus.



**Ramadevi Nimmanapalli, D.Phil**  
Moffitt Cancer Center

**The role of heat shock protein 70 (HSP70) on MM-stromal cell interactions**

This research proposal will investigate the mechanism by which heat shock protein 70 (HSP70) makes myeloma cells attach to bone marrow stromal cells and resist apoptosis, as well as the role of HSP70 in cancer cell growth and resistance to chemotherapy. Drugs that down-regulate HSP70 would potentially serve as suitable therapies especially when combined with other chemotherapy drugs.



**Esther Masih-Khan, PhD**  
Toronto General Research Institute

**The significance of the unfolded protein response pathway in multiple myeloma and its potential as a therapeutic target**

The specific aim of this study is to further understand the significance of a group of genes that is unregulated in multiple myeloma. We propose to achieve this aim by extending our analysis of 45 MM patients on 19000 more genes to describe new genes that may be expressed differently in diseased versus normal plasma cells. We also propose to study the potential of two known genes as potential therapeutic targets.



**Claire Shipman, BSc, PhD**  
University of Oxford

**The role of LIGHT/TNFSF14 and DcR3 in the pathophysiology of multiple myeloma**

The aim of this research is to determine the role of LIGHT, a newly discovered molecule that is decreased in patients with multiple myeloma, and an associate molecule, decoy receptor 3, in the growth of myeloma cells in the bone marrow and the development of myeloma bone disease. We anticipate that this will lead to an increased understanding of how myeloma cells grow and identify new ways to treat this disease.

# MMRF FELLOWS AWARDS CONT.



**Reshma  
Shringarpure , PhD  
Dana-Farber Cancer  
Institute**

**Identification of molec-  
ular markers that deter-  
mine PS-341 sensitivity  
(or resistance) in multi-  
ple myeloma**

Velcade (bortezomib/PS-341) has shown promising clinical activity in patients with relapsed refractory myeloma, but the precise mechanism of action has not been delineated. I will use two closely related cell lines, one extremely sensitive to PS-341 treatment and one very resistant, as models to determine the mechanisms of sensitivity/resistance, which will help to predict responders versus non-responders to treatment.



**Yuan Xiao Zhu , MD  
Toronto General  
Research Institute**

**The role of c-maf in mul-  
tiple myeloma**

The blood cancer myeloma is characterized by the high expression of cancer causing genes. One of them called c-maf has not been studied and the reason it promotes myeloma is unknown. We wish to define its role in this cancer and to develop drugs that will attack its myeloma promoting properties.



## MMRF CLINICAL TRIALS MONITOR

Join the more than 16,000 individuals who have visited the MMRF's Clinical Trials Monitor (CTM). CTM allows you to easily search for trials according to type of trial (e.g., novel drugs), disease stage and location. Trials are added and updated weekly, so check back often. Following are some of the new exciting trials that you can now find on CTM:

Phase III Multicenter, Parallel-Group, Controlled, Randomized, Double-Blind Study of Revimid "CC-5013" Plus Dexamethasone Versus Dexamethasone Alone in Previously Treated Subjects with Multiple Myeloma

Phase II Study of Arsenic Trioxide in Combination with Ascorbic Acid and Dexamethasone in Patients with Relapsed/Refractory Multiple Myeloma (TAD)

Phase II Study of Anakinra (IL-1 Receptor Antagonist) in Patients with Smoldering/Indolent Multiple Myeloma

Phase II Study of VAD (Vincristine, Adriamycin, Dexamethasone) plus Thalidomide (low dose) as frontline therapy for newly diagnosed patients with Multiple Myeloma (MM)

Phase III International, Multi-Center, Randomized, Open-Label Study of PS-341 (VELCADE™) versus High-dose Dexamethasone in Patients with Relapsed or Refractory Myeloma

