



MULTIPLE MYELOMA
Research Foundation



Clinical Studies

May 5, 2023

1

Tech Support

1-719-234-7952

2



3

Resources

- Resource tab includes
 - Speaker bios
 - Copy of the slide presentation
 - Exhibit Hall

**Submit your questions
throughout the program!**

4

MMRF Research Initiatives



CoMMpass StudySM



For more information, visit themmrf.org

5

Speakers

Elizabeth O'Donnell, MD
Dana-Farber Cancer Institute
Harvard University
Boston, Massachusetts

Andrew J. Yee, MD
Harvard Medical School
Massachusetts General Cancer Center
Boston, Massachusetts

6



Clinical Studies: What Are They?

Andrew J. Yee, MD

Harvard Medical School

Massachusetts General Cancer Center

Boston, Massachusetts

7

Goal of Clinical Trials: Making Progress Against Myeloma



Develop treatments and strategies to potentially lengthen lives

- Improve the way we use currently available drugs and regimens
- Develop new medications



Increase the understanding of the disease and how the treatment works

- Identify rational selection of existing drugs

Participants in clinical trials receive specific treatments according to the research plan or protocol created by the investigators to determine the safety and efficacy of the treatment.

8

Impact of Clinical Trials in Myeloma

- Survival rates have nearly doubled; further improvements expected in near future.
- Many new drugs approved since 2003.
- Many new drugs being studied in clinical trials.
- Understanding of the biology of myeloma improving, with the eventual goal of personalized medicine.

9

Therapeutic Options in Myeloma: The Current Landscape

IMiDs	Proteasome inhibitors	Chemotherapy anthracyclines	Chemotherapy alkylators	Steroids	Other mechanisms of action	Antibodies (monoclonal or bispecific)	Cellular therapy (CAR T cells)
Thalomid (thalidomide)	Velcade (bortezomib)	Adriamycin	Cytoxan (cyclophosphamide)	Dexamethasone	XPOVIO (selinexor)	Empliciti (elotuzumab)	Abecma (idecabtagene vicleucel)
Revlimid (lenalidomide)	Kyprolis (carfilzomib)	Doxil (liposomal doxorubicin)	Bendamustine	Prednisone	Venclexta (venetoclax)*	Darzalex (daratumumab)	Carvykti (ciltacabtagene autoleucel)
Pomalyst (pomalidomide)	Ninlaro (ixazomib)		Melphalan			Sarclisa (isatuximab)	
						Tecvayli (teclistamab)†	

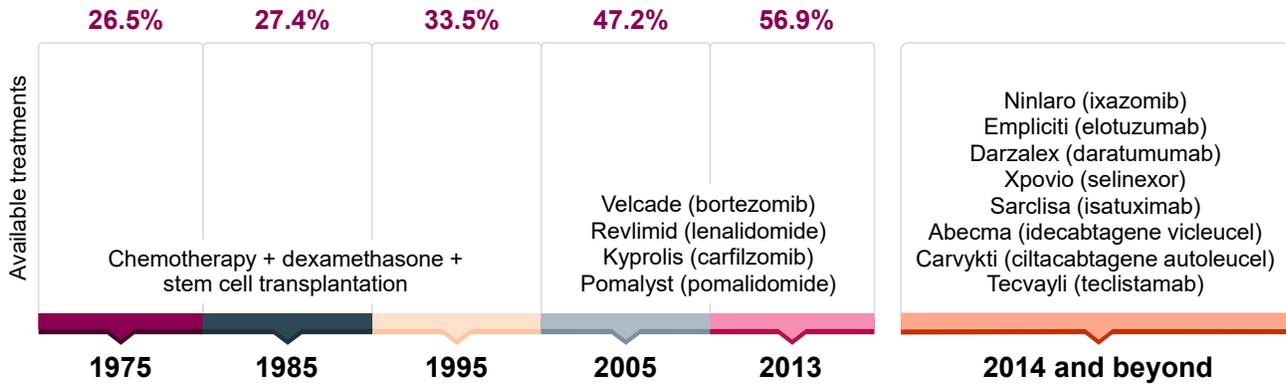
*Not yet FDA-approved for patients with multiple myeloma; †Bispecific antibody

New formulations, new dosing, and new combinations, too!

