What I Tell My Patients:
Faculty Physicians and Nurses Discuss Patient Education About New Treatments and Clinical Trials

Fifteenth Annual RTP Symposium Series Held During the Annual ONS Congress

Acute Myeloid Leukemia, Myelodysplastic Syndromes and Myelofibrosis

Saturday, April 29, 2023
6:00 AM – 7:30 AM

Faculty

Ilene Galinsky, NP
Ruben A Mesa, MD (Virtual)
Daniel A Pollyea, MD, MS
Richard M Stone, MD
Sara M Tinsley-Vance, PhD, APRN, AOCN

Moderator

Neil Love, MD

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Faculty

Ilene Galinsky, NP
Senior Adult Leukemia Program Research
Nurse Practitioner
Dana-Farber Cancer Institute
Boston, Massachusetts

Ruben A Mesa, MD
President, Enterprise Cancer Service Line
Executive Director, Atrium Health Wake Forest
Baptist Comprehensive Cancer Center
Enterprise Senior Vice President, Atrium Health
Vice Dean for Cancer Programs
Professor of Medicine
Wake Forest University School of Medicine
Winston-Salem, North Carolina

Daniel A Pollyea, MD, MS
Professor of Medicine
Clinical Director of Leukemia Services
Associate Chief of Clinical Affairs
Robert H Allen, MD Chair in Hematology Research
Division of Hematology
University of Colorado School of Medicine
Aurora, Colorado

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Chief of Staff
Dana-Farber Cancer Institute
Professor of Medicine
Harvard Medical School
Boston, Massachusetts

Sara M Tinsley-Vance, PhD, APRN, AOCN
Nurse Practitioner and Researcher
Malignant Hematology
Moffitt Cancer Center
Tampa, Florida

Moderator

Neil Love, MD
Research To Practice
Miami, Florida

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Ms Galinsky — Disclosures

Advisory Committee	AbbVie Inc, Bristol-Myers Squibb Company, CTI BioPharma Corp, Novartis

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Dr Mesa — Disclosures

Advisory Committee	AbbVie Inc, Geron, Incyte Corporation, Telios Pharma Inc
Consulting Agreements	Blueprint Medicines, Bristol-Myers Squibb Company, Celgene Corporation, CTI BioPharma Corp, Genentech, a member of the Roche Group, GSK, Incyte Corporation, MorphoSys, Novartis, Protagonist Therapeutics, Sierra Oncology
Contracted Research	Blueprint Medicines, Bristol-Myers Squibb Company, Celgene Corporation, CTI BioPharma Corp, Incyte Corporation, Sierra Oncology
Data and Safety Monitoring Board/Committee	Geron, Telios Pharma Inc

Dr Pollyea — Disclosures

Advisory Committee	AbbVie Inc, Adicet Bio, Arcellx, AstraZeneca Pharmaceuticals LP, BeiGene Ltd, BerGenBio ASA, Bristol-Myers Squibb Company, Daiichi Sankyo Inc, Genentech, a member of the Roche Group, Gilead Sciences Inc, HiberCell, ImmunoGen Inc, Jazz Pharmaceuticals Inc, Kura Oncology, Link Pharma, Magenta Therapeutics, Medivir AB, Novartis, OncoVerity, Qihan Biotech, Rigel Pharmaceuticals Inc, Ryvu Therapeutics, Seres Therapeutics, Sumitomo Dainippon Pharma Oncology Inc, Syndax Pharmaceuticals Inc, Syros Pharmaceuticals Inc, Zentalis Pharmaceuticals
Consulting Agreements	AbbVie Inc, Aptevo Therapeutics, Schrödinger, Syros Pharmaceuticals Inc
Contracted Research	AbbVie Inc, Bristol-Myers Squibb Company, Karyopharm Therapeutics, Teva Oncology
Data and Safety Monitoring Board/Committee	Aptevo Therapeutics, GlycoMimetics Inc

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Dr Stone — Disclosures

Advisory Board	AbbVie Inc, Actinium Pharmaceuticals Inc, Amgen Inc, Arog Pharmaceuticals Inc, AvenCell, Boston Pharmaceuticals, Bristol-Myers Squibb Company, Celularity, CTI BioPharma Corp, Genentech, a member of the Roche Group, GSK, Hemavant, Janssen Biotech Inc, Jazz Pharmaceuticals Inc, Kura Oncology, Lava Therapeutics, Ligand Pharmaceuticals Incorporated, Novartis, Syros Pharmaceuticals Inc, Rigel Pharmaceuticals Inc, Takeda Pharmaceuticals USA Inc
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Dr Tinsley-Vance — Disclosures

Advisory Committee	Bristol-Myers Squibb Company, CTI BioPharma Corp, Incyte Corporation, Jazz Pharmaceuticals Inc
Consulting Agreements	AbbVie Inc, Bristol-Myers Squibb Company, CTI BioPharma Corp, Incyte Corporation
Speakers Bureau	Astellas, Bristol-Myers Squibb Company, CTI BioPharma Corp, Incyte Corporation, Jazz Pharmaceuticals Inc

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Commercial Support

This activity is supported by educational grants from Bristol-Myers Squibb Company, CTI BioPharma Corp, and GSK.

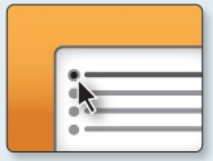
Research To Practice NCPD Planning Committee Members, Staff and Reviewers

Planners, scientific staff and independent reviewers for Research To Practice have no relevant conflicts of interest to disclose.

Clinicians in the Meeting Room

Networked iPads are available.

Review Program Slides: Tap the Program Slides button to review speaker presentations and other program content.



Answer Survey Questions: Complete the pre- and postmeeting surveys. Survey questions will be discussed throughout the meeting.

Ask a Question: Tap Ask a Question to submit a challenging case or question for discussion. We will aim to address as many questions as possible during the program.

Complete Your Evaluation: Tap the NCPD Evaluation button to complete your evaluation electronically to receive credit for your participation.

For assistance, please raise your hand. Devices will be collected at the conclusion of the activity.

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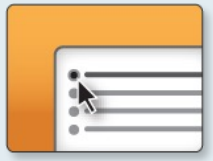
Clinicians Attending via Zoom

Review Program Slides: A link to the program slides will be posted in the chat room at the start of the program.



Answer Survey Questions: Complete the pre- and postmeeting surveys. Survey questions will be discussed throughout the meeting.

Ask a Question: Submit a challenging case or question for discussion using the Zoom chat room.

Get NCPD Credit: An NCPD credit link will be provided in the chat room at the conclusion of the program.

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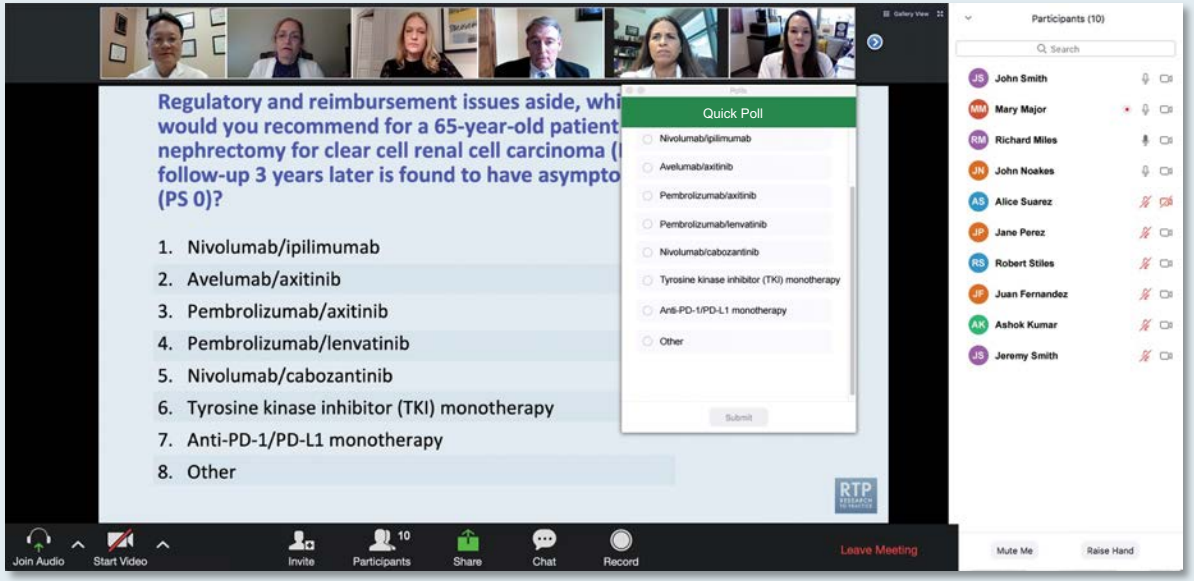
Clinicians, Please Complete the Pre- and Postmeeting Surveys



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About the Enduring Program

- The live meeting is being video and audio recorded.
- The proceedings from today will be edited and developed into an enduring web-based video/PowerPoint program.
  An email will be sent to all attendees when the activity is available.
- To learn more about our education programs, visit our website, **www.ResearchToPractice.com**

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“What I Tell My Patients”
Fifteenth Annual RTP-ONS NCPD Symposium Series
ONS Congress, San Antonio, Texas — April 26 to 29, 2023

Wednesday April 26	**Cervical and Endometrial Cancer** 11:15 AM – 12:45 PM CT (12:15 PM – 1:45 PM ET)
	Breast Cancer 6:00 PM – 8:00 PM CT (7:00 PM – 9:00 PM ET)
Thursday April 27	**Diffuse Large B-Cell Lymphoma** 6:00 AM – 7:30 AM CT (7:00 AM – 8:30 AM ET)
	Chronic Lymphocytic Leukemia 12:15 PM – 1:45 PM CT (1:15 PM – 2:45 PM ET)
	HER2-Targeted Antibody-Drug Conjugates 6:00 PM – 7:30 PM CT (7:00 PM – 8:30 PM ET)
Friday April 28	**Hepatobiliary Cancers** 6:00 AM – 7:30 AM CT (7:00 AM – 8:30 AM ET)
	Ovarian Cancer 12:15 PM – 1:45 PM CT (1:15 PM – 2:45 PM ET)
	Lung Cancer 6:00 PM – 8:00 PM CT (7:00 PM – 9:00 PM ET)
Saturday April 29	**Acute Myeloid Leukemia, Myelodysplastic Syndromes and Myelofibrosis** 6:00 AM – 7:30 AM CT (7:00 AM – 8:30 AM ET)
	Prostate Cancer 12:15 PM – 1:45 PM CT (1:15 PM – 2:45 PM ET)

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What I Tell My Patients

2023 ONS Congress

San Antonio, Texas

Symposia Themes

- New agents and therapies; ongoing trials related to specific clinical scenarios
- Patient education: Preparing to receive new therapies
- The bond that heals
- THANKS! (Great job)

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How was it different to take care of this patient versus another patient in the same oncologic setting?

What unique biopsychosocial factors (eg, attitude, comorbidities, social support) were considered in the overall management of this case?

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Enterprise Senior Vice President, Atrium Health
Vice Dean for Cancer Programs
Professor of Medicine
Wake Forest University School of Medicine
Winston-Salem, North Carolina

Daniel A Pollyea, MD, MS
Professor of Medicine
Clinical Director of Leukemia Services
Associate Chief of Clinical Affairs
Robert H Allen, MD Chair in Hematology Research
Division of Hematology
University of Colorado School of Medicine
Aurora, Colorado

Richard M Stone, MD
Lunder Family Chair in Leukemia
Chief of Staff
Dana-Farber Cancer Institute
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Miami, Florida

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Agenda

Module 1: Overview of MPNs and MF — Impact of Ruxolitinib

Module 2: New Agents in MF — Fedratinib, Momelotinib, Pacritinib

Module 3: MDS — Management of Low- and High-Risk Disease

Module 4: Overview of AML

Module 5: Targeted Therapy for AML

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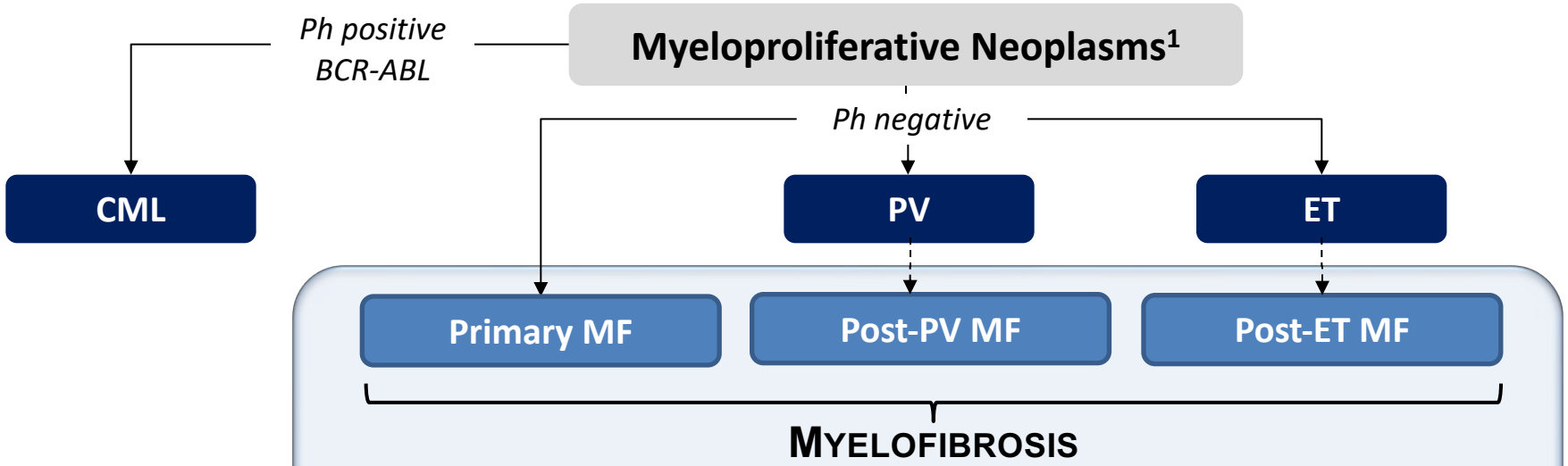
Ilene Galinsky, NP

87-year-old man with PMH of benign pleural effusions and pericardial effusions is diagnosed with MPL mutation-positive myelofibrosis (MF) and receives ruxolitinib

Clinical Research Background

- Overview of myeloproliferative neoplasms
  - Disease classification
  - Impact of ruxolitinib

Overview of Myelofibrosis (MF)



MF prevalence

- Approximately 16,000 to 18,500 patients in the United States[2]

MF is characterized by

- Bone marrow fibrosis, cytopenias, and constitutional symptoms[3]
- Splenomegaly[4]

MF natural history

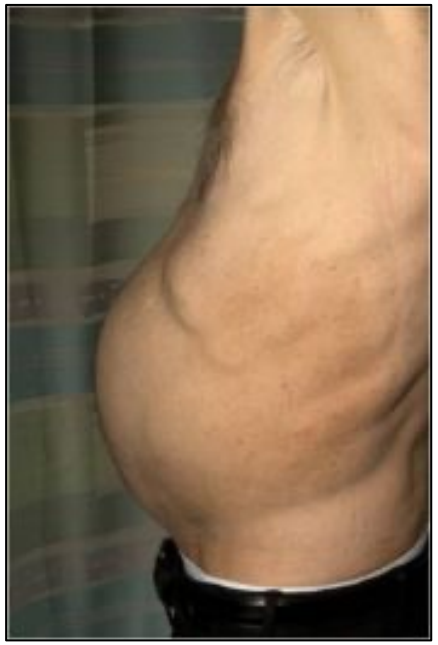
- Time course of disease progression is highly variable but frequently characterized by a period of stable disease followed by a late stage with rapid clinical progression[5,6]

MF, myelofibrosis
PV, polycythemia vera
ET, essential thrombocythemia
CML, chronic myeloid leukemia

[1]Tefferi A, Vardiman JW. *Leukemia*. 2008;22:14-22; [2]Data on file, Incyte Corporation; [3]Verstovsek S. *Clin Can Res*. 2010;16:1988-1996;
[4]Mesa RA. *Blood*. 2009;113(22):5394-5400; [5]Cervantes F, et al. *Blood*. 2009;113:2895-2901; [6]Tam CS, et al. *J Clin Oncol*. 2009;27:5587-5593.

Myelofibrosis: Clinical Manifestations[1]

Constitutional symptoms	Fatigue, weight loss, cachexia, pruritus, night sweats, bone/joint pain, low-grade fever, cough
Marked hepatosplenomegaly	Early satiety, abdominal discomfort, painful splenic infarcts, portal hypertension, cachexia
Nonhepatosplenic extramedullary hematopoiesis (rare)	Cord compression, ascites, pulmonary hypertension, pulmonary embolism, lymphadenopathy, skin tumors
Thrombohemorrhagic complications	
Marked leukocytosis or thrombocytosis; severe anemia, thrombocytopenia, neutropenia; hyperuricemia	
Increased risk of leukemic transformation	

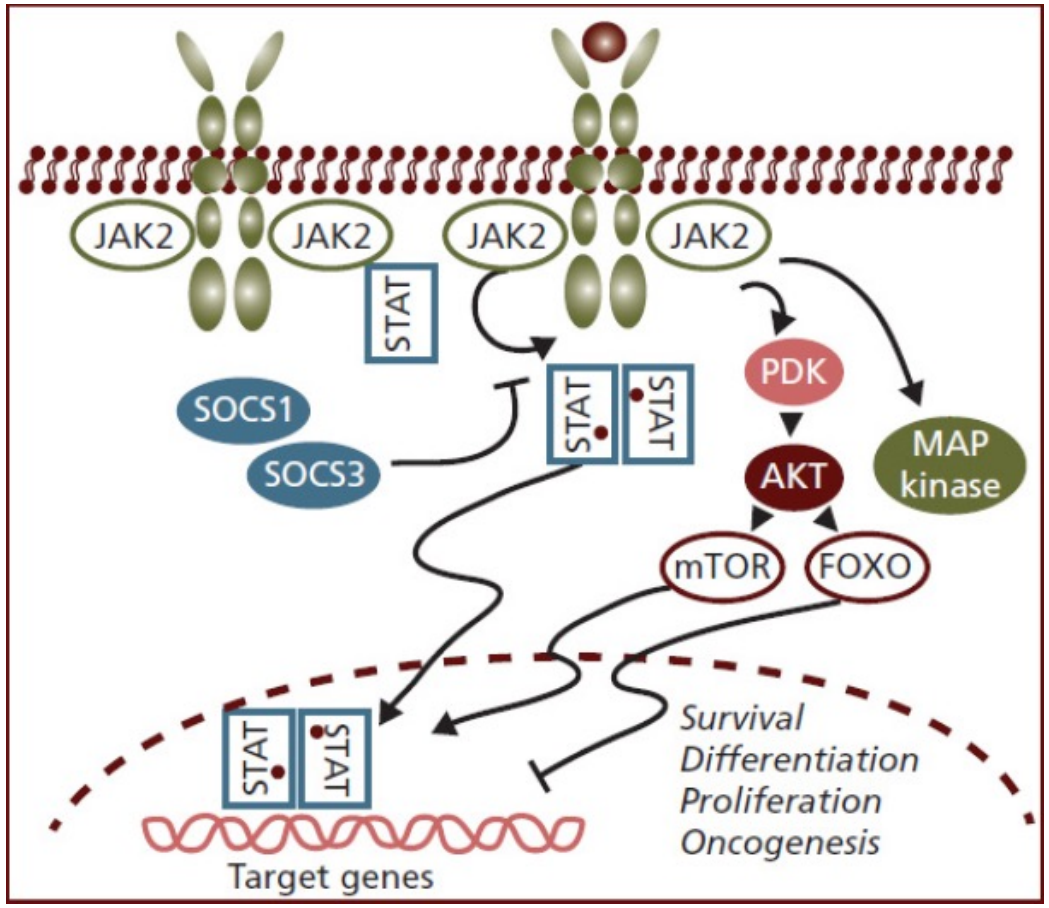


Splenomegaly[2]

1. Barbui T et al. *J Clin Oncol*. 2011;29:761-770. 2. Image provided courtesy of S. Verstovsek.

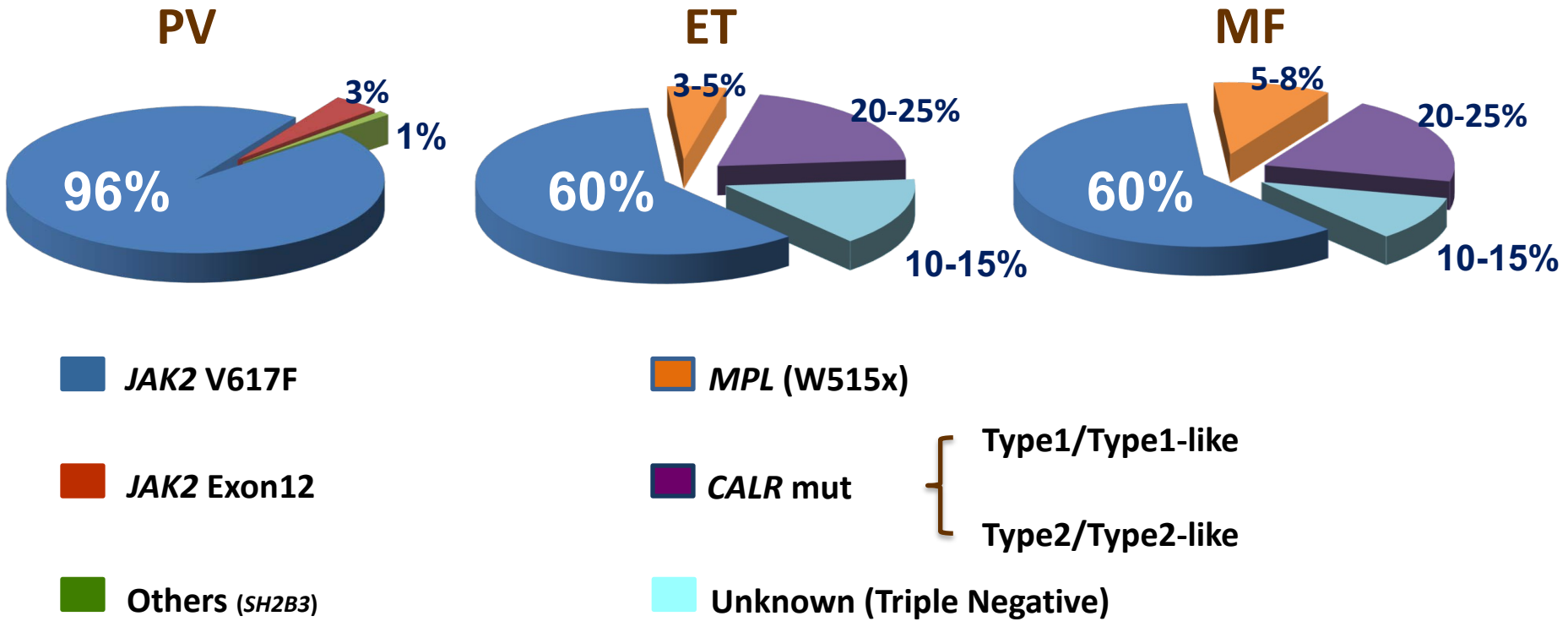
JAK-STAT Pathway Constitutively Activated in Myelofibrosis

- JAK-STAT pathway implicated in normal hematopoiesis[1]
- An activating mutation in the pseudokinase domain of *Janus kinase 2* (*JAK2*) was identified in approximately 50% of MF patients
- Dysregulation of JAK-STAT, regardless of *JAK* mutation status, is a key pathologic feature of MF and other MPNs[1,2]



1. Vannucchi AM et al. *CA Cancer J Clin*. 2009;59:171-191. 2. Anand S et al. *Blood*. 2011;118:1610-1621.

Phenotypic Driver Mutations (they activate JAK-STAT pathway) in MPNs



Klampfl T, et al. *NEJM* 2013;369(25):2379-90; Nangalia J, et al. *NEJM* 2013;369(25):2391-405.