If you would like to post a trial on CTM contact Anne Quinn Young at [quinnyounga@themmrf.org](mailto:quinnyounga@themmrf.org) or at 203-972-1250.  
See the MMRF's Clinical Trials Monitor [www.multiplemyeloma.org/clinical\\_trials](http://www.multiplemyeloma.org/clinical_trials)

# Patient/Caregiver Study

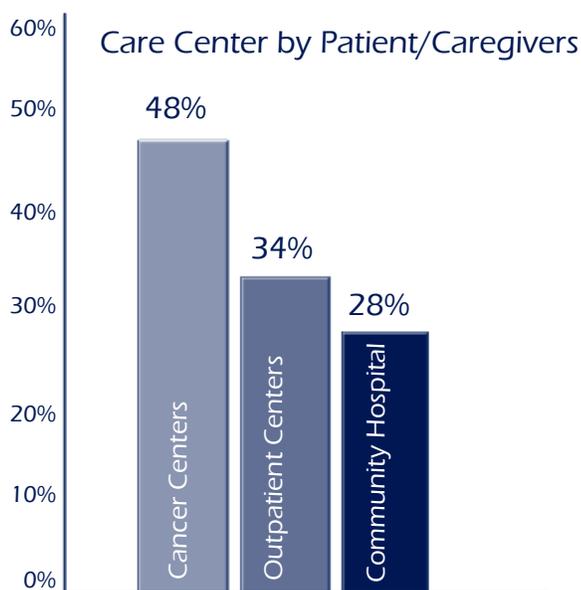
**P**atients and families are important to the MMRF. You are the reason that we work so hard to raise research funds, promote awareness and education and support scientific progress in the search to find a cure for multiple myeloma (MM). This is why it's so important for us to hear from you on a regular basis and to understand the challenges you face, knowledge you have and priorities you share.

Several months ago the Foundation contracted with an independent market research firm to conduct a detailed survey of myeloma patients and caregivers. This survey helped us identify patient priorities and needs for information. The survey results will help the MMRF to continue to make meaningful differences in the lives of those living with myeloma.

## Who Responded

Of a total of 9,800 questionnaires mailed to a random sampling of patients and caregivers from the MMRF database, a total of 3,045 surveys were completed. This was a remarkable 31% response rate.

More than two-thirds of the respondents were patients and about one-fourth were caregivers. Interestingly, 48% of the respondents reported receiving care at academic cancers, 34% at outpatient centers and 28% at community hospitals.

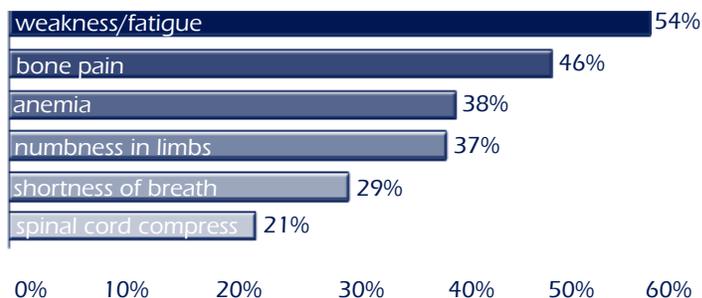


## Key Findings

We were pleased to confirm that the overwhelming majority of respondents were extremely knowledgeable of the basic realities of MM. Clear majorities answered correctly that scientists have not found a cure, that the disease occurs more frequently in the elderly, that MM cell growth can result in anemia and that MM is a cancer of the blood.

The most commonly reported symptoms among patients were weakness, fatigue, bone pain and anemia.

Which of the following symptoms are you/person you care for having the most difficulty with?



Respondents were less certain about being able to identify the stage of their disease and a low percentage indicated awareness of novel therapies in clinical trials. As would be expected, the majority of respondents reported familiarity with standard drug therapies. Lower numbers of patients were aware of new therapies (such as Velcade™ and Revimid™) and will likely become more informed as such new therapies enter phase III trials and get closer to becoming widely available.