10

Myeloma Survival Has Improved Over Time Mainly Due to Current Drugs

The percentage of people expected to survive 5 years or more after being diagnosed with myeloma



11

New Drug Development

STEP 1

Identify a target for therapy in the laboratory



STEP 2

Confirm the anti-cancer activity in laboratory and animal studies



STEP 3

Clinical trials (human studies) to determine safety, dosing, and effectiveness



The whole process costs millions of dollars and years of effort!

12

Designing Clinical Studies



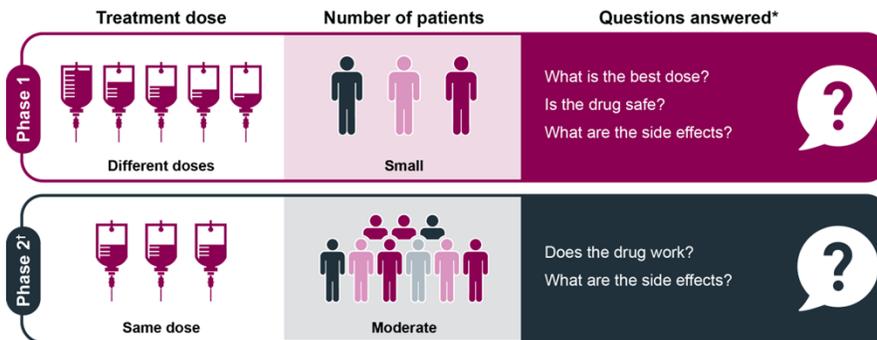
When a treatment is ready to be tested, researchers design a research plan called a protocol that includes such details as:

- How many patients will be enrolled
- How the treatment will be administered
- When and how participants will be monitored
- The goals of the trial: determine safety, identify the right dose, measure the efficacy



Clinical studies pass high standards of scientific design as well as an ethics review to ensure that the trial protects the rights and welfare of all participants.

Traditional Clinical Study Types



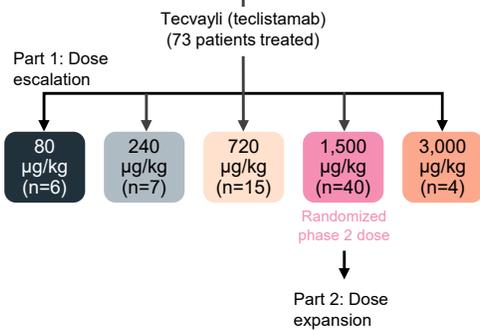
*The FDA approves treatments that are safe, effective, and shown to be better than the standard treatments available.
 †When no standard treatment is available, the FDA may approve drugs based on study results of phase 2 studies.

Examples of Phase 1 and Phase 2 Clinical Studies

New drugs for triple-class refractory patients

Phase 1 MajesTEC-1 Study

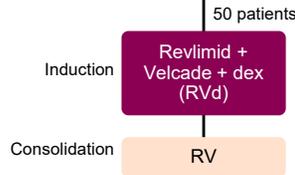
Relapsed/refractory multiple myeloma and progression after ≥ 2 prior treatment regimens