Ruxolitinib

Mechanism of action

- **Orally bioavailable Janus-associated kinase (JAK) 1/2 inhibitor with potential antineoplastic and immunomodulating activities**

Indication

- **For the treatment of intermediate or high-risk myelofibrosis, including primary myelofibrosis, post-polycythemia vera myelofibrosis and post-essential thrombocythemia myelofibrosis in adults**
- **For the treatment of polycythemia vera in patients who have had an inadequate response to or are intolerant of hydroxyurea**

Recommended dose

Myelofibrosis:

- **Starting dose based on patient's baseline platelet count:**
  - **Greater than 200 x 10^9/L: 20 mg given orally twice daily**
  - **100 x 10^9/L to 200 x 10^9/L: 15 mg given orally twice daily**
  - **50 x 10^9/L to less than 100 x 10^9/L: 5 mg given orally twice daily**
- **Monitor complete blood counts every 2 to 4 weeks until doses are stabilized, and then as clinically indicated Modify or interrupt dosing for thrombocytopenia**

Polycythemia vera:

- **Starting dose is 10 mg given orally twice daily**

Ruxolitinib package insert, 1/2023.

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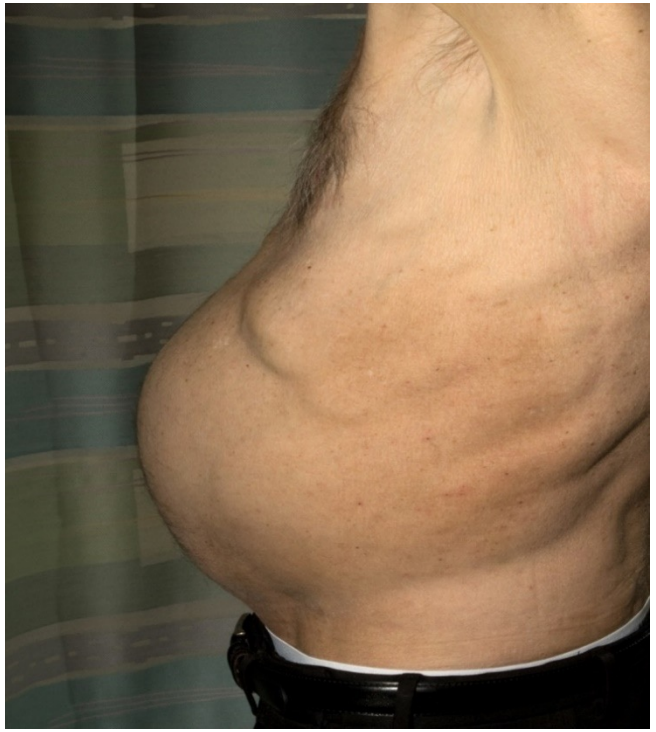
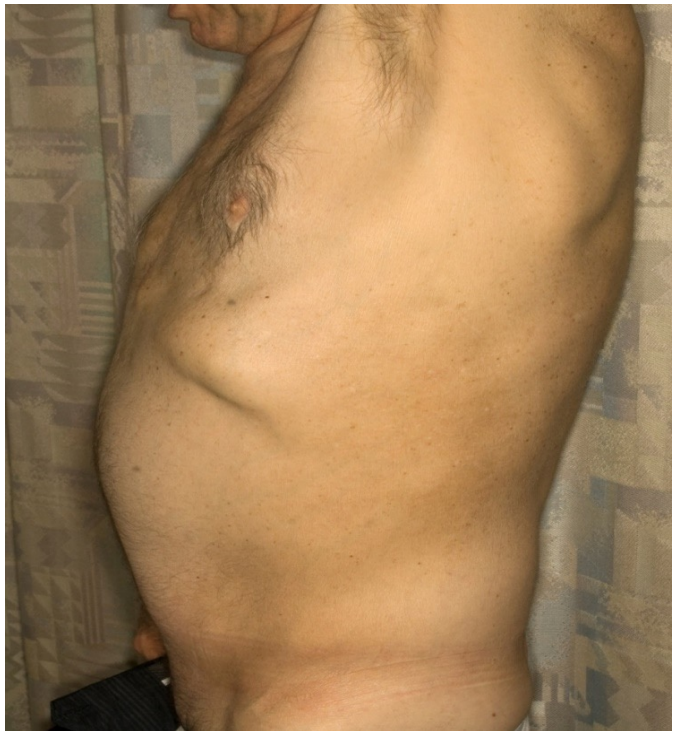
What Does Ruxolitinib Do?



Patient Pre-Ruxolitinib Therapy



After 2 Months of Therapy

It is good for spleen and symptoms

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Module 5: Targeted Therapy for AML

Sara M Tinsley-Vance, PhD, APRN, AOCN

75-year-old male with MF who received fedratinib and developed altered mental status and decreased thiamine levels

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Fedratinib

Mechanism of action

- JAK2 inhibitor

Indication

- For the treatment of adult patients with intermediate-2 or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis (MF)

Recommended dose

- 400 mg orally once daily with or without food for patients with a baseline platelet count of greater than or equal to 50 x 10^9/L

Fedratinib package insert, 10/2022.

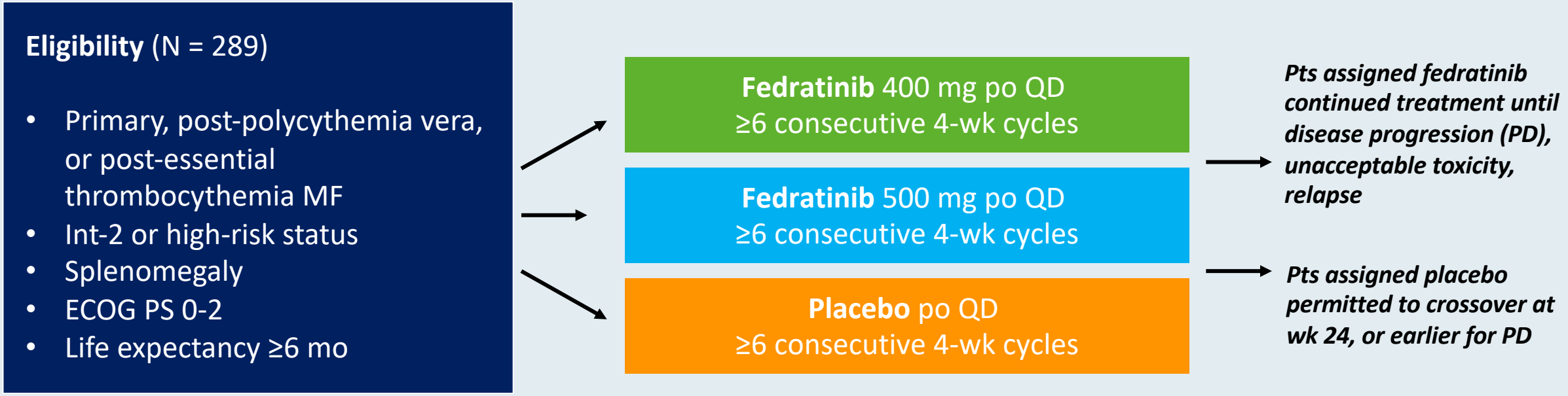
bjh short report

Updated results of the placebo-controlled, phase III JAKARTA trial of fedratinib in patients with intermediate-2 or high-risk myelofibrosis

Animesh Pardanani,[1] Ayalew Tefferi,[1] Tamás Masszi,[2] Elena Mishchenko,[3] Mark Drummond,[4] Eric Jourdan,[5] Alessandro Vannucchi,[6] Mindaugas Jurgutis,[7] Vincent Ribrag,[8] Alessandro Rambaldi,[9,10] Liang Piu Koh,[11] Shelonitda Rose,[12] Jun Zhang[12] and Claire Harrison[13]

Br J Haematol 2021;195:244-8.

JAKARTA Phase III Study Design

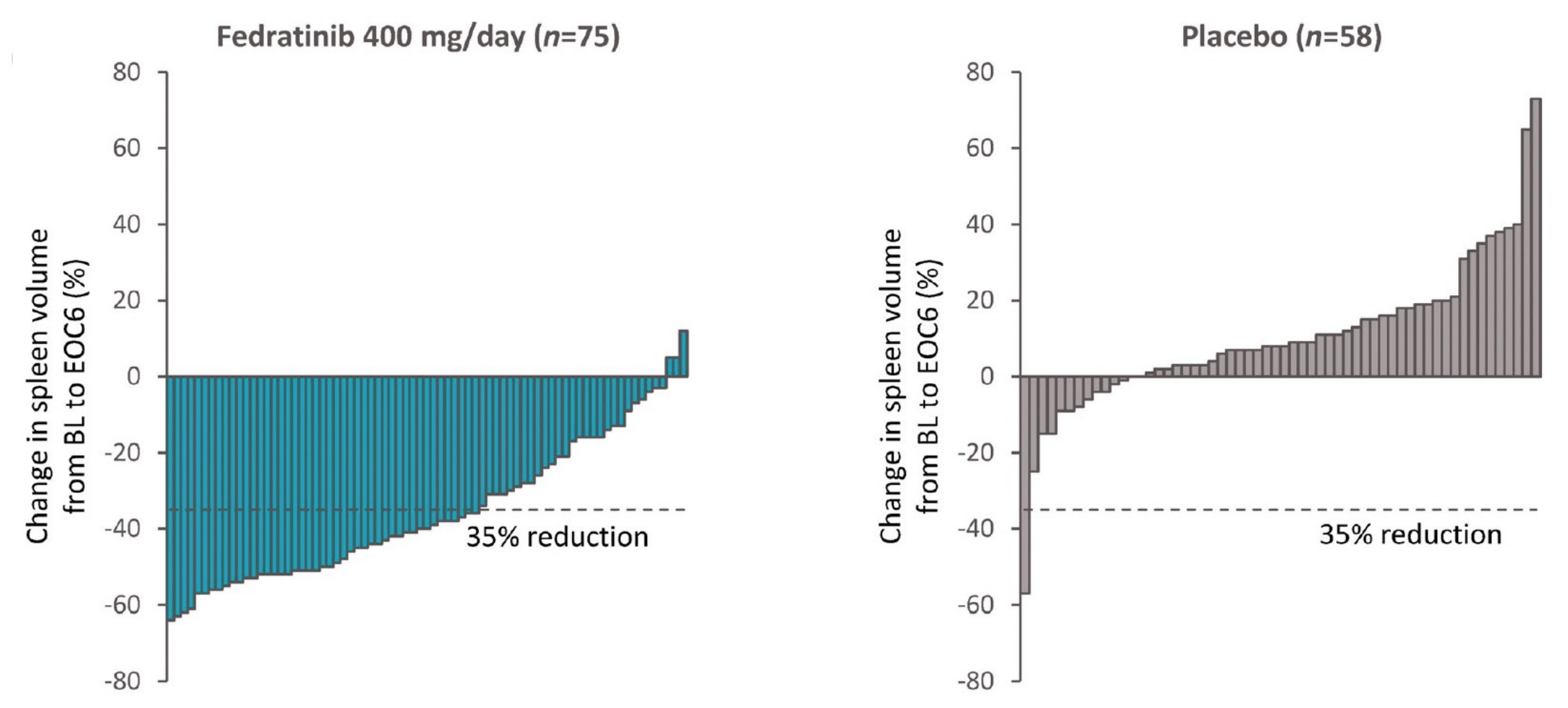


Primary endpoint: Spleen response (≥35% reduction in spleen volume vs baseline) at wk 24, and confirmed 4 wk later

Secondary endpoints: Symptom response (≥50% reduction in total symptom score), safety

Pardanani A et al. *JAMA Oncol* 2015;1:643-51.

JAKARTA: Change in Spleen Volume from Baseline to End of Cycle 6



Pardanani A et al. *Br J Haematol* 2021;195:244-8.

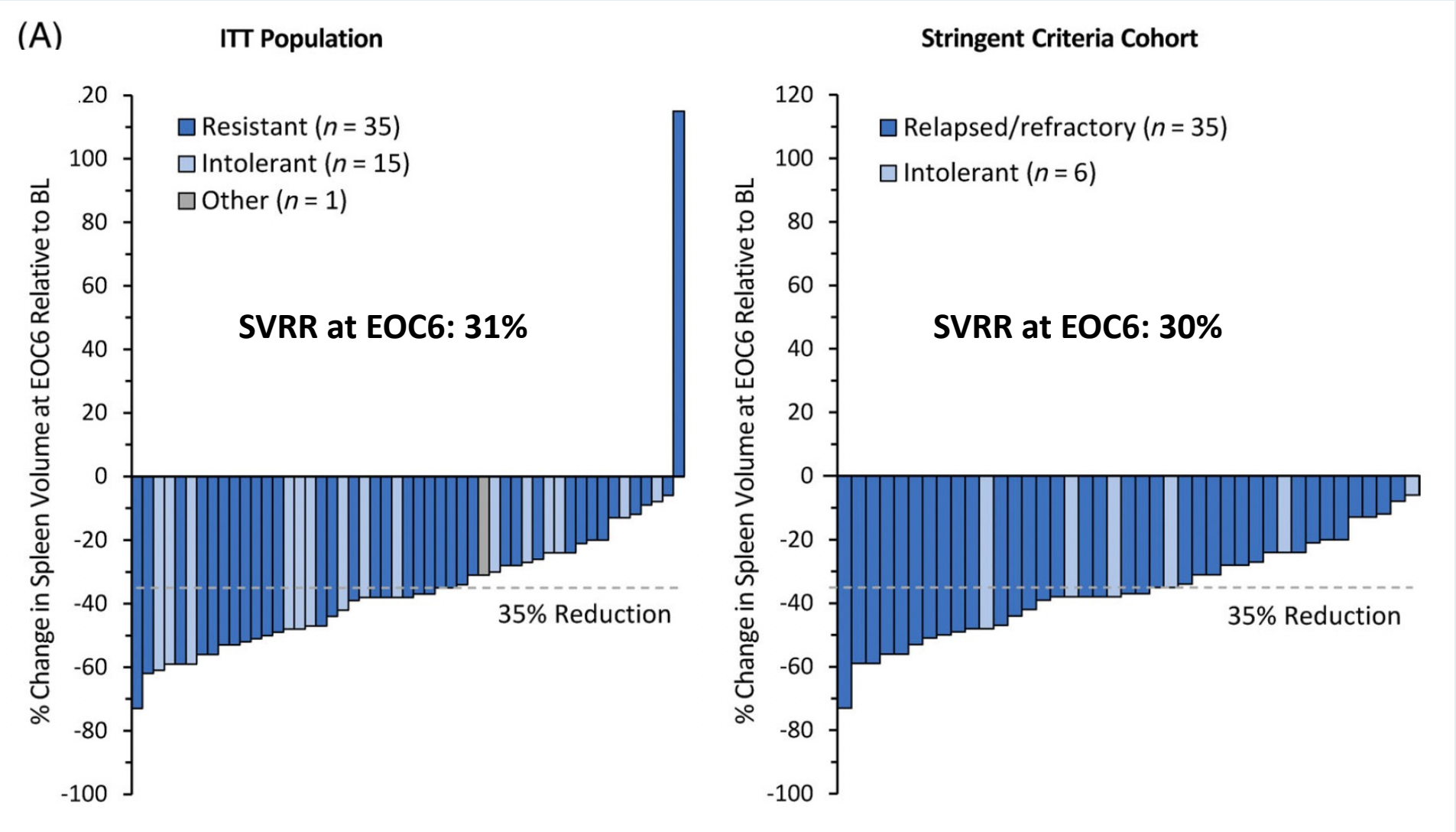
RESEARCH ARTICLE

Am J Hematol 2020;95:594-603

Fedratinib in patients with myelofibrosis previously treated with ruxolitinib: An updated analysis of the JAKARTA2 study using stringent criteria for ruxolitinib failure

Claire N. Harrison[1] | Nicolaas Schaap[2] | Alessandro M. Vannucchi[3] | Jean-Jacques Kiladjian[4] | Eric Jourdan[5] | Richard T. Silver[6] | Harry C. Schouten[7] | Francesco Passamonti[8] | Sonja Zweegman[9] | Moshe Talpaz[10] | Srdan Verstovsek[11] | Shelonitda Rose[12] | Juan Shen[12] | Tymara Berry[12] | Carrie Brownstein[12] | Ruben A. Mesa[13]

JAKARTA-2: Change from Baseline in Spleen Volume



ITT = intent-to-treat; SVRR = spleen volume response rate; EOC6 = end of cycle 6

Harrison CN et al. *Am J Hematol* 2020;95:594-603.

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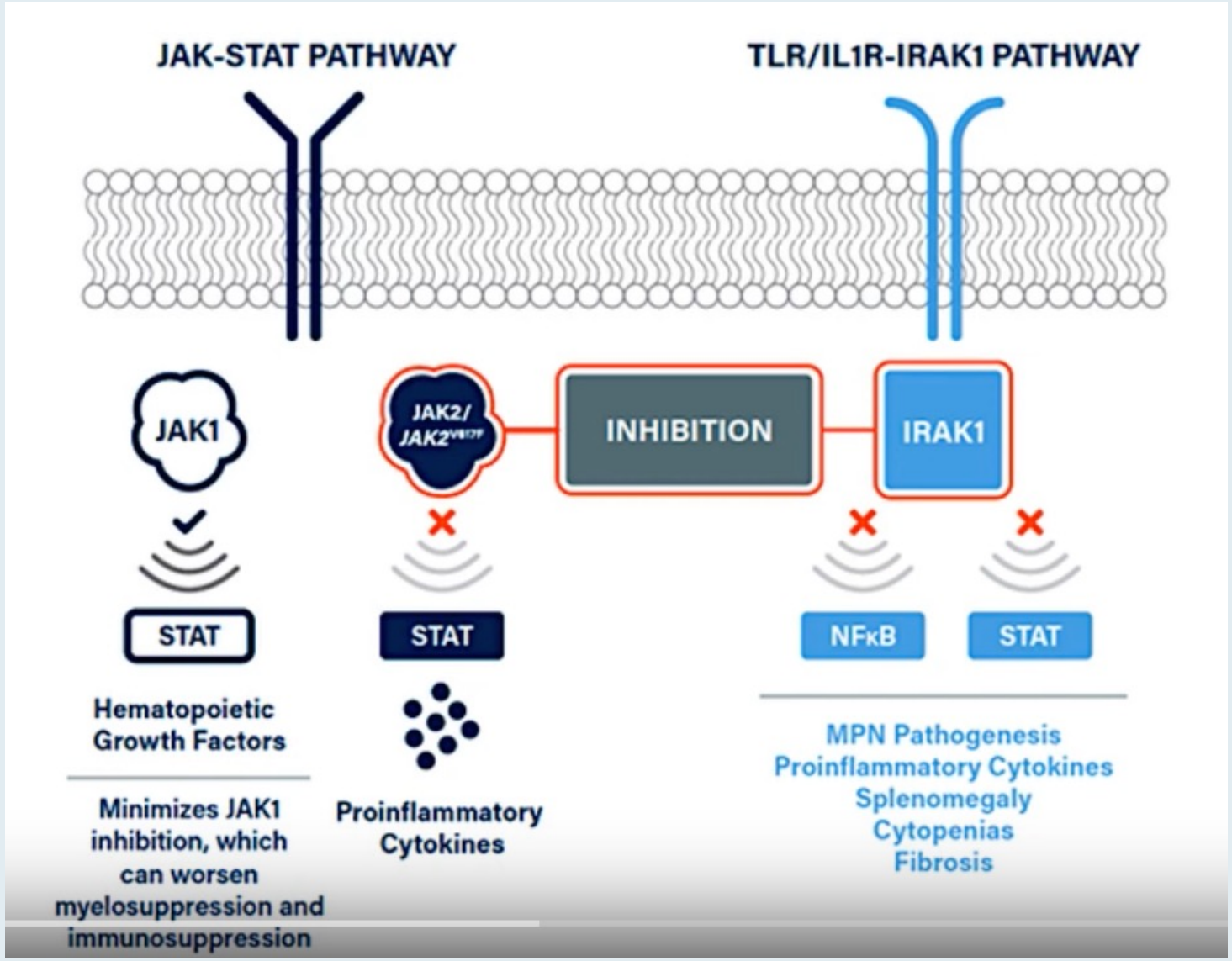
Ilene Galinsky, NP

83-year-old woman with multiple comorbidities and SRSF2 mutation-positive MF who received pacritinib

Pacritinib Mechanism of Action: JAK2 and IRAK1 Inhibitor



https://www.youtube.com/watch?v=z8Dkkp-vnIA

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Pacritinib

Mechanism of action

- **Oral inhibitor of JAK2 and FLT3**

Indication

- **For the treatment of intermediate- or high-risk primary or secondary (post-polycythemia vera or post-essential thrombocythemia) myelofibrosis in adult patients with a platelet count below 50 x 10^9/L**

Recommended dose

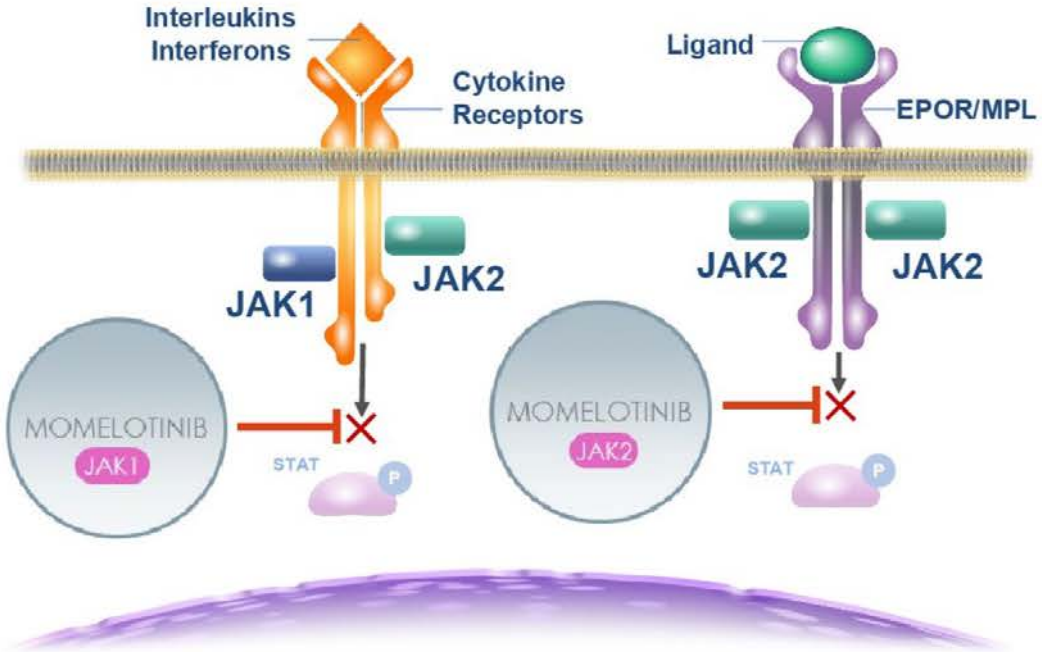
- **200 mg BID with or without food**

Pacritinib package insert, 2/2022.