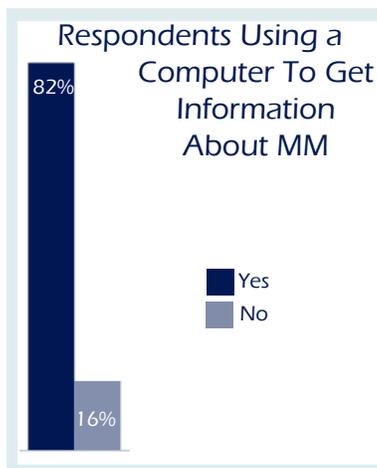
## Ranking of Familiarity With Myeloma Therapies:

- |              |               |
|--------------|---------------|
| 1. Thalomid® | 6. Neupogen®  |
| 2. Aredia®   | 7. Revimid™   |
| 3. Procrit®  | 8. Velcade™   |
| 4. Zometa®   | 9. Gleevec™   |
| 5. Alkeran™  | 10. Trisenox® |

# Patient/Caregiver Study

Still, we think that getting information about new therapies to patients as soon as possible is important, and we have taken steps to do just this. The MMRF recently redesigned and updated our website to include detailed information on new therapies. We have also recently updated our Multiple Myeloma Treatment Overview brochure to reflect the most current information, and we plan to continuously provide extensive new product information via our symposia and weekly SmartBrief email alerts.

doctors don't recommend them -- we are increasing physician awareness of CTM. CTM is updated continually to provide you with the most current information.



While many patients indicated that they use a computer to retrieve information about multiple myeloma, there is still a strong preference for printed materials. The MMRF takes this into account as we continue to provide regular mailings and printed informational materials to patients and their families.

We also learned that getting patients information about new therapies is only one part of the equation; information about how to access new therapies as part of a clinical trial is, as our survey determined, patients' top information request.

## Ranking of Items Important For The MMRF To Offer

1. Distribute brochures
2. Clinical trial information
3. Money for grants
4. Up-to-date website
5. Seminars for patients
6. Newsletter/SmartBrief
7. Seminars for healthcare
8. Educational teleconference
9. Webcasts and conferences
10. Referral service

We are proud that the survey confirmed the MMRF's standing as one of the first places patients and caregivers turn for information and guidance on MM. We are also proud to know that our most important mission -- accelerating the search for a cure through the funding of myeloma research -- is one of the most highly ranked of our activities.

Your understanding of the important role we play in funding research reaffirms what we see as our most important goal. This year alone we will fund \$5 million in research grants to scientists making promising discoveries in myeloma. Now, thanks to you, we know exactly how and what to provide to allow patients access to the best medical care.

## Preferences For Getting Information About MM

- |               |                 |
|---------------|-----------------|
| 1. Print      | 4. Face to face |
| 2. Email News | 5. Seminar      |
| 3. Web        |                 |

The MMRF has met this request with the launch of our Clinical Trials Monitor (CTM) in 2002. This online database allows patients to tailor their search of clinical trials to their disease status, geographic location and preferred trial types, linking them directly to doctors and research institutions available to provide more information and guidance. Since we now understand the oncologists' key role in getting patients enrolled -- the #1 reason patients don't participate in clinical trials is that their



**The MMRF Thanks**  
the following corporations for their support of Myeloma Focus



# MMRF ROUNDTABLES

## Molecular Genetics of Myeloma

**P**aris, France was the setting for the **MMRF Research Roundtable** held February 20-23rd. The roundtable brought together individual groups involved in myeloma genetics research to discuss current knowledge and to identify directions for future study. Rafael Fonseca, MD (Mayo Clinic) and Herve Avet-Loiseau, MD (Institute of Biology, Nantes, France) chaired the roundtable.

The forum was highly interactive and facilitated open discussion among the participants. According to Dr. Fonseca, it is increasingly apparent that myeloma is not a single disease entity, but rather one composed of different subtypes best identified by specific genetic abnormalities. For this reason, genetic testing is becoming a more important tool in determining clin-



(L-R) Drs. Jesus Hernandez, Leif Bergsagel and John Shaughnessy

ical implications such as prognosis. For example, loss of chromosome 13 and others, and certain translocations (switching of chromosome segments), such as t(4;14) and t(14;16), may be associated with a more aggressive disease. In contrast, the presence of extra chromosomes and t(11;14) may offer improved survival.