Best drug and combinations

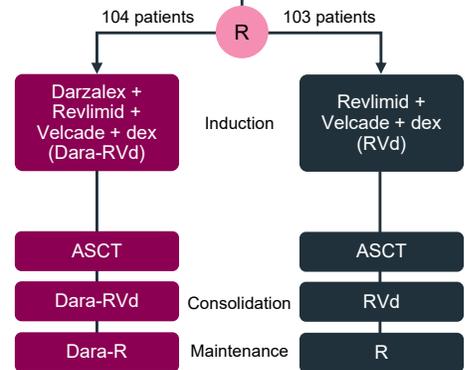
Phase 2 RVD-Lite Study

Transplant-ineligible newly diagnosed myeloma patients



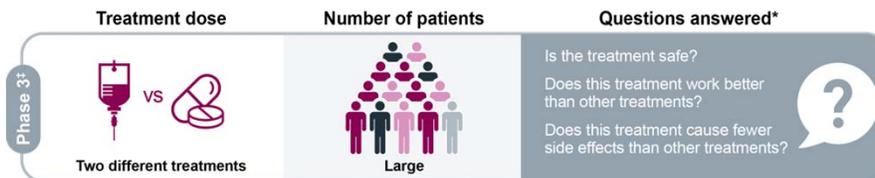
Phase 2 GRIFFIN Study

Newly diagnosed myeloma patients



15

Traditional Clinical Study Types



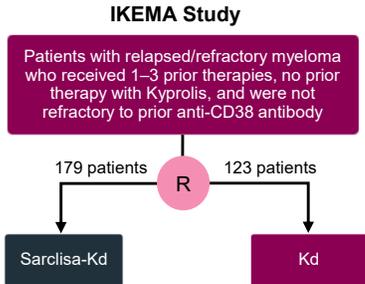
*The FDA approves treatments that are safe, effective, and shown to be better than the standard treatments available.

[†]Conducted to receive FDA approval of new drugs, in most cases.