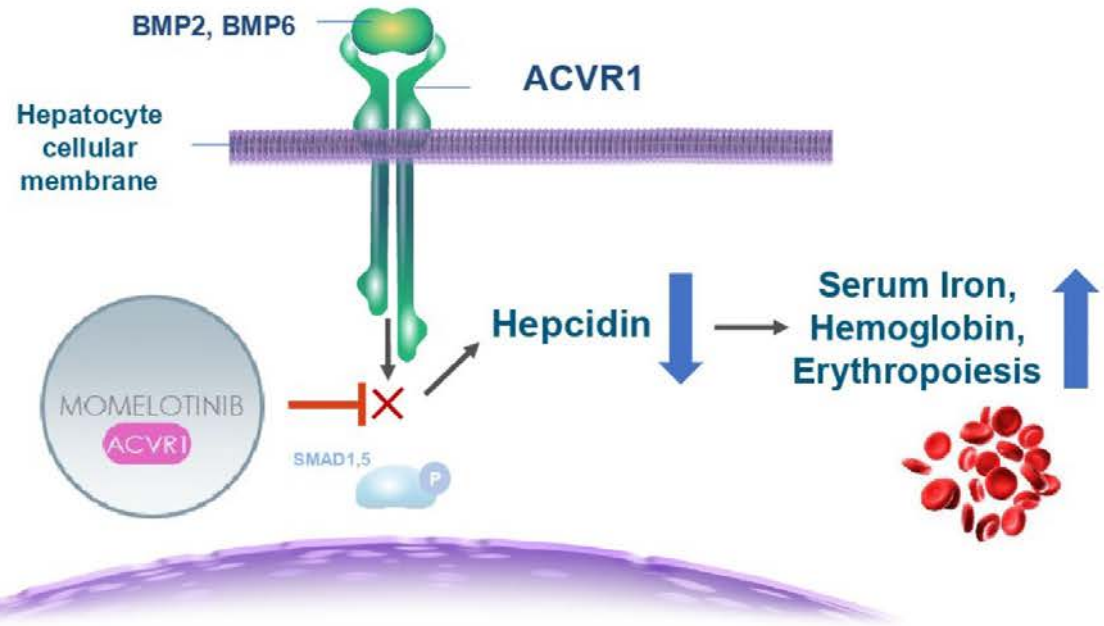
Sara M Tinsley-Vance, PhD, APRN, AOCN

79-year-old man with post-polycythemia vera MF who received momelotinib

Momelotinib Inhibits JAK1, JAK2 and ACVR1 to Address MF Symptoms, Spleen and Anemia



Dysregulated **JAK-STAT signaling** in MF drives overproduction of inflammatory cytokines, **bone marrow fibrosis, systemic symptoms,** and clonal proliferation resulting in extramedullary hematopoiesis and **splenomegaly**.[1,2]



Chronic inflammation also drives hyperactivation of **ACVR1**, elevated **hepcidin**, dysregulated iron metabolism, and **anemia** of MF.[3,4]

Mesa R et al. ASCO 2022;Abstract 7002.

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Momelotinib

Mechanism of action

- **Highly selective JAK1/2 and activin A receptor, Type 1 inhibitor**

Indication

- **Investigational**

Key clinical data

- **Momelotinib versus danazol in symptomatic patients with anemia and myelofibrosis (MOMENTUM): Results from an international, double-blind, randomized, controlled, Phase III study**

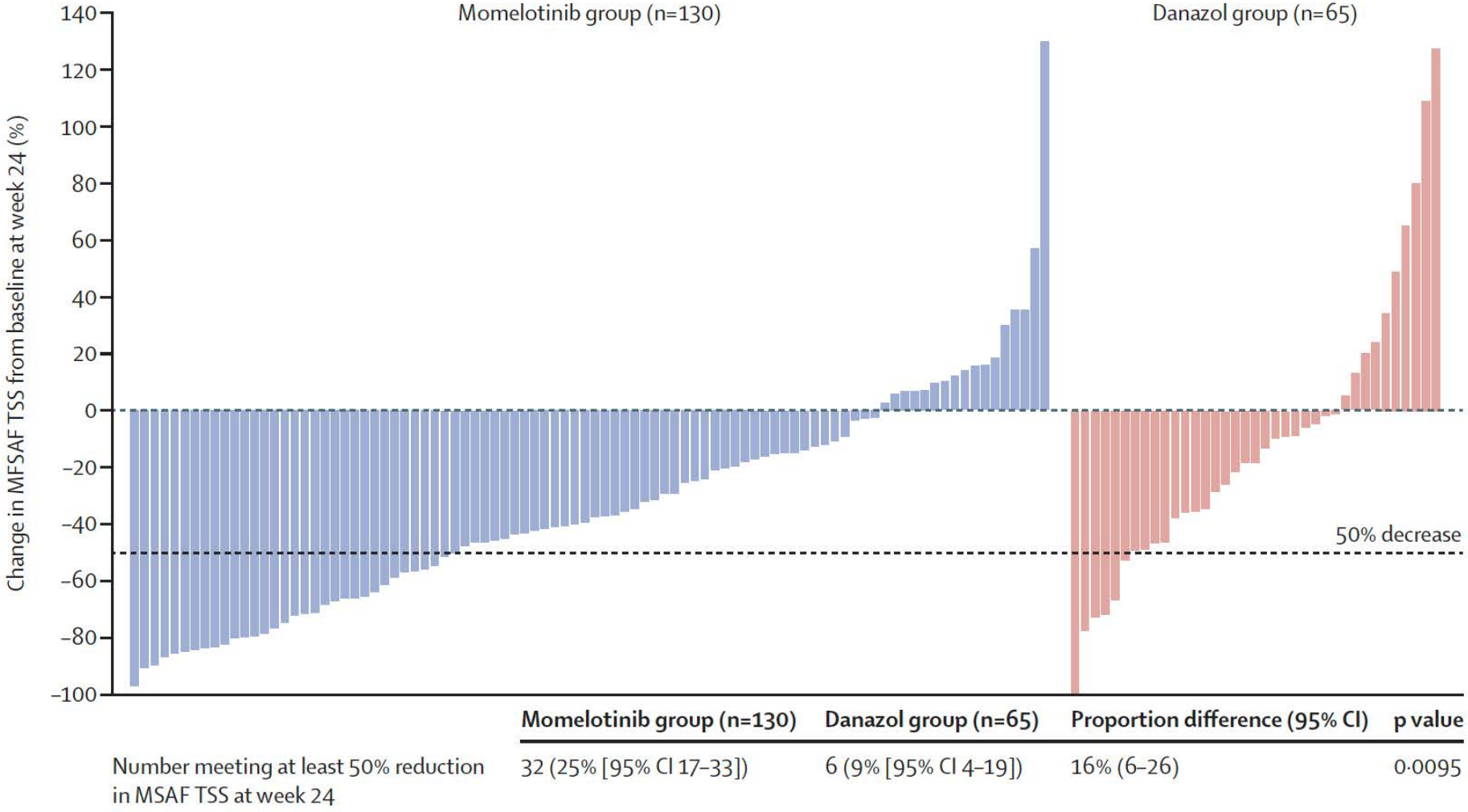
Verstovsek S et al. *Lancet* 2023 January 28;401(10373):269-80.

Lancet 2023;401-269-80

Momelotinib versus danazol in symptomatic patients with anaemia and myelofibrosis (MOMENTUM): results from an international, double-blind, randomised, controlled, phase 3 study

*Srdan Verstovsek, Aaron T Gerds, Alessandro M Vannucchi, Haifa Kathrin Al-Ali, David Lavie, Andrew T Kuykendall, Sebastian Grosicki, Alessandra Iurlo, Yeow Tee Goh, Mihaela C Lazaroiu, Miklos Egyed, Maria Laura Fox, Donal McLornan, Andrew Perkins, Sung-Soo Yoon, Vikas Gupta, Jean-Jacques Kiladjian, Nikki Granacher, Sung-Eun Lee, Luminita Ocroteala, Francesco Passamonti, Claire N Harrison, Barbara J Klencke, Sunhee Ro, Rafe Donahue, Jun Kawashima, Ruben Mesa, on behalf of MOMENTUM Study Investigators**

MOMENTUM: Percentage Change of TSS from Baseline to Week 24 for Each Patient



	Momelotinib group (n=130)	Danazol group (n=65)	Proportion difference (95% CI)	p value
Number meeting at least 50% reduction in MSAF TSS at week 24	32 (25% [95% CI 17–33])	6 (9% [95% CI 4–19])	16% (6–26)	0·0095

MFSAF=Myelofibrosis Symptom Assessment Form TSS=total symptom score

Verstovsek S et al. *Lancet* 2023;401:269-80.

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Ilene Galinsky, NP

81-year-old man with JAK2 mutation-positive MDS with ringed sideroblasts and anemia requiring blood transfusions who received luspatercept

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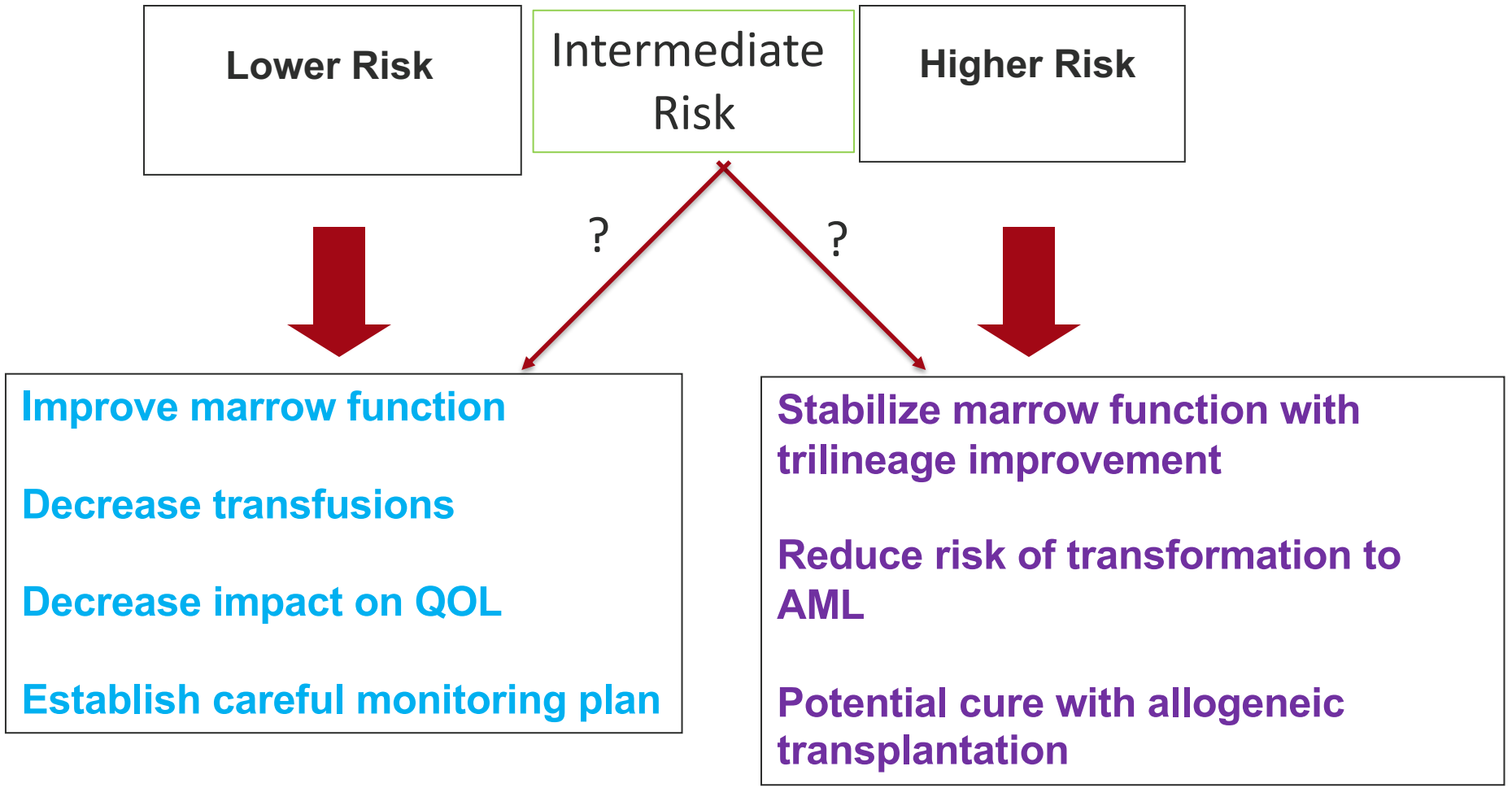
Clinical Research Background

- **Myelodysplastic syndromes**
  - **Use of luspatercept for low-risk disease**
  - **Potential role of venetoclax/HMA for high-risk disease**

Treatment goals in MDS

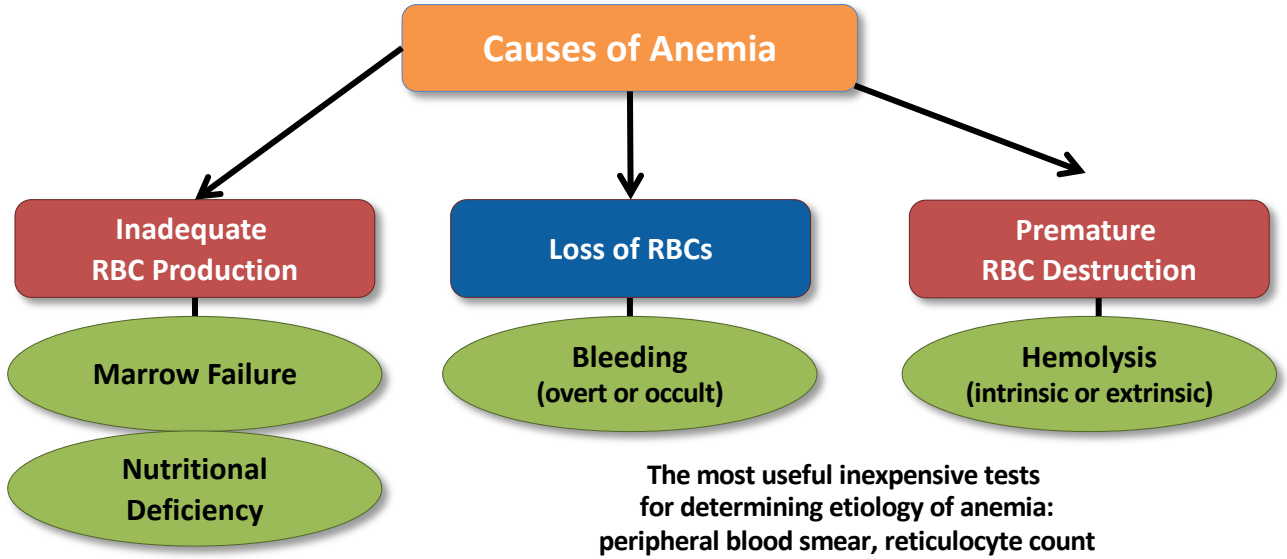


Weill Cornell Medicine NewYork-Presbyterian

Courtesy of Gail J Roboz, MD

Anemia

- Anemia is a sign of disease, not a disease itself
- Dozens of causes, many common
- Organized approach essential
- A specific cause can be found in almost all cases



Wolanskyj AP, et al. Hematology. In: Ficalora RD, ed. *Mayo Clinic Internal Medicine Board Review*. 10th ed. 2013;497-511.

Luspatercept

Mechanism of action

- Binds TGF-β ligands to reduce SMAD2/3 signaling

Indication

- For the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell (RBC) transfusions
- For the treatment of anemia after failure of an erythropoiesis stimulating agent and requiring 2 or more RBC units over 8 weeks in adult patients with very low- to intermediate-risk myelodysplastic syndromes with ring sideroblasts or with myelodysplastic/myeloproliferative neoplasm with ring sideroblasts and thrombocytosis

Recommended dose

- 1 mg/kg once every 3 weeks by subcutaneous injection

Luspatercept package insert, 9/2022.

N Engl J Med 2020;382:140-51.

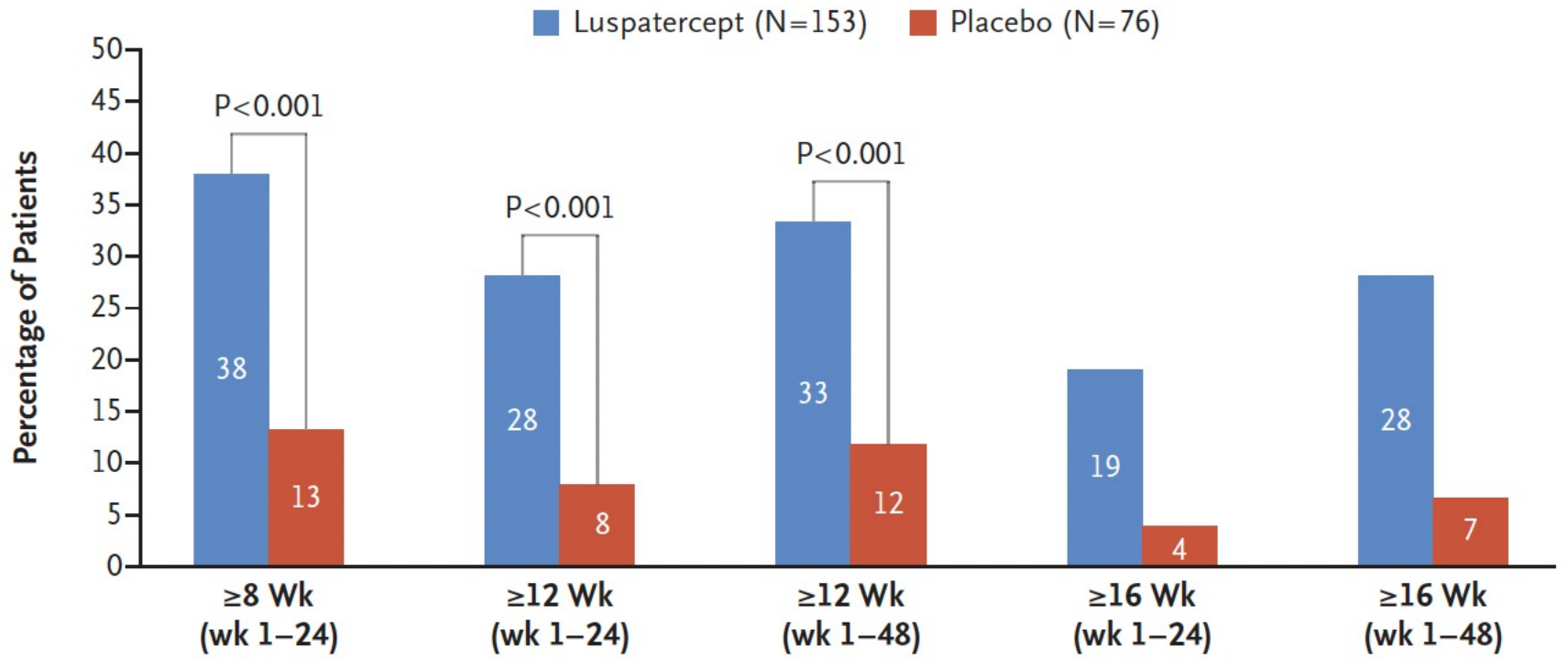
The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Luspatercept in Patients with Lower-Risk Myelodysplastic Syndromes

P. Fenaux, U. Platzbecker, G.J. Mufti, G. Garcia-Manero, R. Buckstein, V. Santini, M. Díez-Campelo, C. Finelli, M. Cazzola, O. Ilhan, M.A. Sekeres, J.F. Falantes, B. Arrizabalaga, F. Salvi, V. Giai, P. Vyas, D. Bowen, D. Selleslag, A.E. DeZern, J.G. Jurcic, U. Germing, K.S. Götze, B. Quesnel, O. Beyne-Rauzy, T. Cluzeau, M.-T. Voso, D. Mazure, E. Vellenga, P.L. Greenberg, E. Hellström-Lindberg, A.M. Zeidan, L. Adès, A. Verma, M.R. Savona, A. Laadem, A. Benzohra, J. Zhang, A. Rampersad, D.R. Dunshee, P.G. Linde, M.L. Sherman, R.S. Komrokji, and A.F. List

MEDALIST: Independence from Red Blood Cell Transfusion



No. of Patients with Response (% [95% CI])					
Luspatercept	58 (38 [30–46])	43 (28 [21–36])	51 (33 [26–41])	29 (19 [13–26])	43 (28 [21–36])
Placebo	10 (13 [6–23])	6 (8 [3–16])	9 (12 [6–21])	3 (4 [1–11])	5 (7 [2–15])

Fenaux P et al. *N Engl J Med* 2020;382:140-51.