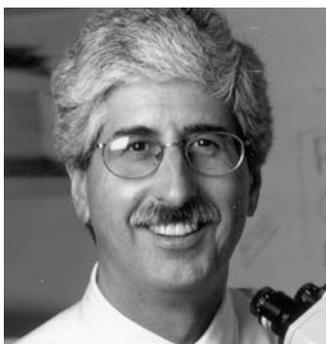
Future studies will help us refine our knowledge with regard to the clinical implication of these genetic abnormalities, particularly how they affect patient response to treatment. Forums such as this roundtable will also help us integrate the information obtained from the various genetic testing techniques being used today and help us develop genetic testing recommendations for patients with myeloma.

## Mouse Models in Myeloma

**T**he first ever workshop on mouse models in myeloma was held March 12-13 at the National Cancer Institute in Bethesda, MD. Formally titled "The Bone Marrow and Plasma Cell Tumor Development Workshop," the program brought together international experts in various specialties in order to help develop mouse models of myeloma that more accurately mimic human disease. These models help us understand what happens in the body when myeloma develops and provide tools for testing new therapies for efficacy and safety before they are tested in humans. The MMRF provided funding for the program, which was chaired by Brian Van Ness, PhD (Univ. Minnesota), and Michael Potter, MD (NCI).

According to Dr. Van Ness, one goal of the workshop was to identify the strengths and lim-

itations of current mouse models. In addition, experts in immune function and bone marrow biology were invited to help identify better approaches to develop models that mimic what happens in the bone in myeloma disease.



Brian Van Ness, PhD

Some of these new mouse models are already being used to measure the effect of novel therapies -- such as the IMiDs<sup>®</sup>, bisphosphonates and proteasome inhibitors -- on both tumor cell burden and bone disease. Mouse models are also being developed that mimic the genetic abnormalities in human myeloma, allowing us to determine how these defects affect response to treatment. The dialog initiated by the workshop participants set the stage for continued collaborative and coordinated approaches in modeling myeloma disease.



# MMRF INSTITUTIONAL INSIGHTS

# DATES TO REMEMBER

**F**rom Miami to Cleveland and on to Boston, the **MMRF Institutional Insights** symposia proved to be great successes this year by bringing over 600 patients and professionals the latest cutting-edge research and treatment information on multiple myeloma from the experts themselves. The MMRF kicked off the 2003 calendar at the University of Miami School of Medicine in Miami, FL on February 6-7th. Dr. Kelvin Lee chaired the program, while guest faculty Drs. Nizar Bahlis, Donna Weber, David Vesole and Keith Stewart presented on disease biology and staging, contemporary therapies, stem cell transplantation, novel therapies and current clinical trials. The MMRF was honored to dedicate this program to the family of Howard Tamlyn who contributed to the symposium. We would also like to thank Robert and Cindy Feltzin for their help in gathering volunteers to make the program registration process run smoothly.



Dr. Donna Weber (left) with guest



Dr. Keith Stewart (center) with symposium guests

At the Cleveland Clinic Foundation, Dr. Mohamad Hussein chaired the program on March 25th with excellent presentations given by Drs. Philip Greipp, Morie Gertz, Nikhil Munshi and Isador Lieberman. Faculty, staff and attendees were impressed with the many thought-provoking questions from the audience. Thank you Jim Bond and many other patients for sharing your stories publicly; you are true heroes and inspiration to us all.

The Dana-Farber/Partners Cancer-Care joined the MMRF in sponsoring the symposium in Boston, MA, April 2nd and 3rd. Dr. Kenneth Anderson chaired the program. Doctors James Berenson, William Bensinger, Donna Weber and Paul Richardson presented and piloted a new course format that included an audience response system (ARS) and case study discussions with the lectures based on the attendees' comments on the evaluation forms. The new MMRF ARS format was a success.