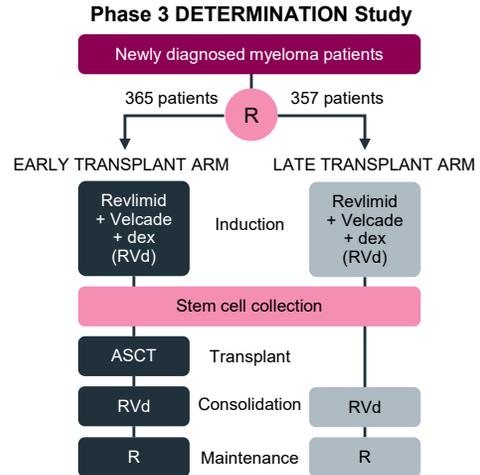
16

Examples of Phase 3 Clinical Studies

Finding the better drug combination

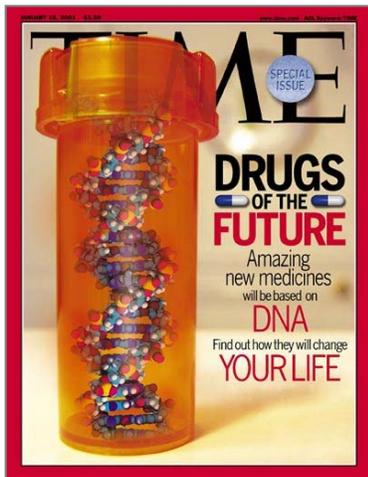


The timing of ASCT

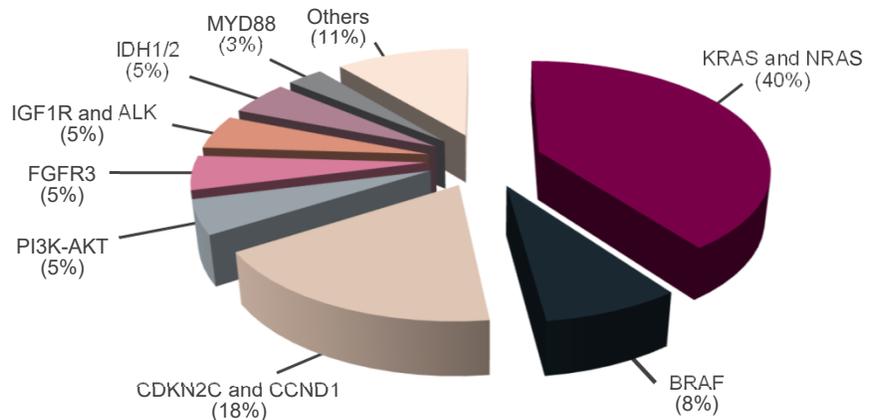


17

Actionable Alterations in MM

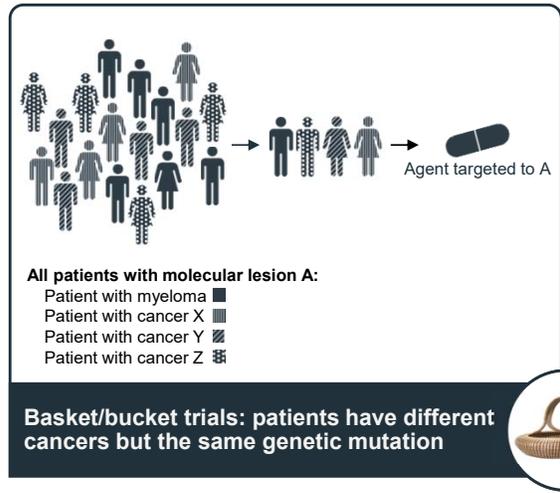
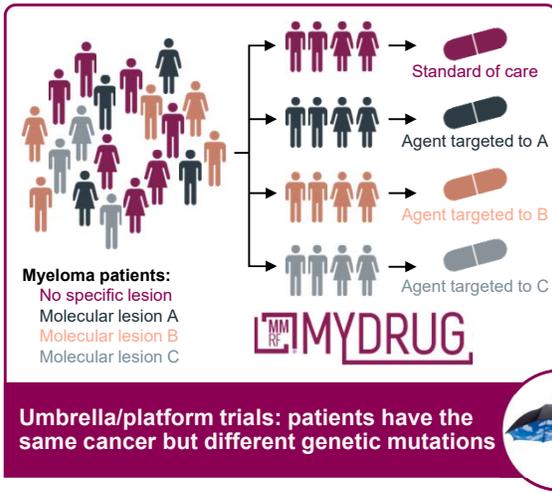


Personalized medicine efforts have identified molecular alterations for which there are drugs in the clinic



18

Innovative Trial Designs: Guiding the Future of Cancer Research Toward Personalized Medicine



Pawlyn C, Davies F. *Blood*. 2019;133:660.

19

Other Types of Clinical Studies

Longitudinal Studies

- Long-term studies with a large number of patients

Registry Studies

- Patients are treated using available therapies
- Efficacy and safety are analyzed following treatment
- Typically involve a large number of patients

Expanded-Access Programs

- Allow early access to experimental therapies when no alternatives are available

CoMMpass StudySM

MM RF CureCloud[®]

20



Participation in a Clinical Study

Elizabeth O'Donnell, MD
Dana-Farber Cancer Institute
Harvard University
Boston, Massachusetts

21

Aren't clinical studies for people who are running out of options?

- Today, clinical studies are used at all stages of disease
 - Clinical studies are available for induction (first) therapy, maintenance therapy, all stages of relapsed disease, and myeloma precursor conditions
- If you have become resistant to standard therapies, clinical studies may offer you another type of treatment—but that is not the only situation in which they are useful



22

If I go on this clinical study, will I be cured?

Patients who participate in clinical studies are the first to receive the newest therapies.

However, it is important to remember that the treatments studied in clinical studies may or may not be more effective than existing treatments, and they do not come with any guarantee as to the outcome.

The treatments may also have side effects.

By participating in a clinical study, you not only give hope to yourself but also to other myeloma patients, including those who will be diagnosed with multiple myeloma in the future.

23

How is my treatment on a clinical study going to be different from standard treatment?

Clinical studies may have fairly strict rules for timing and procedures.

You will work with a clinical study coordinator in addition to your regular providers.

You may have additional office visits or lab work depending upon the requirements of the study.

24

How do I know if I qualify to participate?

Eligibility

- Each study has specific requirements for patients to be eligible

Challenges to Eligibility*

- Renal failure
- Low blood counts
- Recent diagnosis with another type of cancer
- Myeloma that is hard to measure

**This is specific to each study, however, so don't automatically count yourself out!*

25

The Finances of Participating in a Clinical Study



You may receive medications at no cost as part of the study.