Phase III COMMANDS Trial of First-Line Luspatercept-aamt in Patients with Transfusion-Dependent MDS Meets its Primary Endpoint

Press Release: October 31, 2022

The COMMANDS study, a Phase 3, open-label, randomized trial evaluating luspatercept-aamt met its primary endpoint, demonstrating a highly statistically significant and clinically meaningful improvement in red blood cell transfusion independence (RBC-TI) with concurrent hemoglobin (Hb) increase in the first-line treatment of adult patients with very low-, low- or intermediate-risk myelodysplastic syndromes (MDS) who require RBC transfusions. This result was based on a pre-specified interim analysis conducted through an independent review committee. Safety results in the trial were consistent with the safety profile of luspatercept-aamt previously demonstrated in the MEDALIST study (NCT02631070), and no new safety signals were reported.

https://news.bms.com/news/details/2022/Bristol-Myers-Squibb-Announces-Positive-Topline-Results-of-Phase-3-COMMANDS-Trial/default.aspx

blood advances

American Society of Hematology
2021 L Street NW, Suite 900,
Washington, DC 20036
Phone: 202-776-0544 | Fax 202-776-0545
bloodadvances@hematology.org

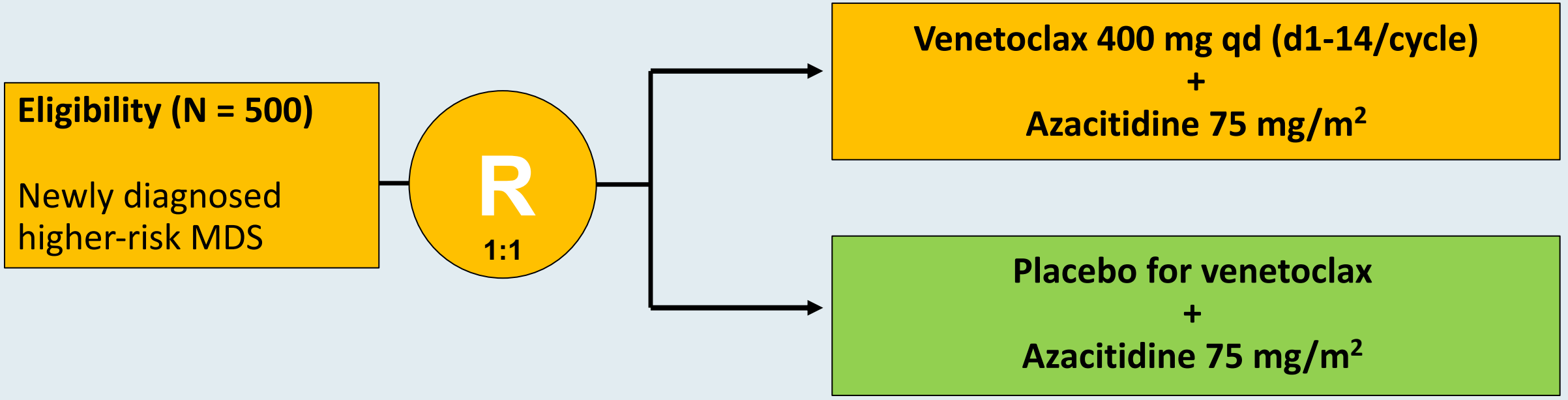
Activity of luspatercept and ESAs combination for treatment of anemia in lower-risk myelodysplastic syndromes

Tracking no: ADV-2023-009781R2

Rami Komrokji (H. Lee Moffitt Cancer Center, United States) Luis E. Aguirre (H. Lee Moffitt Cancer Center, United States) Najla Al-Ali (H. Lee Moffitt Cancer Center, United States) Onyee Chan (Moffitt Cancer Center, United States) Zhuoer Xie (Moffitt Cancer Center, United States) Andrew Kuykendall (H. Lee Moffitt Cancer Center, United States) Kendra Sweet (H. Lee Moffitt Cancer Center, United States) Jeffrey Lancet (Moffitt Cancer Center, United States) Eric Padron (H. Lee Moffitt Cancer Center, United States) David Sallman (Moffitt Cancer Center and Research Institute, United States)

***Blood Adv* 2023;Apr 14 [epub ahead of print].**

VERONA Phase III Study Design



Until relapse, progression or unacceptable toxicity

Dual primary endpoints: Complete remission and overall survival
Secondary endpoints: RBC and platelet transfusion independence for patients who are transfusion dependent at baseline, change from baseline in fatigue, time to deterioration of physical functioning, overall response

Zeidan AM et al. ASCO 2021;Abstract TPS7054.

Agenda

Module 1: Overview of MPNs and MF — Impact of Ruxolitinib

Module 2: New Agents in MF — Fedratinib, Momelotinib, Pacritinib

Module 3: MDS — Management of Low- and High-Risk Disease

Module 4: Overview of AML

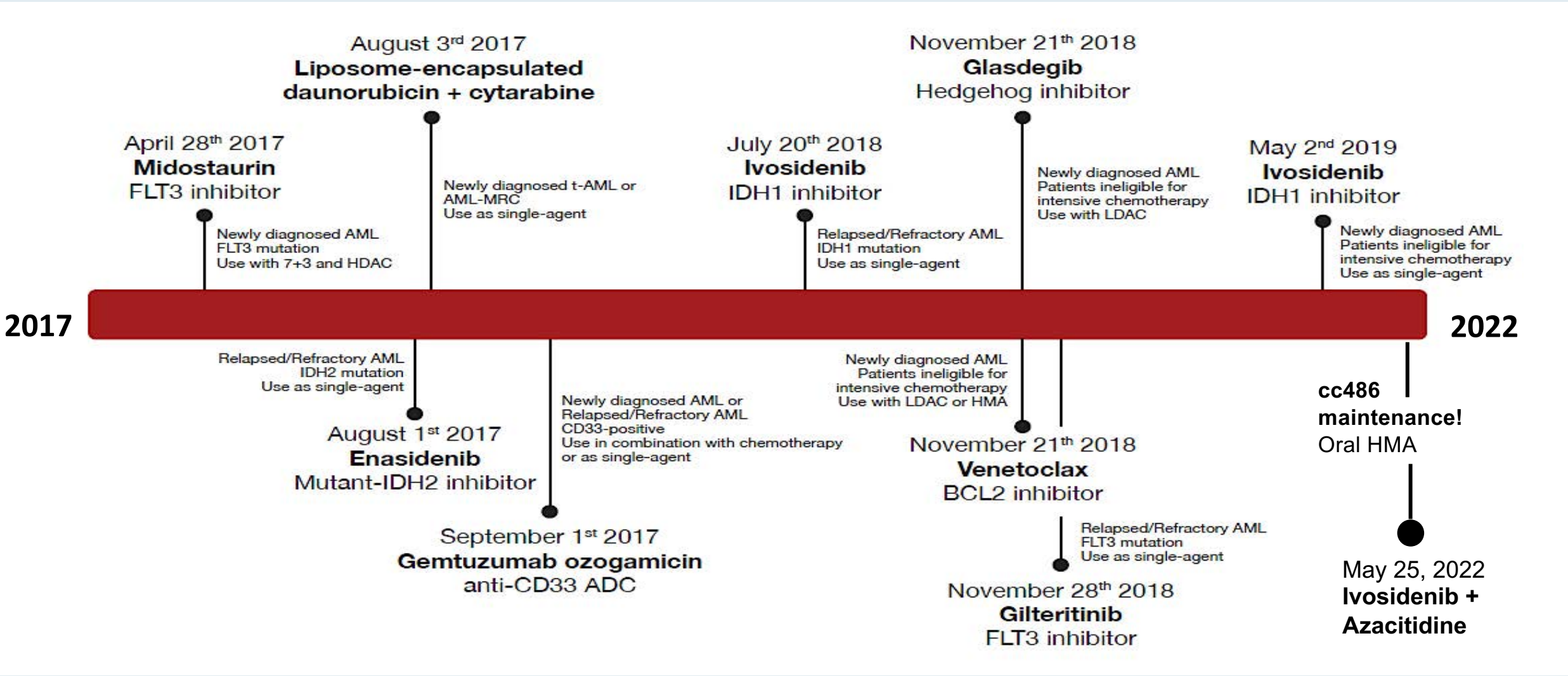
Module 5: Targeted Therapy for AML

Sara M Tinsley-Vance, PhD, APRN, AOCN

89-year-old man with newly diagnosed AML who received venetoclax/azacitidine

RTP
RESEARCH
TO PRACTICE

The Rapidly Evolving Treatment Landscape of AML: FDA Approvals

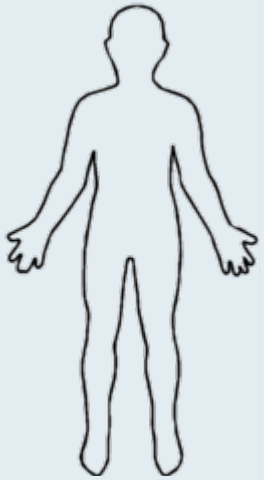


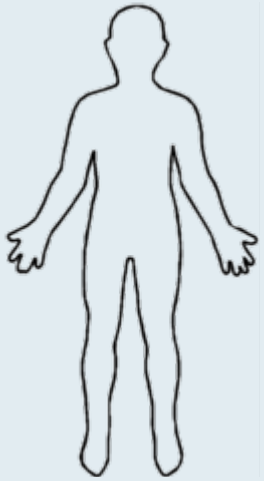
Richard-Carpentier G, DiNardo CD. *Hematology Am Soc Educ Program* 2019(1):548-56.

Content Courtesy of Courtney D DiNardo, MD, MSCE

RTP RESEARCH TO PRACTICE

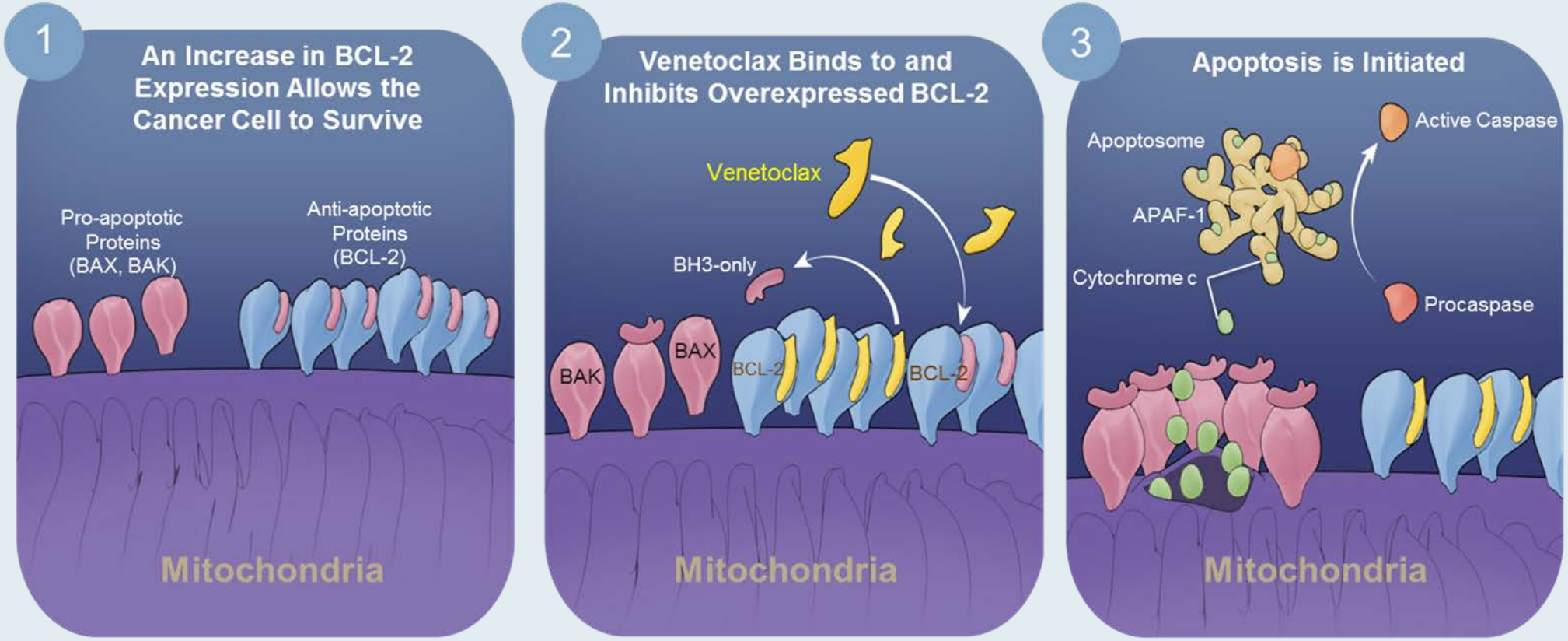
Management of AML in 2023





Courtesy of Andrew M Brunner, MD

Venetoclax Mechanism of Action



- Cancer cells increase the expression of antiapoptotic proteins to offset the increase in proapoptotic proteins, tipping the balance toward cell survival
- The large number of proapoptotic proteins bound and sequestered by Bcl-2 in AML make them “primed” for death

Kumar S et al. ASCO 2015;Abstract 8576.

RTP RESEARCH TO PRACTICE

ASH 2022 | Abstract 219

Long-Term Follow-Up of the Phase 3 VIALE-A Clinical Trial of Venetoclax Plus Azacitidine for Patients With Treatment-Naïve Acute Myeloid Leukemia Ineligible for Intensive Chemotherapy

Keith W. Pratz[1], Brian A. Jonas[2], Vinod Pullarkat[3], Michael J. Thirman[4], Jacqueline S. Garcia[5], Walter Fiedler[6], Kazuhito Yamamoto[7], Jianxiang Wang[8], Sung-Soo Yoon[9], Ofir Wolach[10], Jun-Ho Jang[11], Su-Peng Yeh[12], Grace Ku[13], Catherine Miller[14], Ying Zhou[14], Brenda Chyla[14], Jalaja Potluri[14], Courtney D. DiNardo[15]

VIALE-A Phase III Study Design

Eligibility

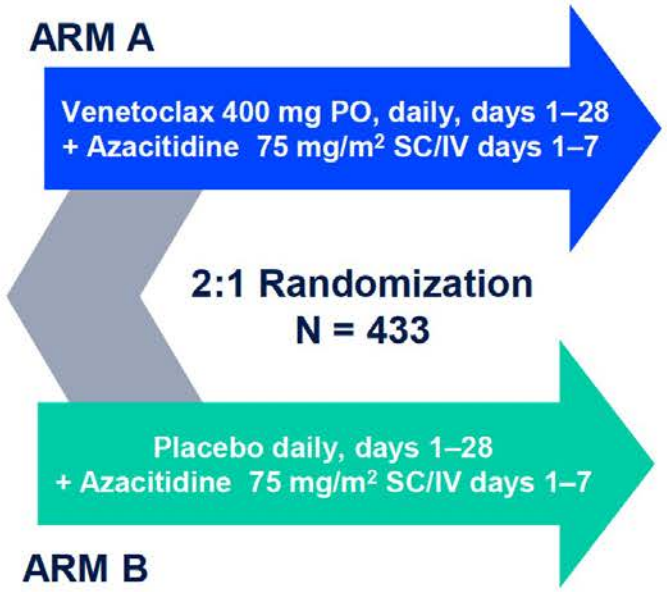
Key Inclusion Criteria

● AML previously untreated ● Age ≥ 75 years or 18-74 years with co-morbidities ineligible for standard induction regimens ● ECOG of 0-2 for pts ≥ 75 years or 0 to 3 for pts ≥ 18-74 years

Key Exclusion Criteria

● Prior receipt of any HMA, Ven, or chemotherapy for MDS
● Favorable risk cytogenetics per NCCN 2016 ● AML secondary to MPN, CML ● Acute promyelocytic leukemia ● Active CNS involvement

Treatment



Randomization Stratification Factors Age (< 75 vs. ≥ 75 years); Cytogenetic Risk (intermediate, poor); Region

Venetoclax dosing ramp-up **Cycle 1 ramp-up** Day 1: 100 mg, Day 2: 200 mg, Day 3 - 28: 400 mg
Cycle 2 Day 1-28: 400 mg

Key Endpoints

Key Primary Endpoints:

● Overall survival (OS)*

Key Secondary Endpoints:

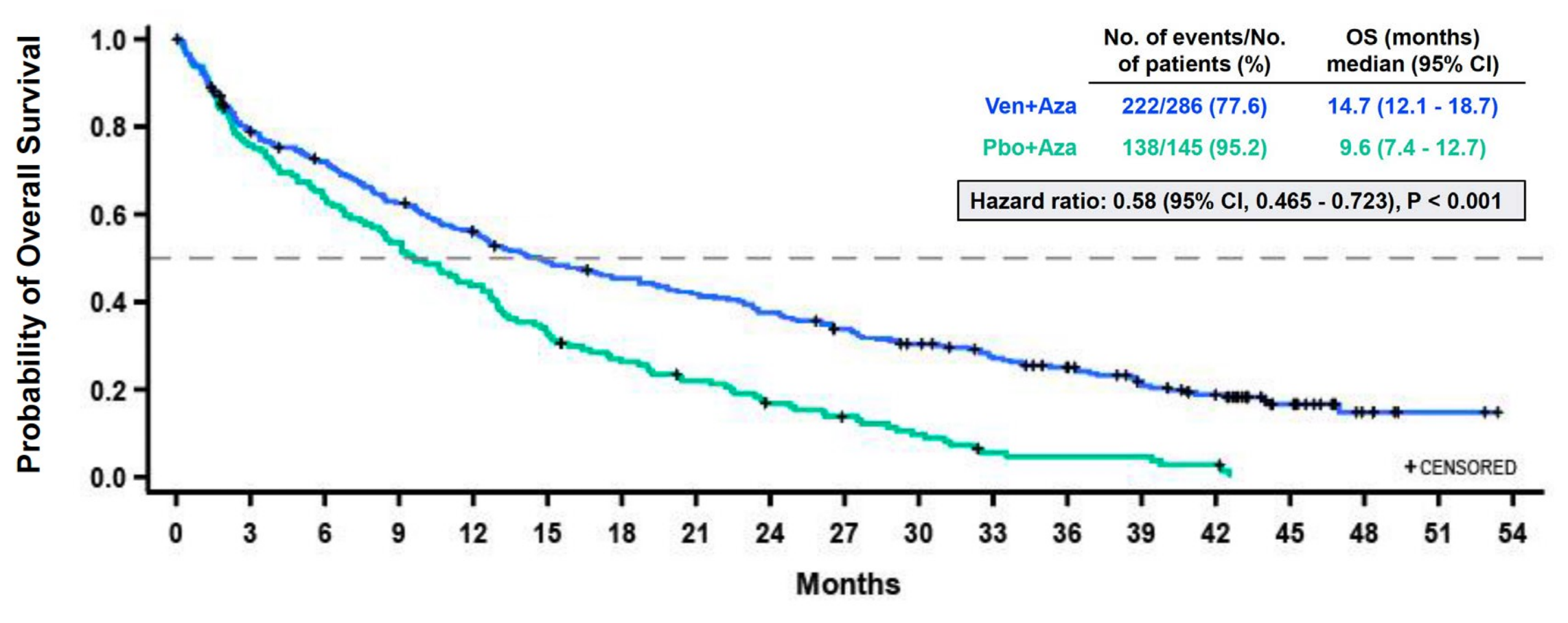
● CR+CRi rate*, CR rate

● OS, CR+CRi in mol. subgroups

● MRD negativity remission rate

Pratz KW et al. ASH 2022;Abstract 219.

VIALE-A: Overall Survival (Median Follow-Up: 43.2 Months)



Pratz KW et al. ASH 2022;Abstract 219.