The MMRF would like to thank all of the speakers for their time dedicated to and in support of our programs. If you wish to receive the handouts from any of these programs, please contact Sara Nichols at [nicholss@themmrf.org](mailto:nicholss@themmrf.org) or call at 203-972-1250.



Dr. Paul Richardson (L) with symposium guests

**June 5, 2003**

**Teleconference**

Update from the IX<sup>th</sup> International Workshop on Multiple Myeloma

**June 9, 2003**

**New York, NY**

New York City Awards Dinner. Contact Jenny McMahon at 203-972-1250

**June 17-18, 2003**

**Washington, DC**

Blood Cancer Advocacy Days  
For more information contact Anne Quinn Young at 203-972-1250

**June 20, 2003**

**New York, NY**

\*Institutional Insights on Myeloma

**July 19, 2003**

**Seattle, WA**

MMRF Race for Research 5K Walk/Run

**Sept 14, 2003**

**Chicago, IL**

MMRF Race for Research 5K Walk/Run

**Oct 2-3, 2003**

**Toronto, ON Canada**

\*Institutional Insights on Myeloma

**Oct 25, 2003**

**Greenwich, CT**

MMRF Friends for Life Fall Gala

**Oct 5, 2003**

**Atlanta, GA**

MMRF Race for Research 5K Walk/Run

**Nov 5 & 6, 2003**

**Chicago, IL**

\*Institutional Insights on Myeloma

**Nov 13-14, 2003**

**Tampa, FL**

\*Institutional Insights on Myeloma

**Nov 16, 2003**

**Washington, DC**

MMRF Race for Research 5K Walk/Run

\*For more information on MMRF Institutional Insights call (203) 972-1250 or at [www.multiplemyeloma.org/events](http://www.multiplemyeloma.org/events)



# ASK THE EXPERT



Kelvin Lee, MD

This issue's **Ask the Expert** features Kelvin Lee, MD, Associate Professor of Medicine at the University of Miami.

## 1. Dr. Lee, where does arsenic trioxide fit in the grand scheme of myeloma treatment?

Arsenic trioxide (Trisenox<sup>®</sup>, Cell Therapeutics, Inc.) is in clinical testing in patients with myeloma who have failed other standard therapies and thus have refractory

or relapsed disease. The agent appears to have good efficacy against myeloma that is resistant to other therapies, including thalidomide, so it may be an option for the treatment of resistant disease. Arsenic trioxide is often used in combination with ascorbic acid (vitamin C), which is thought to potentiate its effectiveness and reduce toxicity. The agent is already approved for use in acute promyelocytic leukemia (APL).

Arsenic trioxide is currently being evaluated in several multicenter myeloma trials as part of combination therapy (see box below) and investigators are still identifying the best way to use the drug with regard to dosing and scheduling. Preliminary data suggests that arsenic trioxide may act synergistically with melphalan or dexamethasone, resulting in improved efficacy over either agent alone. There is the potential for arsenic trioxide to be tested in patients with earlier stage disease.

### Ongoing Trisenox Clinical Trials

- ☞ In combination with dexamethasone
- ☞ In combination with ascorbic acid and dexamethasone (TAD)
- ☞ Administered twice weekly and in combination with high-dose dexamethasone
- ☞ In combination with thalidomide
- ☞ As maintenance therapy following high-dose chemotherapy and stem cell transplant

See the MMRF's Clinical Trials Monitor [www.multiplemyeloma.org/clinical\\_trials](http://www.multiplemyeloma.org/clinical_trials) for more information

## 2. What are the side effects of arsenic trioxide therapy?

Generally the combination of arsenic trioxide and ascorbic acid is well tolerated. It has a slower onset of action than some chemotherapy regimens, with less abrupt and severe side effects. These side effects include nausea, a slight decrease in white blood cell counts, dry skin, dry mouth, a metallic taste, and occasionally, fluid retention and fatigue. Some patients experience transient peripheral neuropathy (numbness or tingling in the hands or feet) during therapy. However, many of these patients have pre-existing neuropathy. Although some heartbeat irregularities have been reported in APL patients who have received the agent, we have not seen any cardiac problems in patients with myeloma.