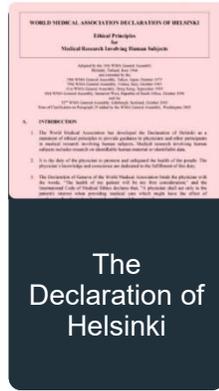
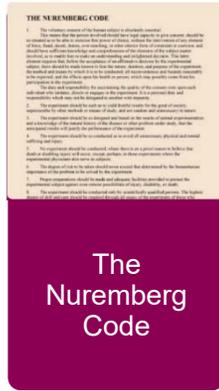
Other standard-of-care treatment will be billed to your insurance as usual.

You may be able to receive money for hotel stays, gas, food, and airfare to defray some of the cost if you are traveling to participate in the study.

Ask your coordinator for details on who is responsible for what costs and discuss any areas of financial concern, as assistance may be available.

26

Will I be treated like a guinea pig? No!



Three influential documents

Benefits of Clinical Trials

- You will have normal standard of care in terms of office visits, lab work, etc
- You may even have additional care and investigation as a part of the clinical trial
- You will generally see your health care providers and will also have a research coordinator involved in your care
- You will likely even have a higher standard of care than normal!



Will I be obligated to continue this study no matter what?

- Most studies have predefined criteria for *progressive disease*, after which patients are taken off the trial
- If a drug is not working, you will not remain on it and can move on to other options for therapy
- Most studies also have criteria for side effects that require discontinuation if they are severe enough
- YOU are still ALWAYS in charge of what goes into your body, and you always have the right to discontinue participation; you will still receive quality care and respectful treatment and will in no way be punished for deciding not to participate



29

Considering Entering Clinical Trials

- Find a clinical trial
 - Contact the MMRF Patient Navigator Center at 1-888-841-6673
 - Visit themmrf.org/resources/clinical-trial-finder/
 - Ask your treating hematologist-oncologist about any available trials
 - Check with any academic medical centers close to your home
- Talk to your doctor about your eligibility
- Meet with the research nurse to learn more
- Carefully review the informed consent paperwork



30

Questions to Ask Your Care Team

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- How does the study work? How often will I need to see my doctor or visit the cancer center?
 - Will I need to undergo additional tests?
 - What is currently known about the new drug or combination?
 - What benefits can I expect?
 - What side effects should I expect? Who should I notify if I have side effects?
 - Can I take my vitamins or other medications?
 - Can I get the treatment with my local doctor?
 - Will my insurance pay for my participation in the clinical study?

31

Summary

- Myeloma survival rates have nearly doubled; further improvements are expected.
- Many new drugs approved in the last two decades.
- The drive of research and clinical trials has brought us to where we are.
- Clinical trials are available for patients at all stages of myeloma, including those who have precursor conditions, those who are newly diagnosed, and those who have received previous treatments and whose myeloma has relapsed.
- No one is expected to be a guinea pig; research and clinical trials are under very tight supervision and standards.
- Open, clear communication between the physician and the patient is essential.

32



MULTIPLE MYELOMA
Research Foundation

25th
ANNIVERSARY

Questions & Answers

33

Phase 1 Phase 2 Phase 3

Multiple Myeloma High-Impact Topic
CLINICAL TRIALS

HOW CLINICAL TRIALS WORK

PROTOCOL

For more information, please visit <https://themmrf.org/resources/education-programs/>

Check out our **NEW High-Impact Topic** videos

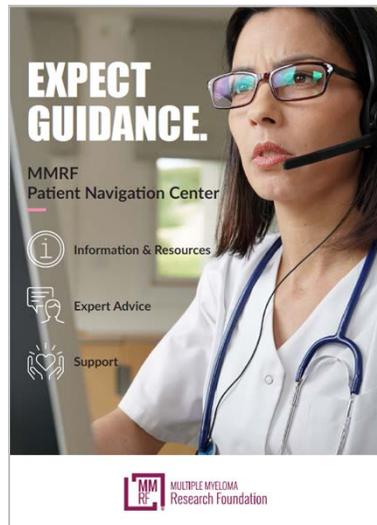
Multiple Myeloma High-Impact Topic
AUTOLOGOUS STEM CELL TRANSPLANT