Agenda

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Module 2: New Agents in MF — Fedratinib, Momelotinib, Pacritinib

Module 3: MDS — Management of Low- and High-Risk Disease

Module 4: Overview of AML

Module 5: Targeted Therapy for AML

Ilene Galinsky, NP

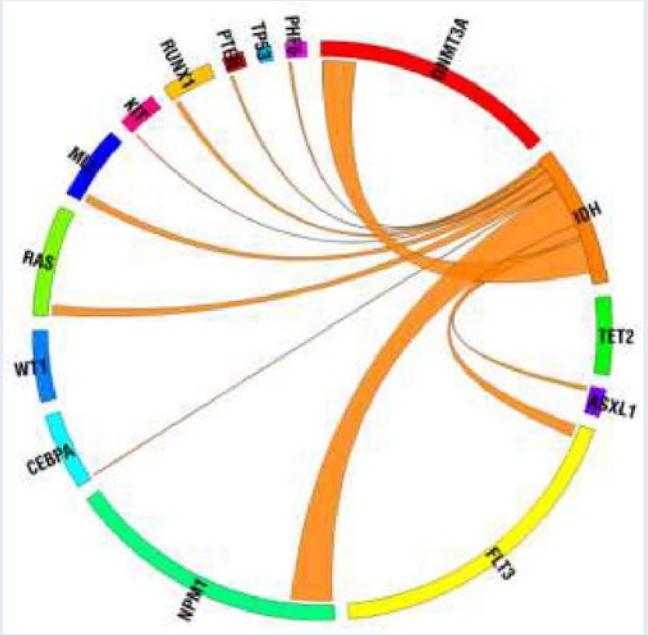
66-year-old man with IDH2 mutation-positive relapsed AML who received enasidenib

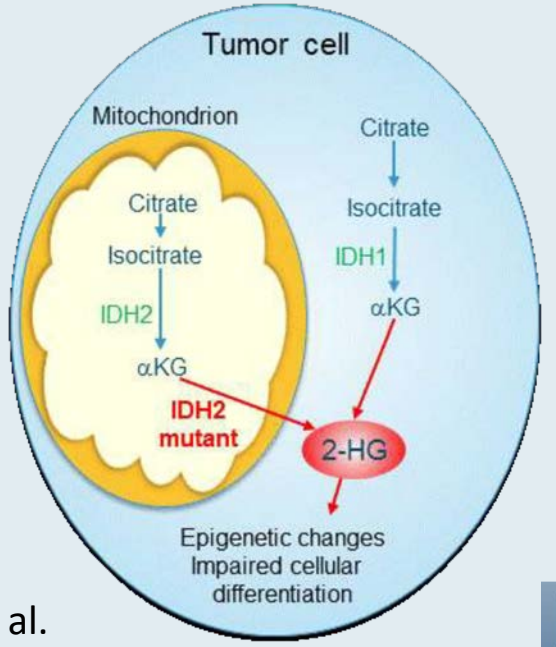
Clinical Research Background

- **Targeted therapy for AML**
  - **Frequency of targeted mutations**
  - **IDH1/2 inhibitors**
  - **FLT3 inhibitors**

IDH in Leukemia

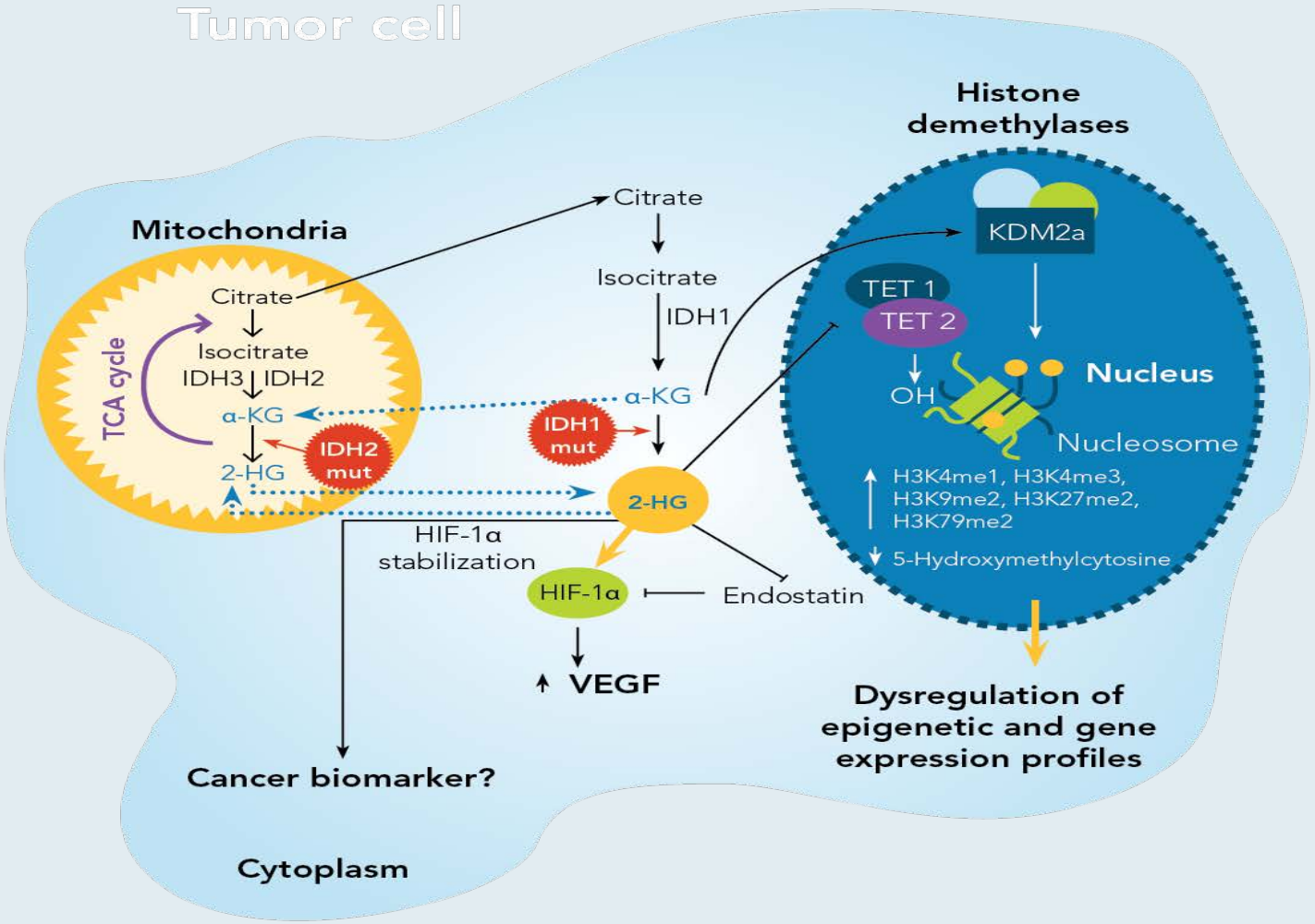
- **IDH mutations occur in ~20% of AML**
  - **Frequency: 6% to 16% IDH1 and 8% to 18% IDH2**
  - **Majority (85%) with diploid or +8 cytogenetics**
  - **↑ prevalence with ↑ patient age**
  - **Prognostic effect in AML remains controversial**
  - **IDH1 and IDH2 mutations may have different effects on prognosis**





Dang L et al. *Trends Mol Med* 2010;16(9):387-97. Chou WC et al. *Leukemia* 2011;25(2):246-53. Patel JP et al. *N Engl J Med* 2012;366(2):1079-89. Medeiros BC et al. *Leukemia* 2017;31:272-81.

IDH in AML



- **IDH1 in cytoplasm and IDH2 in mitochondria**
- **Cancer-associated IDHm produces 2-hydroxyglutarate (R-2-HG)**
- **IDH1/IDH2 mutations induce Bcl-2 dependence (Majeti, *Nat Med*, 2015)**
- **R-2-HG suppresses homologous recombination (Bindra, *Sci Transl Med*, 2017)**

Prensner and Chinnaiyan *Nature*, 2011

RTP RESEARCH TO PRACTICE

Enasidenib

Mechanism of action

- **IDH2 inhibitor**

Indication

- **For the treatment of relapsed or refractory AML with an IDH2 mutation as detected by an FDA-approved test**

Recommended dose

- **100 mg orally once daily until disease progression or unacceptable toxicity**

Enasidenib package insert, 8/2022.

Sara M Tinsley-Vance, PhD, APRN, AOCN

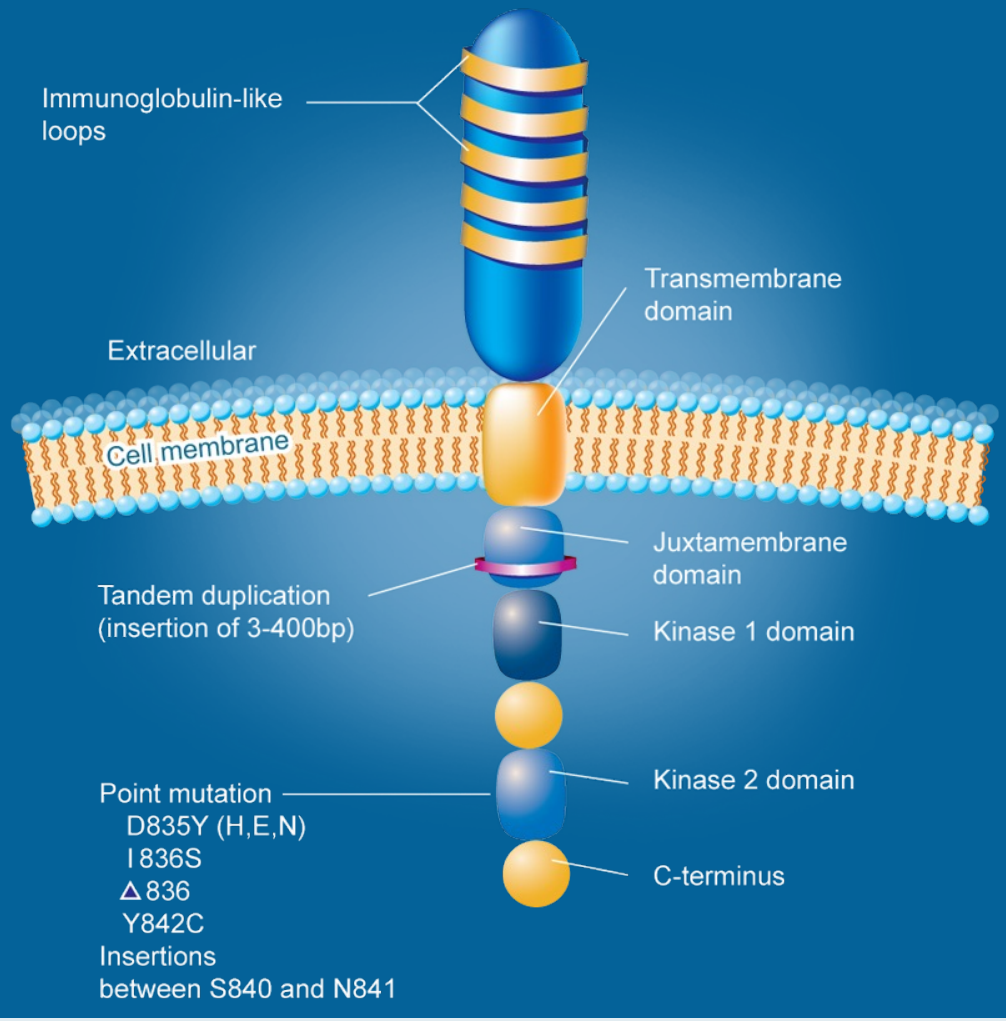
67-year-old man with relapsed FLT3 mutation-positive AML who received oral azacitidine in combination with a FLT3 inhibitor

FLT3 Mutations in AML

Approximately 30% of patients with AML have a FLT3 mutation

FLT3-ITD: 25% of patients with AML

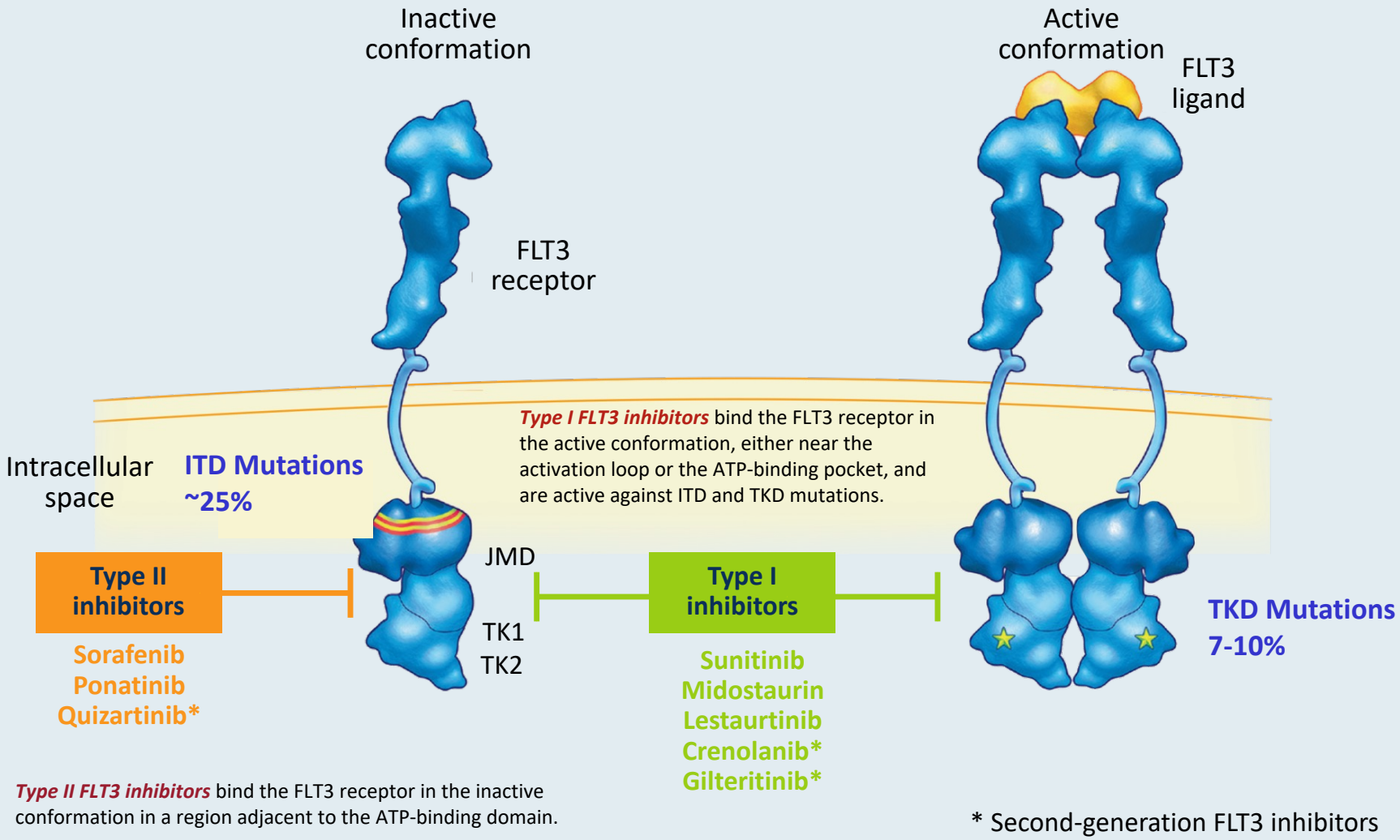
FLT3-TKD: 5% of patients with AML



- **FLT3 ligand (FL) binding activates downstream pathways (↑ cell proliferation)**
- **FLT3 mutations associated with a poor prognosis**

Litzow MR. *Blood* 2005;106:3331-2; Small D. *Hematology Am Soc Hematol Educ Program* 2006:178-84; Swords R et al. *Leukemia* 2012;26(10):2176-85; Griffith J et al. *Mol Cell* 2004;13(2):169-78; Levis M. *Hematology Am Soc Hematol Educ Program* 2013;2013:220-6.

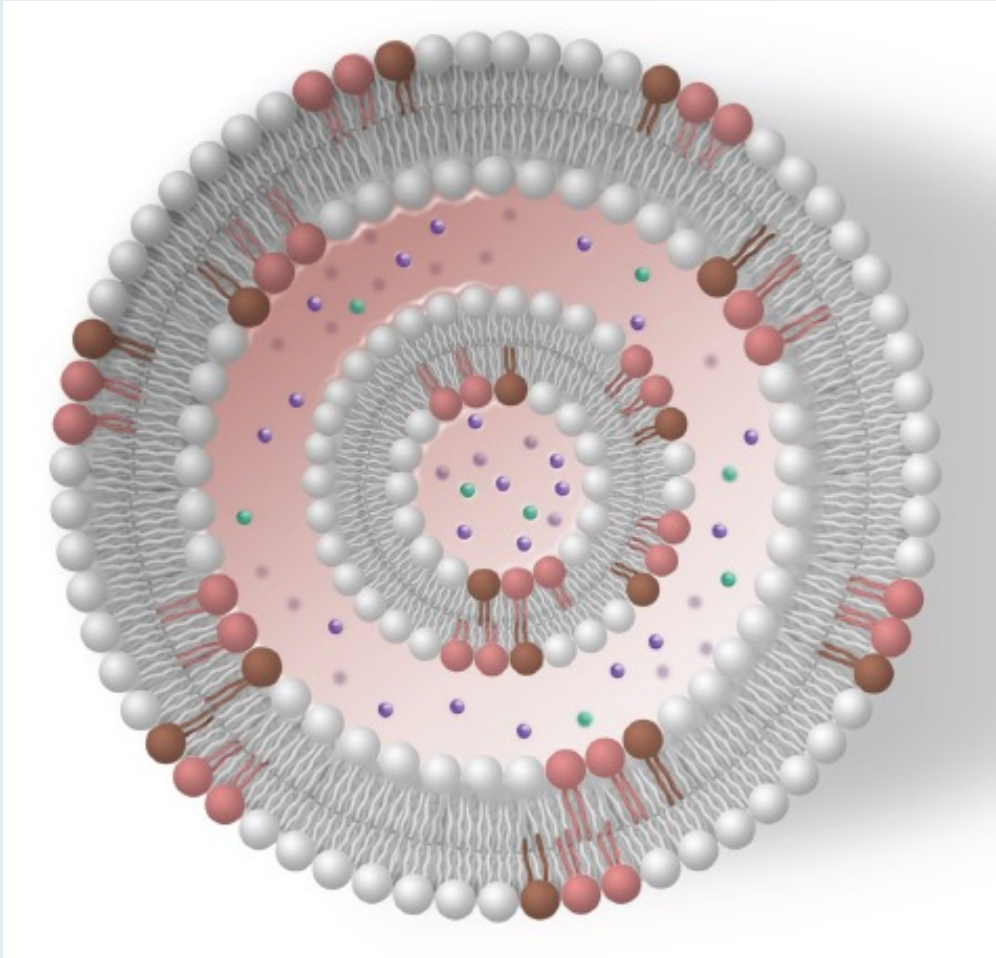
FLT3 Mutations (ITD and TKD) Occur in Approximately 30% to 35% of Patients with AML



Daver N et al. *Leukemia* 2019;33:299-312.

CPX-351

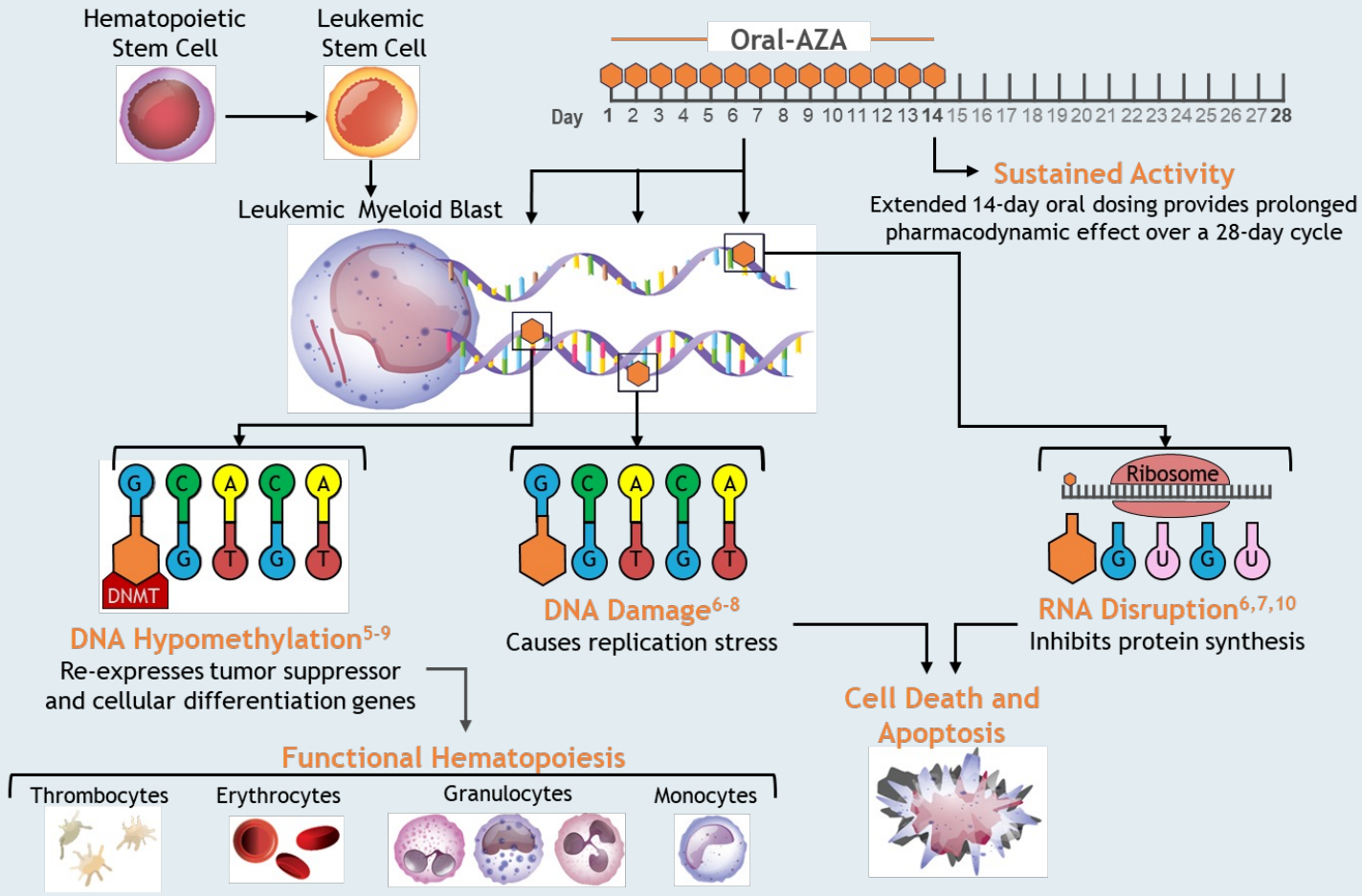
- CPX-351 is a liposomal coformulation of cytarabine and daunorubicin designed to achieve synergistic antileukemia activity
  - 5:1 molar ratio of cytarabine: daunorubicin provides synergistic leukemia cell killing in vitro[1]
  - In patients, CPX-351 preserved delivery of the 5:1 drug ratio for over 24 hours, with drug exposure maintained for 7 days[2]
  - Selective uptake of liposomes by bone marrow leukemia cells in xenograft models[3]



1. Tardi P et al. *Leuk Res.* 2009;33(1):129-139. 2. Feldman EJ et al. *J Clin Oncol.* 2011;29(8):979-985;
3. Lim WS et al. *Leuk Res.* 2010;34(9):1245-1223.