## 3. I have been on Aredia<sup>®</sup> for some time and recently started receiving Zometa<sup>®</sup>. Are there any risks in long-term bisphosphonate therapy?

Bisphosphonates such as Aredia (pamidronate, Novartis) and Zometa (zoledronic acid, Novartis) are effective in preventing skeletal events and maintaining quality of life in patients with myeloma. Thus, bisphosphonates should be continued as long as they provide benefit. Although renal (kidney) toxicity can occur with these agents, the risk is minimized if the recommended dose is administered at the appropriate infusion rate (see box below) and patients remain properly hydrated. It appears that if renal toxicity does not develop early on, it is not likely to develop later unless a patient's clinical situation changes in such a way that their risk for toxicity is increased (for example, they start taking other drugs that can damage the kidneys, or if disease progresses). Patients' serum creatinine levels and urine albumin (indicators of renal function) should be determined prior to each treatment as well as every 3 to 6 months during therapy to monitor for any changes in kidney function. In addition, patients should notify their doctor if they notice any change in their normal urination patterns after receiving bisphosphonate therapy.

### Recommended Bisphosphonate Administration

- ☞ 90 mg of Aredia in 250 ml of solution given over at least 2 hours, or
- ☞ 4 mg of Zometa in 100 ml of solution given over at least 15 minutes
- ☞ Both given every 3 to 4 weeks

# ADVOCACY DAY 2003

## Calling for Myeloma Advocates

Please join us in Washington, DC for the third annual **Blood Cancer Advocacy Day** event on June 17th and 18th. The MMRF and the Lymphoma Research Foundation (LRF) are once again teaming up in a united front for blood cancer patients to advance blood cancer research and ensure access to critical treatments.

This year's event will build on last year's successes. In 2002 over 300 blood cancer advocates made lobbying visits to Senators and Representatives from over 30 states, and Congress approved and President Bush signed the Hematological Cancer Research Investment and Education Act of 2002, sponsored by Senator Kay Bailey Hutchison (R-TX) and Senator Barbara Mikulski (D-MD).



Returning from Advocacy Day 2002 (L-R) Kathy Giusti, Allan Bailey, Senator Kay Bailey Hutchison and Geraldine Ferraro

This year determined advocates will once again storm Capitol Hill. The objectives are to ensure that adequate funding is provided for the Hematological Cancer Research Investment and Education Act, including funding for the expansion and coordination of blood cancer research programs at the National Cancer Institute, and the creation of a related education program for patients and the general public (the Geraldine Ferraro Cancer Education Program).



Aimee Swartz, Bruce Holmberg, MMRF Exec. Dir. Scott Santarella, David Spade, Carol Goldschein and Sue Korn

Advocates will lobby for passage of the Access to Cancer Therapies Act, which would require coverage of oral drugs, including thalidomide, and encourage the creation of a strong blood cancer research effort at the Department of Defense.

Advocacy Day 2003 promises to be a great success and will include leadership from Senator Kay Bailey Hutchison, Geraldine Ferraro and NCI Director Andy von Eschenbach. There will be disease specific educational sessions with Dr. Ken Anderson and Dr. Donna Weber. Advocates can look forward to interactive training; a Congressional reception and award ceremony and lobbying visits with key members of Congress.