Multiple Myeloma High-Impact Topic
MULTIPLE MYELOMA PRECURSOR CONDITIONS

Multiple Myeloma High-Impact Topic
THE RIGHT TRACK

34

MMRF Patient Resources



MMRF Patient Navigation Center

You and your care team will have many decisions to make along your treatment journey. The Patient Navigation Center is a space for multiple myeloma patients and their caregivers to connect with patient navigators – who are professionals specializing in oncology – for guidance, information, and support. You can connect with a patient navigator via phone, or email. Whatever questions you may have, our patient navigators are here to help.

MMRF Patient Navigators include:

- Grace Allison, RN, BSN, OCN, RN-BC
- Brittany Hartmann, RN-BSN
- Erin Mensching, RN-BSN, OCN

THE RIGHT TRACK

Get on the right track for you

The MMRF's Right Track program puts you on the path to the best results for you.

 Right Team Access experts and centers that have extensive experience treating multiple myeloma.	 Right Tests Get the information, tests, and precise diagnoses to make the right treatment decisions.	 Right Treatment Work with your team to consider the best treatment plan and identify clinical trials that are right for you.
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Contact the Patient Navigation Center Today
 Looking for guidance? We're here to help.
 Monday – Friday | 9:00am – 7:00pm ET
 Phone: 1-888-841-MMRF (6673) | Online: TheMMRF.org/PatientNavigationCenter
 Email: patientnavigator@themmrf.org

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35



Myeloma Mentors® allows patients and caregivers the opportunity to connect with trained mentors. This is a phone-based program offering an opportunity for a patient and/or caregiver to connect one-on-one with a trained patient and/or caregiver mentor to share his or her patient journeys and experiences.

No matter what your disease state—smoldering, newly diagnosed, or relapsed/refractory—our mentors have insights and information that can be beneficial to both patients and their caregivers.

Contact the Patient Navigation Center at 888-841-6673 to be connected to a Myeloma Mentor or to learn more.

36

MMRF Events

Our events are returning live and in-person, and there are so many ways to get involved. Most have a virtual option, too.

Join us today!

Endurance Events



5K Walk/Run Events



Independent Events



FIND AN EVENT AND JOIN US: <https://themmrf.org/get-involved/mmr-f-events/>

37

Upcoming Patient Education Events

Save the Date

Topic	Date and Time (ET)	Speakers
<i>Facebook Live FAQs</i>	Wednesday, May 17 11:00 AM to 12:00 PM	Noopur Raje, MD Kathleen J. Lively, RN
<i>Patient Summit</i> New York, New York	Saturday, May 20 9:00 AM to 3:45 PM	Saad Usmani, MD Faith Davies, MBBCh Justina Kiernan, PA Neha Korde, MD Sham Mailankody, MBBS Gunjan Shah, MD

For more information or to register,
please visit themmrf.org/resources/education-program

38



39

Resources

- Resource tab includes
 - Speaker bios
 - Copy of the slide presentation
 - Exhibit Hall

40

Need help with travel to a clinical study?

- The MMRF has partnered with the Lazarex Cancer Foundation to help provide more equitable access to clinical studies for multiple myeloma patients
- This partnership is one facet of the MMRF's commitment to improve diversity and representation in myeloma clinical trials
- MMRF has provided \$100,000 over 2 years to Lazarex to fund travel, lodging, and food for patients (and a travel companion) so that they can participate in clinical studies that are appropriate for them
- Patients are funded according to income guidelines and will be reimbursed for allowed expenses
- For more information on this program and to be connected with Lazarex, call our Patient Navigation Center at 1-888-841-6673



41



Thank you!

42