Oral Azacitidine (Oral-AZA, CC-486)

- **Oral hypomethylating agent with a distinct pharmacokinetic/ pharmacodynamic profile from injectable AZA; the two are not bioequivalent[1,2]**
- **Approved in the United States for continued Tx of AML in adult patients who achieved first CR/CRi post-IC and are not able to complete intensive curative therapy (eg, HSCT)[3]**
  - **Oral dosing allows for extended drug exposure during each Tx cycle to prolong AZA activity[1,2]**



1. Garcia-Manero et al. *J Clin Oncol*. 2011;29(18):2521–7. 2. Laille et al. *PLoS One*. 2015;10(8):e0135520. 3. Azacitidine tablets [package insert]. Princeton, NJ: Bristol-Myers Squibb Company; Rev. 9/2020. 4. Savona et al. *Am J Hematol*. 2018;93(10):1199–206. 5. Stresemann et al. *Mol Cancer Ther*. 2008;7:2998–3005. 6. Hollenbach et al. *PLoS One*. 2010;5(2):e9001. 7. Scott LJ. *Drugs*. 2016;76(8):889–900. 8. Stresemann C, Lyko F. *Int J Cancer*. 2008;123(1):8–13. 9. Aimiuwu et al. *Blood*. 2012;119(22):5229–38.

AML, acute myeloid leukemia; AZA, azacitidine; CR, complete remission; CRi, CR with incomplete blood count recovery; HMA, hypomethylating agent; HSCT, hematopoietic stem cell transplant; IC, intensive chemotherapy; PD, pharmacodynamic; PK, pharmacokinetic; pts, patients; Tx, treatment.

Courtesy of Richard M Stone, MD

CC-486 (Oral Azacitidine)

Mechanism of action

- **Oral DNA hypomethylating agent and epigenetic modifier**

Indication

- **For the treatment of acute myeloid leukemia in adult patients who achieved first complete remission or complete remission with incomplete blood count recovery following intensive induction chemotherapy and are not able to complete intensive curative therapy**

Recommended dose

- **300 mg orally once daily on days 1 through 14 of each 28-day cycle**
- **Administer an antiemetic before each dose for at least the first 2 cycles**

CC-486 package insert, 10/2022.

APPENDIX

Myelofibrosis

Ruxolitinib

Verstovsek *et al. Journal of Hematology & Oncology* (2017) 10:156
DOI 10.1186/s13045-017-0527-7

Journal of Hematology & Oncology

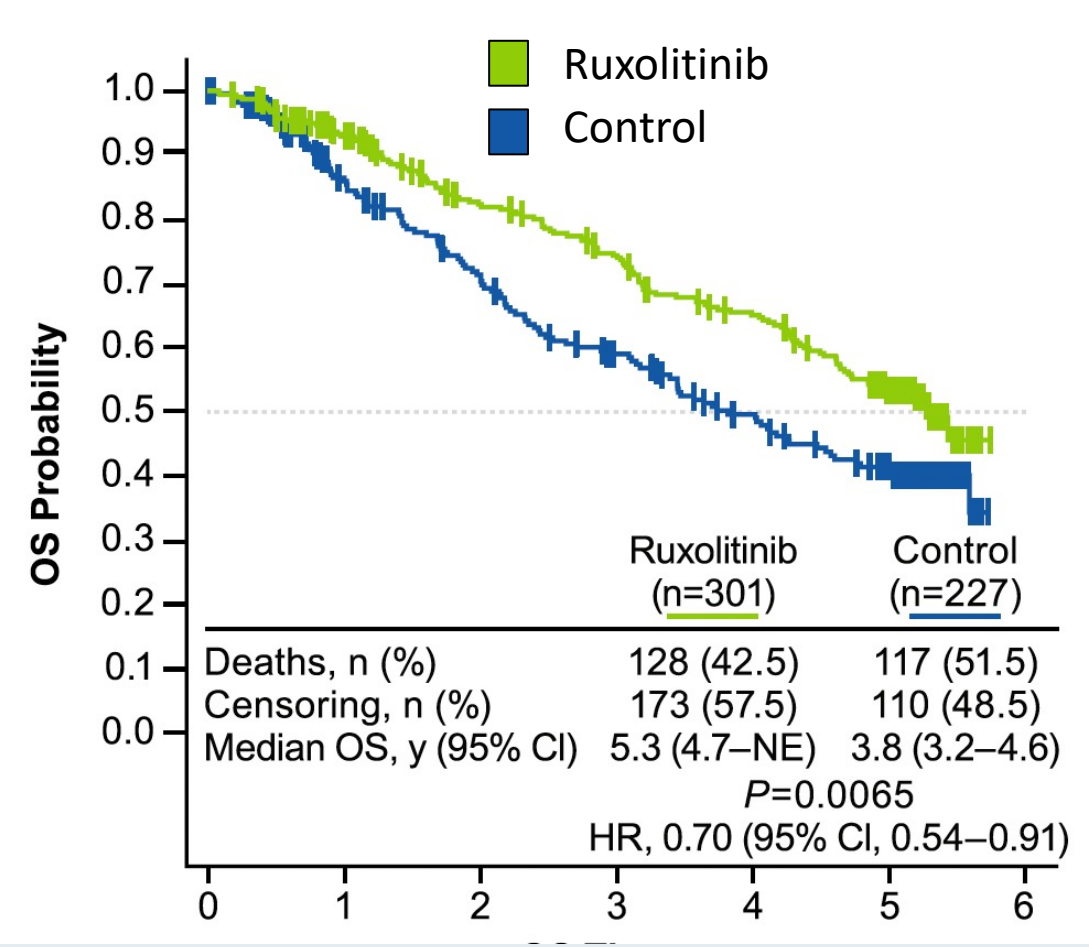
RESEARCH Open Access

Long-term survival in patients treated with ruxolitinib for myelofibrosis: COMFORT-I and -II pooled analyses

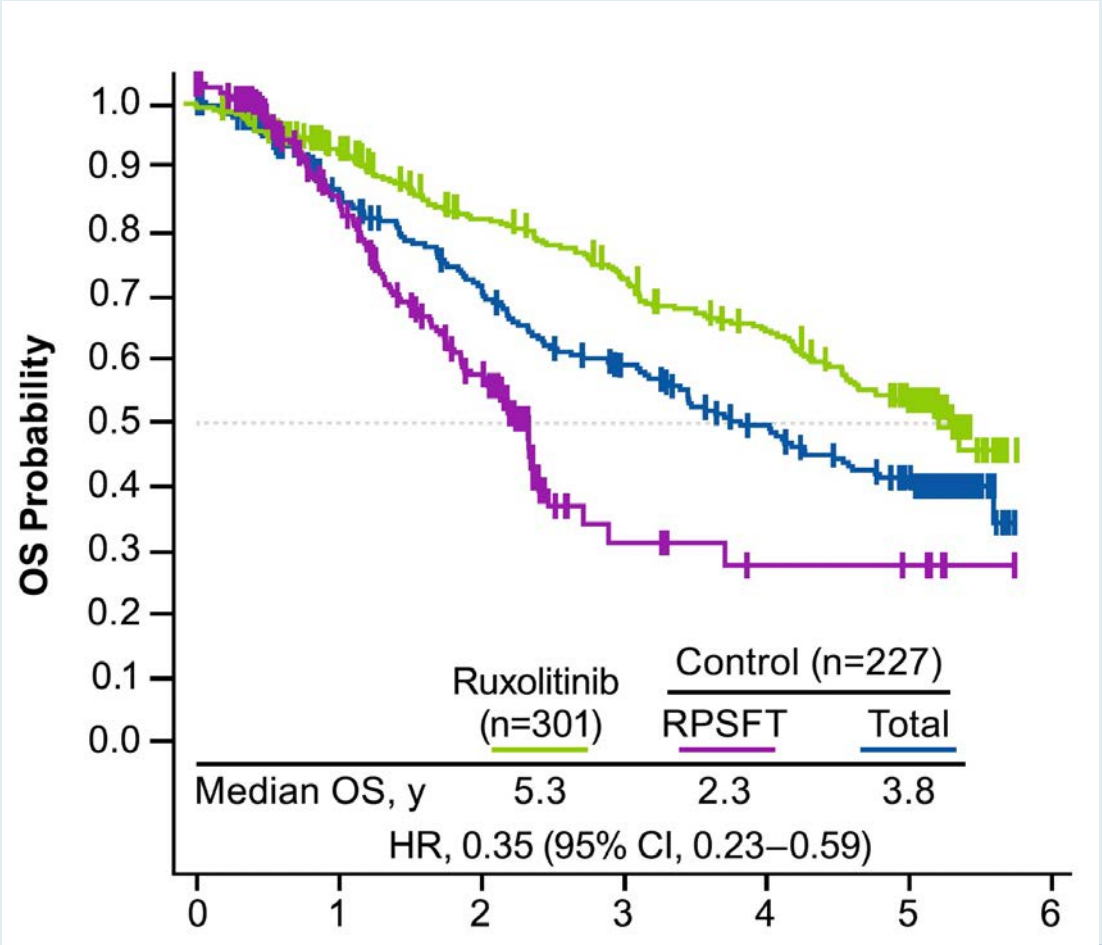
Srdan Verstovsek[1*], Jason Gotlib[2], Ruben A. Mesa[3], Alessandro M. Vannucchi[4], Jean-Jacques Kiladjian[5], Francisco Cervantes[6], Claire N. Harrison[7], Ronald Paquette[8], William Sun[9], Ahmad Naim[9], Peter Langmuir[9], Tuochuan Dong[10], Prashanth Gopalakrishna[11] and Vikas Gupta[12]

COMFORT-I and II Pooled Analyses: Long-Term Survival with Ruxolitinib

OS (5-Year ITT Population)



OS (Corrected for Crossover)



Verstovsek S et al. *J Hematol Oncol* 2017;10:156.

RTP RESEARCH TO PRACTICE

Fedratinib

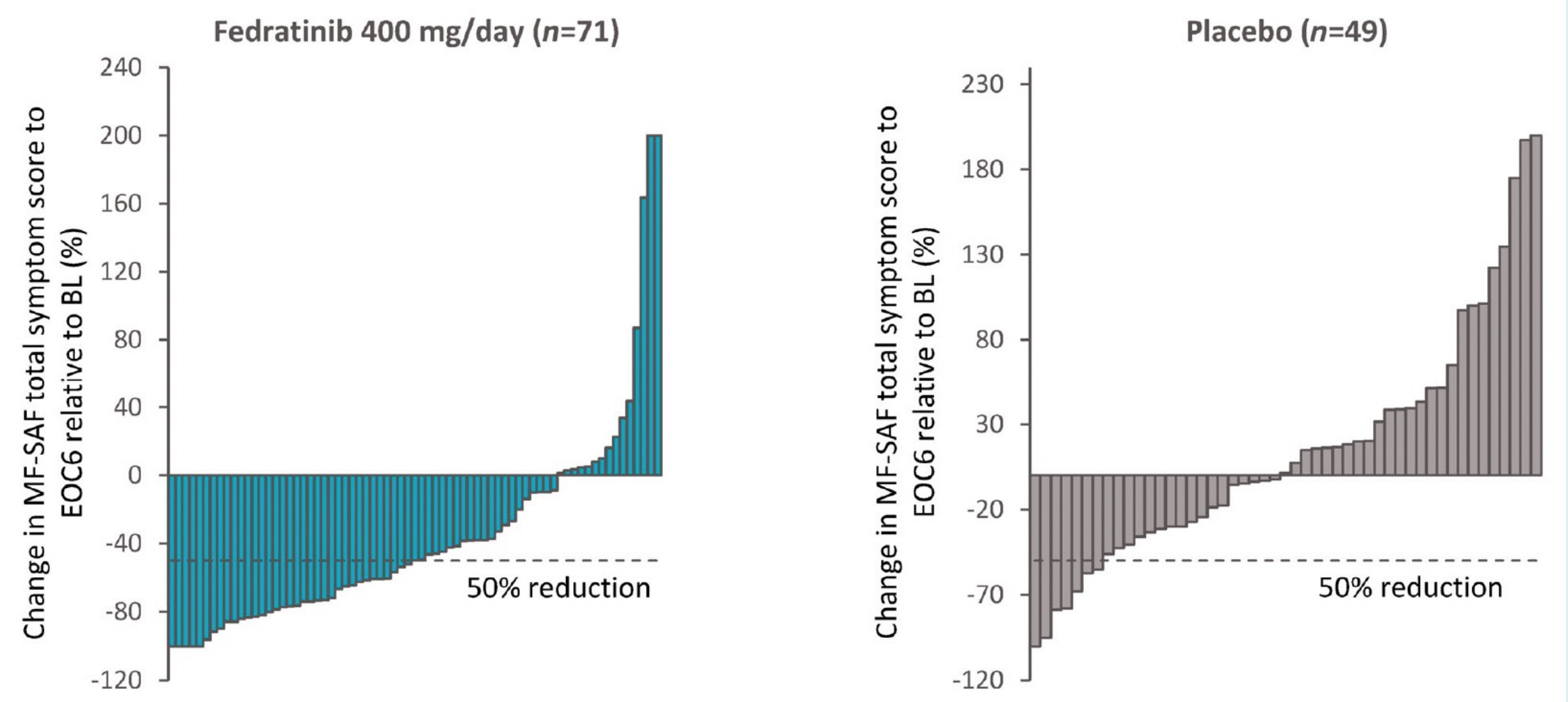
bjh short report

Updated results of the placebo-controlled, phase III JAKARTA trial of fedratinib in patients with intermediate-2 or high-risk myelofibrosis

Animesh Pardanani,[1] Ayalew Tefferi,[1] Tamás Masszi,[2] Elena Mishchenko,[3] Mark Drummond,[4] Eric Jourdan,[5] Alessandro Vannucchi,[6] Mindaugas Jurgutis,[7] Vincent Ribrag,[8] Alessandro Rambaldi,[9,10] Liang Piu Koh,[11] Shelonitda Rose,[12] Jun Zhang[12] and Claire Harrison[13]

Br J Haematol 2021;195:244-8.

JAKARTA: Change in Total Symptom Scores from Baseline to End of Cycle 6



Pardanani A et al. *Br J Haematol* 2021;195:244-8.

JAKARTA: Selected Adverse Events

	Fedratinib 400 mg (n = 96)		Placebo (n = 95)	
Adverse events	**All grades**	**Grade ≥3**	**All grades**	**Grade ≥3**
Diarrhea	66%	5%	16%	0
Nausea	62%	0	15%	0
Anemia	40%	30%	14%	7%
Vomiting	39%	3.1%	5%	0
Fatigue	19%	5%	16%	1.1%
Laboratory parameters				
Anemia	74%	34%	32%	10%
Thrombocytopenia	47%	12%	26%	10%
Neutropenia	23%	5%	13%	3.3%
Biochemistry				
Lipase increased	35%	10%	7%	2.2%

Pardanani A et al. *Br J Haematol* 2021;195:244-8.

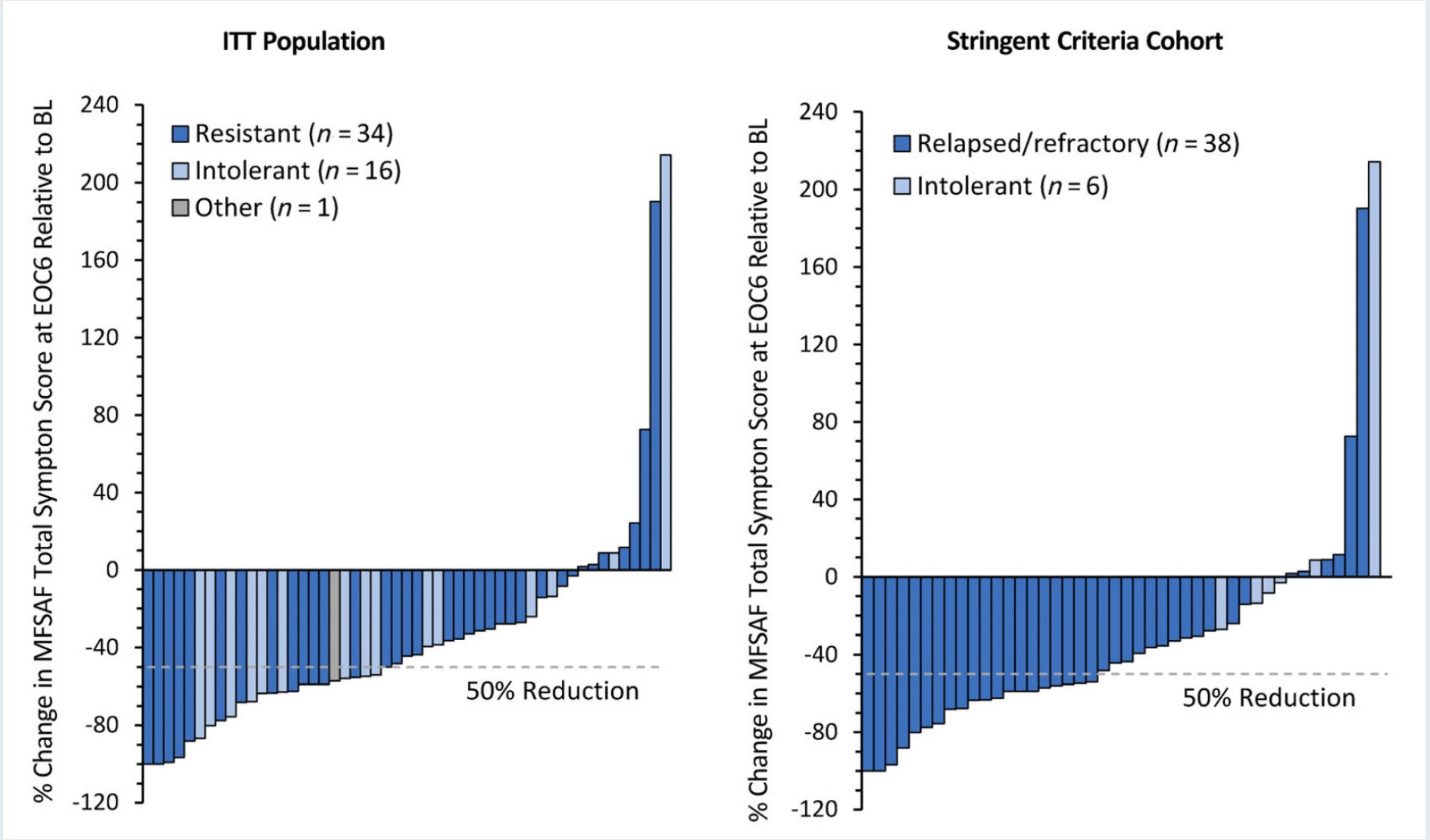
RESEARCH ARTICLE

Am J Hematol 2020;95:594-603

Fedratinib in patients with myelofibrosis previously treated with ruxolitinib: An updated analysis of the JAKARTA2 study using stringent criteria for ruxolitinib failure

Claire N. Harrison[1] | Nicolaas Schaap[2] | Alessandro M. Vannucchi[3] | Jean-Jacques Kiladjian[4] | Eric Jourdan[5] | Richard T. Silver[6] | Harry C. Schouten[7] | Francesco Passamonti[8] | Sonja Zweegman[9] | Moshe Talpaz[10] | Srdan Verstovsek[11] | Shelonitda Rose[12] | Juan Shen[12] | Tymara Berry[12] | Carrie Brownstein[12] | Ruben A. Mesa[13]

JAKARTA-2: Change from Baseline in Symptom Score



Harrison CN et al. *Am J Hematol* 2020;95:594-603.

RTP RESEARCH TO PRACTICE

JAKARTA-2: Treatment-Emergent Adverse Events (ITT Population)

	ITT population (N = 97)	
Preferred term	Any Grade n (%)	Grade 3–4 n (%)
Diarrhea	60 (62)	4 (4)
Nausea	54 (56)	0
Anemia	47 (49)	37 (38)
Thrombocytopenia	26 (27)	21 (22)
Vomiting	40 (41)	0
Constipation	20 (21)	1 (1)
Pruritus	17 (18)	0
Fatigue	15 (16)	2 (2)

No case of Wernicke encephalopathy occurred

Harrison CN et al. *Am J Hematol* 2020;95:594-603.