The MMRF is grateful for the continued leadership from **Bruce Holmberg**. Bruce, for the second consecutive year, is spearheading the Executive Committee that oversees recruitment from across the country, screens the applications and communicates consistently with the advocates. This year, he and **Anne Quinn Young**, Program Director of the MMRF, co-Chair the Executive Committee, which includes:

|                         |                     |
|-------------------------|---------------------|
| <b>Jo Cavallo</b>       | <b>Beth Compton</b> |
| <b>Gerianne Delaney</b> | <b>Bob Howard</b>   |
| <b>Sue Korn</b>         | <b>Dave Spade</b>   |
| <b>Aimee Swartz</b>     |                     |

who are all working tirelessly to boost country-wide representation of myeloma advocates.



If you are interested in going to Washington to be an official advocate on June 17th and 18th, please fill out an advocate application at [www.multiplemyeloma.org/downloads/Advocacy.doc](http://www.multiplemyeloma.org/downloads/Advocacy.doc) and send to Bruce Holmberg, 3 Old Creek CT, Rockville, MD 20854. For information email Bruce Holmberg at [BPholmberg@comcast.net](mailto:BPholmberg@comcast.net) or call at 301-509-4106.

# INDUSTRY UPDATE

## Phase III Revimid™ Trial Now Open

Two multicenter Phase III trials comparing Revimid (CC-5013, Celgene) and dexamethasone versus dexamethasone in previously-treated patients with myeloma have begun enrollment. A total of 604 patients will be enrolled in two studies at 55 centers in the US, Canada, Europe and Australia.

Revimid is an oral immunomodulatory agent that is chemically similar to thalidomide and displays significant anti-myeloma activity. Patients with active myeloma (Stage II or III) who have received no more than 3 previous anti-myeloma therapies are eligible for the study. Patients must not have received high-dose dexamethasone within the previous 6 months or have had disease progression during high-dose dexamethasone therapy.

Qualified patients will receive Revimid and high-dose dexamethasone or dexamethasone and placebo in 4-week cycles. The total number of cycles will depend on individual patient response. A companion study will be made available that will provide single-agent Revimid therapy to the dexamethasone plus placebo control-arm patients who have developed disease progression.

See the MMRF's Clinical Trial Monitor at [www.multiplemyeloma.org/clinical\\_trials/](http://www.multiplemyeloma.org/clinical_trials/) for more information about this trial.

## Thalidomide May Delay Progression of Early-stage Myeloma

Final results of a non-randomized Phase II study conducted at the Mayo Clinic show that thalidomide has significant activity in early-stage myeloma and may delay progression to symptomatic disease. Patients with early-stage myeloma are typically observed without treatment until they develop symptomatic disease. However, on average, such patients will progress to this stage within 1 to 2 years.

The study included 29 previously untreated patients with smoldering or indolent myeloma who received thalidomide (200 mg/day to start). Of these, 10 patients (34%) had a 50% or greater reduction in serum and urine monoclonal protein. About 80% of the treated patients had not progressed to symptomatic disease after 1 year of therapy and 63% had not progressed at 2 years. The author's note that thalidomide is not without side effects and this approach must be further tested in randomized trials before it can be recommended for general usage.

## Enrollment in Phase III Trial of Thalidomide-Dexamethasone in Previously Untreated Myeloma

The multicenter Phase III trial comparing combination thalidomide plus dexamethasone therapy versus dexamethasone alone in previously untreated myeloma has recently begun enrolling patients. A total of 436 patients with active, untreated myeloma will be enrolled at 85 sites in the US, Canada, Australia and Europe. Patients will receive treatment (thalidomide plus high-dose dexamethasone or a capsule identical in appearance to thalidomide plus high-dose dexamethasone) in 4-week cycles until they experience disease progression or discontinue the study. Patients who progress will be eligible to receive Revimid™ as part of a separate open-label study.

See the MMRF's Clinical Trial Monitor at [www.multiplemyeloma.org/clinical\\_trials/](http://www.multiplemyeloma.org/clinical_trials/) for more information about this trial.

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ACCELERATING THE SEARCH FOR A CURE

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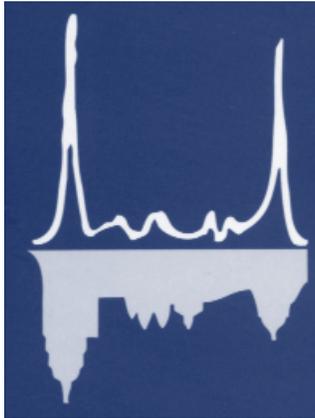
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The information herein is not intended to replace the services of trained health professionals (or to be a substitute for medical advice.) You are advised to consult with your healthcare professional with regard to matters relating to your health, and in particular, regarding matters which may require diagnosis or medical attention.

## Upcoming Highlights from the IX<sup>th</sup> International Myeloma Workshop from Spain



The MMRF is proud to once again be the official web-caster of the International Myeloma Workshop. The Workshop will take place in Salamanca, Spain from May 23-27. This workshop is held every two years and provides insights into the basic mechanisms of the disease and innovative diagnostic and therapeutic strategies for this yet incurable disease.

The MMRF, in collaboration with Workshop organizers Doctors Jesus San Miguel and Joan Blade, will webcast live presentations from leading myeloma experts from around the world. The webcast will include live audio interviews, full transcripts of the interviews and key slides from actual Workshop presentations, all focusing on the latest advances related to understanding and treating multiple myeloma. Be sure to monitor SmartBrief and [www.multiplemyeloma.org](http://www.multiplemyeloma.org) for more information and updates.

## Teleconference With Q&A Session

On June 5th, in collaboration with CancerCare, a long-time MMRF educational partner, the MMRF will bring you a live, hour-long teleconference with two of the researchers who presented at both the International Myeloma Workshop and at the American Society of Clinical Oncology (ASCO) meeting in Chicago. Cutting-edge research on the latest myeloma treatments from the experts will be made available to you and you will have the opportunity to interact with them during a Q&A session. The MMRF is proud that our teleconferences have one of the largest attendee draws from among all the CancerCare teleconferences.

## Special Edition Newsletter Updates on the Latest Research

The MMRF will bring you highlights from the International Myeloma Workshop and from ASCO in a special edition newsletter that will feature leading international experts as guest editors. You will read about the latest research on the disease and its treatments and see some of the slides that were presented at these meetings.

Be sure to monitor SmartBrief and [www.multiplemyeloma.org](http://www.multiplemyeloma.org)  
for more information and updates.





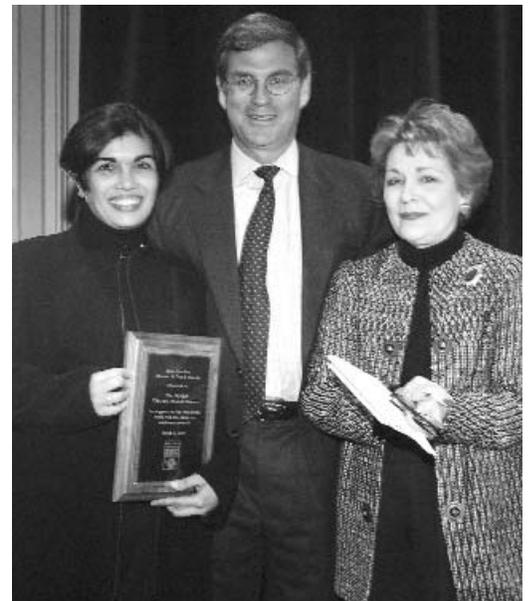
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*Accelerating the Search for a Cure*

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# A Chicago Evening that Accelerates the Search for a Cure!

Judith and George Goldman, founders of the Goldman Philanthropic Partnership, honor Kathy Giusti, President of the MMRF, with the Angel Award for her outstanding efforts in raising awareness and research funds while improving the lives of multiple myeloma patients and care givers



Scott Smith, CEO of the Tribune Company (center) and Margo Howard (right) presented Dr. Seema Singhal (left) a plaque of recognition as the recipient of the \$200,000 MMRF Ann Landers Research Award