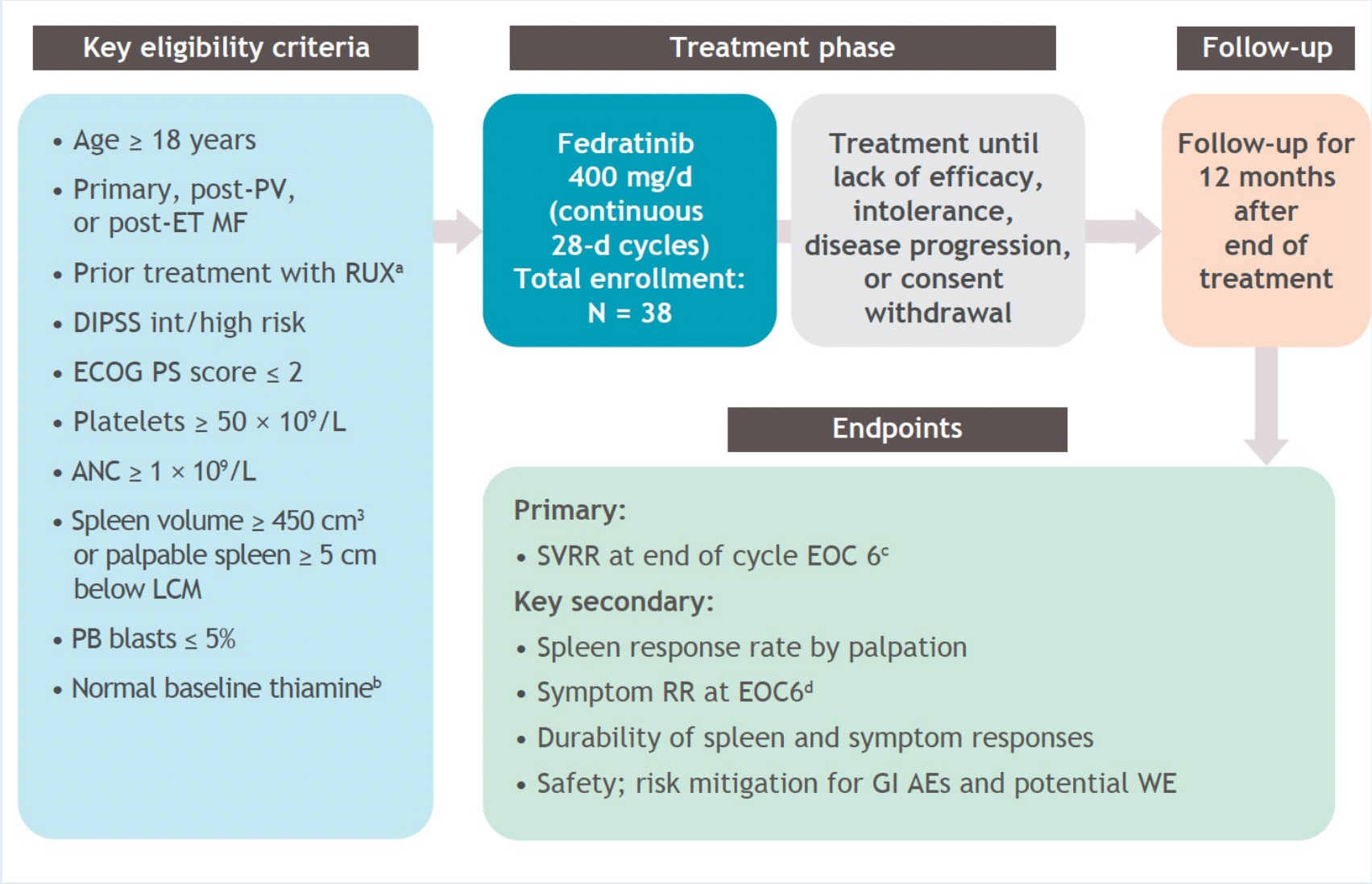
RTP RESEARCH TO PRACTICE

Safety and Efficacy of Fedratinib in Patients with Primary, Post-Polycythemia Vera, and Post-Essential Thrombocythemia Myelofibrosis Previously Treated with Ruxolitinib: Primary Analysis of the FREEDOM Trial

Gupta V et al

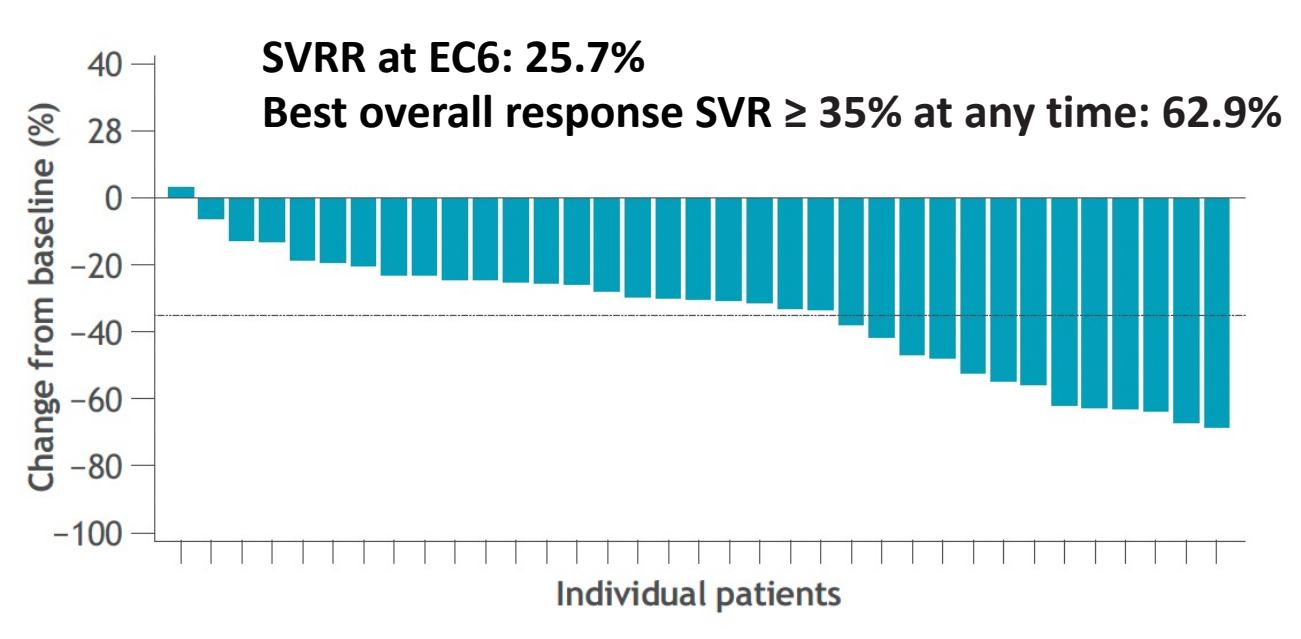
ASH 2022; Abstract 1711

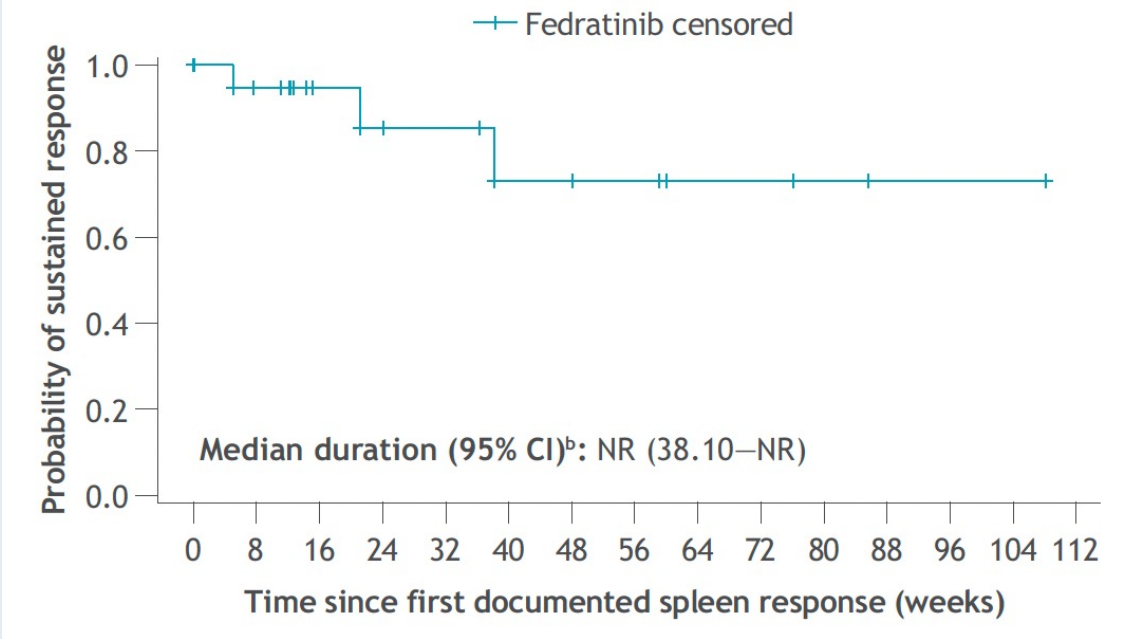
FREEDOM Phase III Study Design



Gupta V et al. ASH 2022;Abstract 1711.

FREEDOM: Change in Baseline Spleen Volume by MRI/CT at EOC6 and Durability of Spleen Size Response

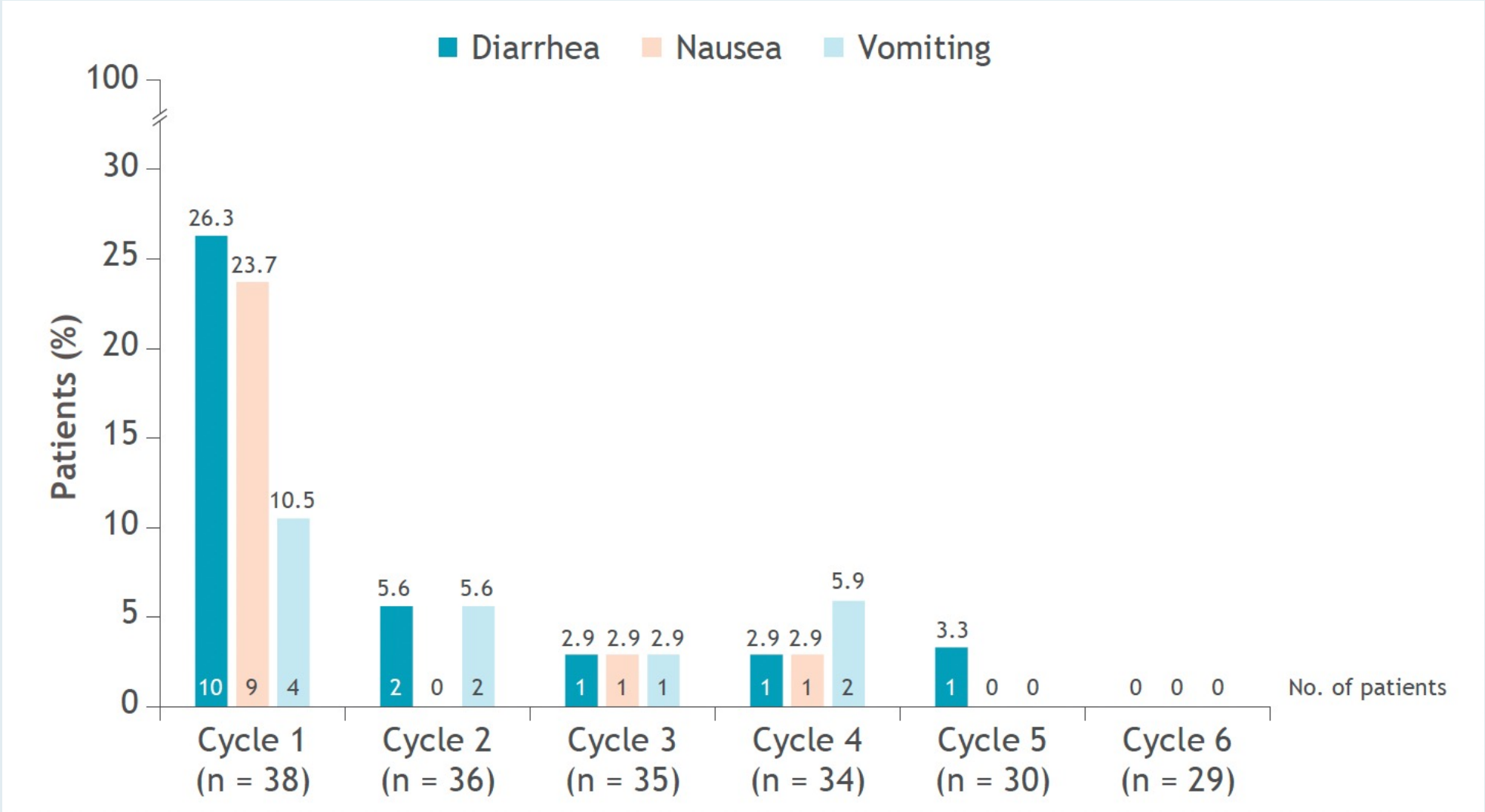




Gupta V et al. ASH 2022;Abstract 1711.

RTP RESEARCH TO PRACTICE

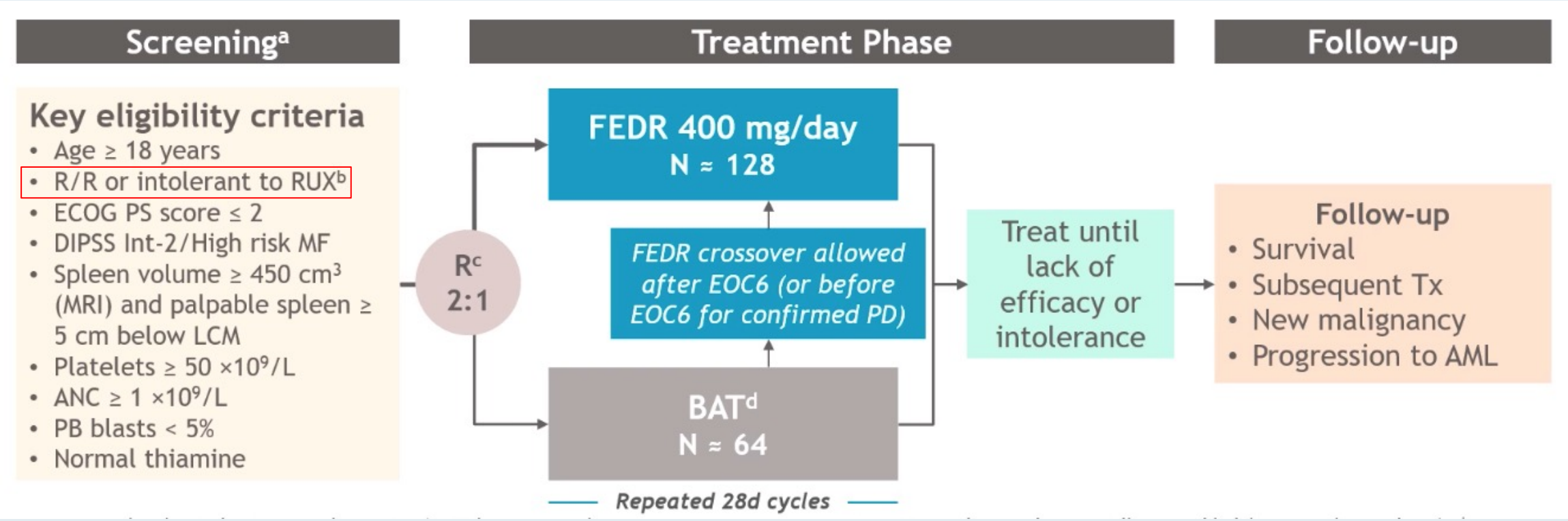
FREEDOM: Gastrointestinal Treatment-Emergent Adverse Events by Treatment Cycle



Gupta V et al. ASH 2022;Abstract 1711.

RTP RESEARCH TO PRACTICE

FREEDOM-2 Phase III Study Design



Vianelli N et al. ASH 2021;Abstract 3643.

Pacritinib Granted Accelerated Approval for Use in Myelofibrosis with Severe Thrombocytopenia

Press Release: February 28, 2022

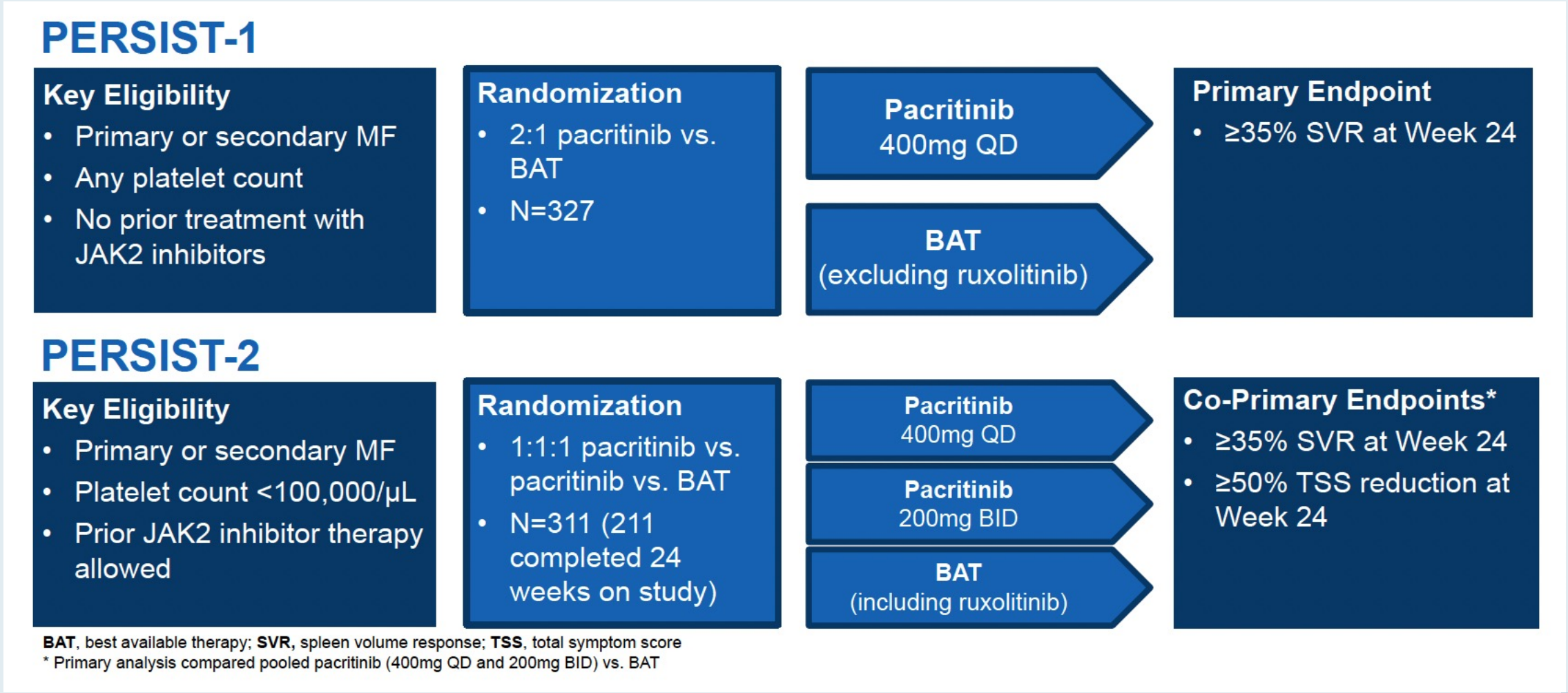
"Pacritinib received accelerated approval from the FDA at a twice daily, 200-mg dose for patients with intermediate- or high-risk primary or secondary myelofibrosis who are experiencing severe thrombocytopenia with a platelet count below 50 × 10^9/L. The agency's decision comes from results of the phase 3 PERSIST-2 study (NCT02055781).

Treatment with pacritinib at 200 mg resulted in a reduction in spleen volume of at least 35% for 29% of patients, vs 3% of patients who received the best available therapy, including ruxolitinib. As part of the post-approval plans for pacritinib, the phase 3 PACIFICA trial (NCT03165734) will be completed with results estimated in 2025.

The PERSIST-2 study, which assessed the use of pacritinib compared with best available therapy in patients with myelofibrosis and thrombocytopenia, enrolled 311 patients. Those who enrolled were randomized into 1 of 3 treatment regimens, including pacritinib once daily (n = 104), pacritinib twice daily (n = 107), or a best alternative treatment (n = 100). Best alternative treatments included ruxolitinib (45%), hydroxyurea (19%), and prednisone and/or prednisolone (13%).

https://www.cancernetwork.com/view/pacritinib-granted-accelerated-approval-for-use-in-myelofibrosis-with-severe-thrombocytopenia

Phase III PERSIST-1 and PERSIST-2 Study Designs



BAT, best available therapy; **SVR,** spleen volume response; **TSS**, total symptom score
* Primary analysis compared pooled pacritinib (400mg QD and 200mg BID) vs. BAT

Mesa RA et al. ASH 2019;Abstract 4195.

Research

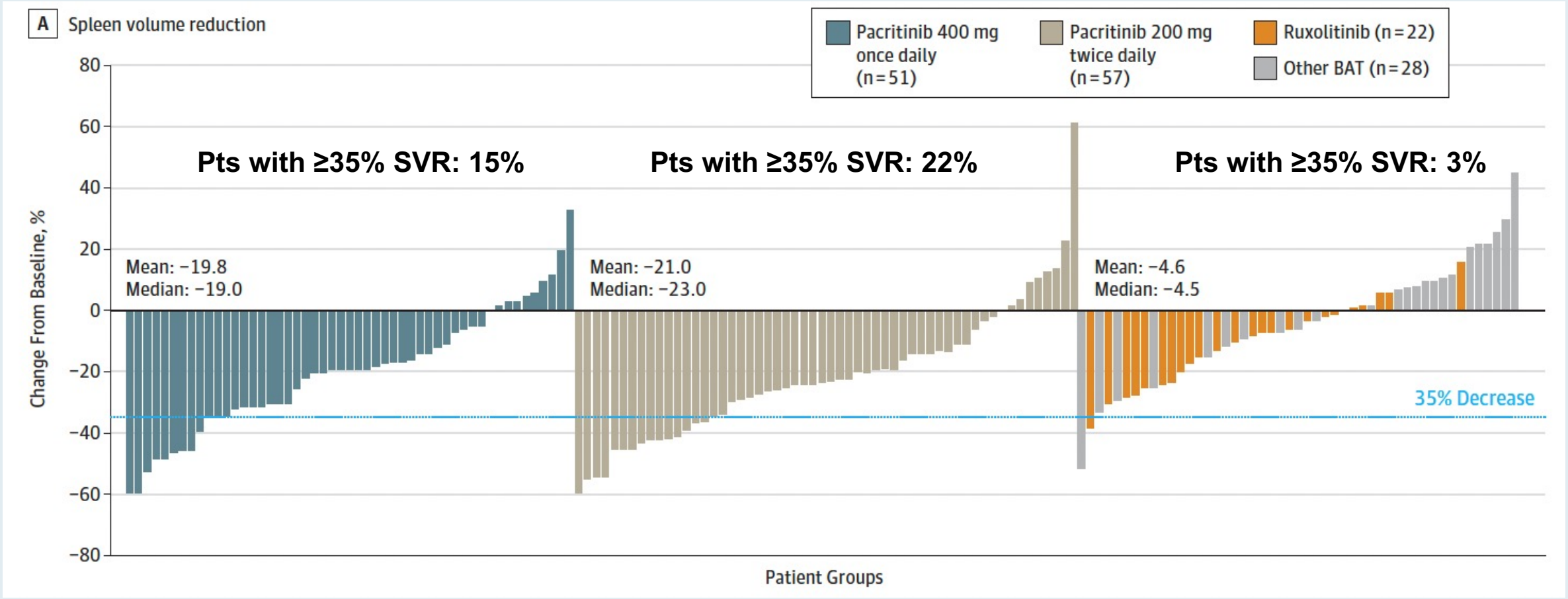
JAMA Oncol 2018;4(5):652-9.

JAMA Oncology | Original Investigation

Pacritinib vs Best Available Therapy, Including Ruxolitinib, in Patients With Myelofibrosis
A Randomized Clinical Trial

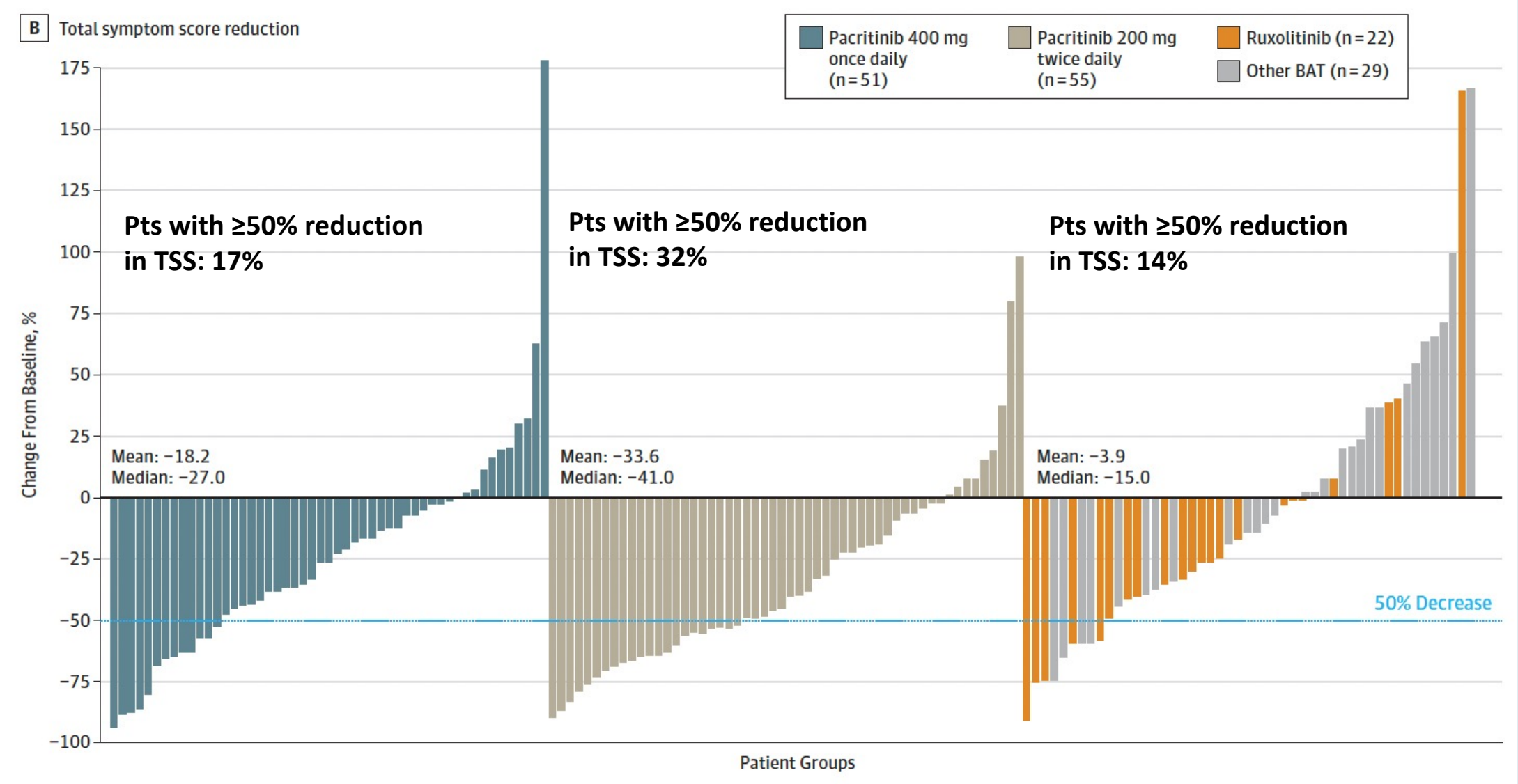
John Mascarenhas, MD; Ronald Hoffman, MD; Moshe Talpaz, MD; Aaron T. Gerds, MD; Brady Stein, MD; Vikas Gupta, MD, FRCP, FRCPath; Anita Szoke, MD; Mark Drummond, MBChB, PhD, FRCPath; Alexander Pristupa, MD; Tanya Granston, PhD; Robert Daly, PhD; Suliman Al-Fayoumi, PhD; Jennifer A. Callahan, MS; Jack W. Singer, MD; Jason Gotlib, MD; Catriona Jamieson, MD, PhD; Claire Harrison, MD, DM, FRCP, PRCPath; Ruben Mesa, MD, FACP; Srdan Verstovsek, MD, PhD

PERSIST-2: Spleen Volume Reduction



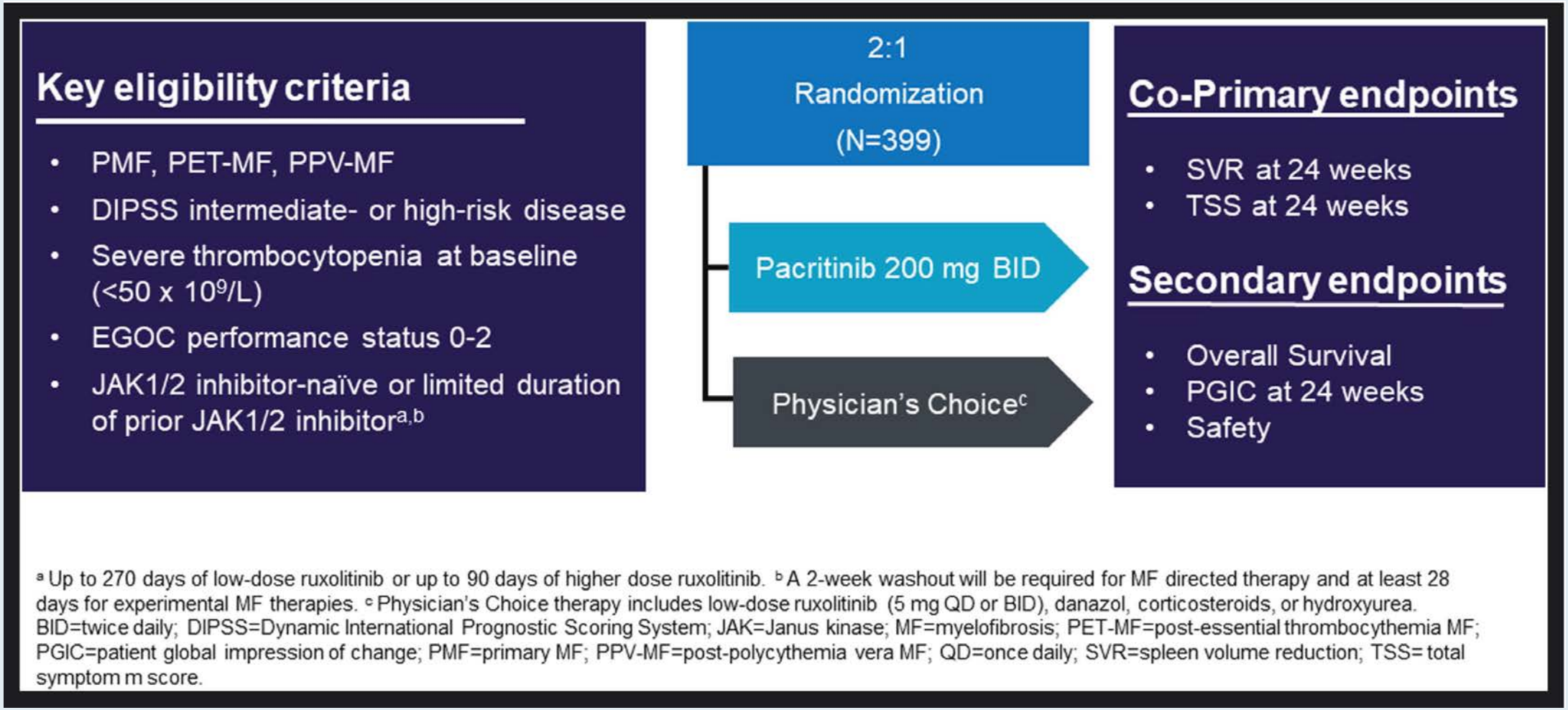
Mascarenhas J et al. *JAMA Oncol* 2018;4(5):652-9.

PERSIST-2: Reduction in Total Symptom Score



Mascarenhas J et al. *JAMA Oncol* 2018;4(5):652-9.

PACIFICA (PAC303) Phase III Study Design

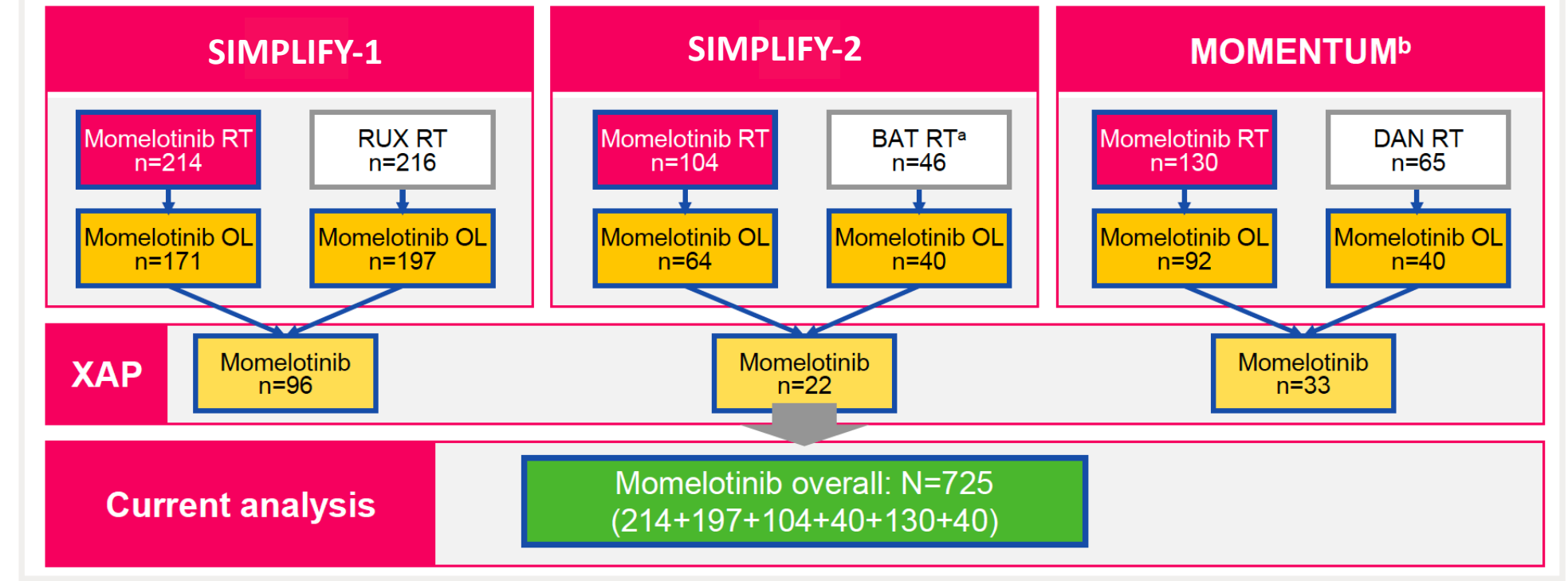


[a] Up to 270 days of low-dose ruxolitinib or up to 90 days of higher dose ruxolitinib. [b] A 2-week washout will be required for MF directed therapy and at least 28 days for experimental MF therapies. [c] Physician's Choice therapy includes low-dose ruxolitinib (5 mg QD or BID), danazol, corticosteroids, or hydroxyurea.
BID=twice daily; DIPSS=Dynamic International Prognostic Scoring System; JAK=Janus kinase; MF=myelofibrosis; PET-MF=post-essential thrombocythemia MF; PGIC=patient global impression of change; PMF=primary MF; PPV-MF=post-polycythemia vera MF; QD=once daily; SVR=spleen volume reduction; TSS= total symptom m score.

Mascarenhas J et al. ASH 2022.

Momelotinib: Significant Anemia and/or Transfusion Dependence

Long-Term Safety with Momelotinib: Pooled Data from 3 Phase III Randomized-Controlled Trials

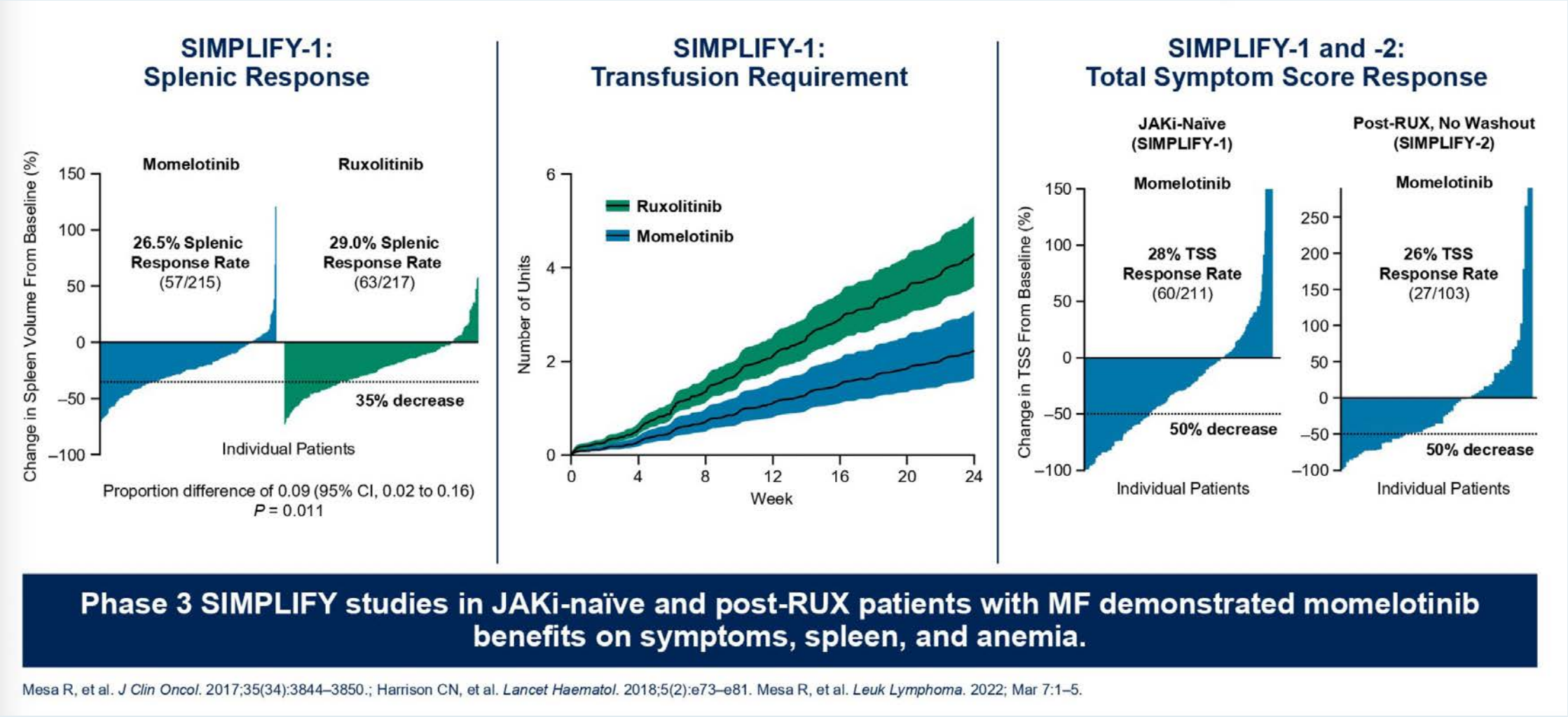


[a]Forty-six patients received at least 1 dose of RUX. [b]Rollover to XAP was not complete at time of data cut.
BAT, best available therapy; DAN, danazol; JAK, Janus kinase; JAKi, Janus kinase inhibitor; OL, open-label; RT, randomized treatment; RUX, ruxolitinib; S-1, SIMPLIFY-1; S-2, SIMPLIFY-2; XAP, extended access protocol.

- Median follow-up time was 20 months in S-1, 10 months in S-2, and 7 months in MOMENTUM
- The total follow-up time was 1261 patient-years in the 725 patients

Verstovsek S et al. ASH 2022;Abstract 4348.

Overview of Phase III SIMPLIFY-1 and SIMPLIFY-2 Study Results



Phase 3 SIMPLIFY studies in JAKi-naïve and post-RUX patients with MF demonstrated momelotinib benefits on symptoms, spleen, and anemia.

Mesa R, et al. *J Clin Oncol*. 2017;35(34):3844–3850.; Harrison CN, et al. *Lancet Haematol*. 2018;5(2):e73–e81. Mesa R, et al. *Leuk Lymphoma*. 2022; Mar 7:1–5.

Mesa R et al. ASCO 2022;Abstract 7002.

RTP RESEARCH TO PRACTICE

Leukemia 2022;36:2261–68

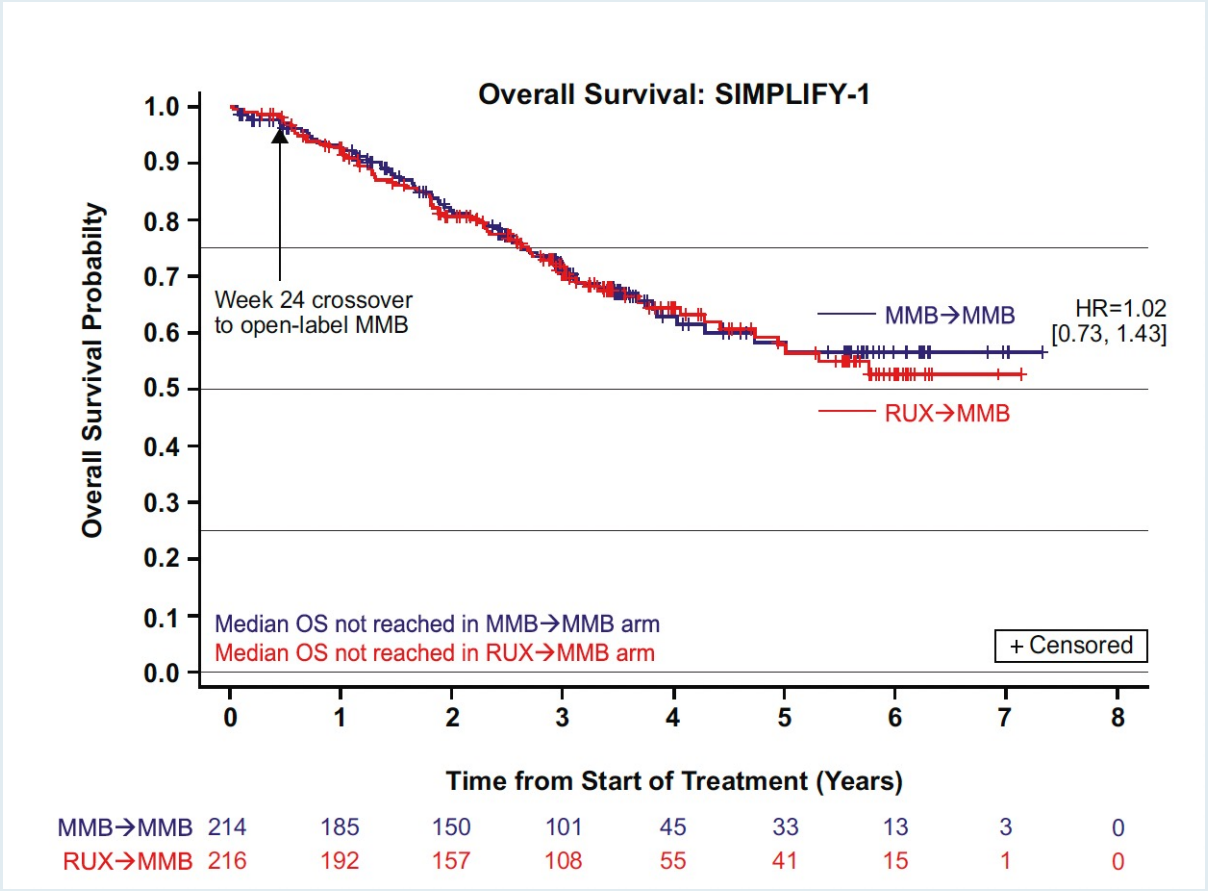
ARTICLE OPEN

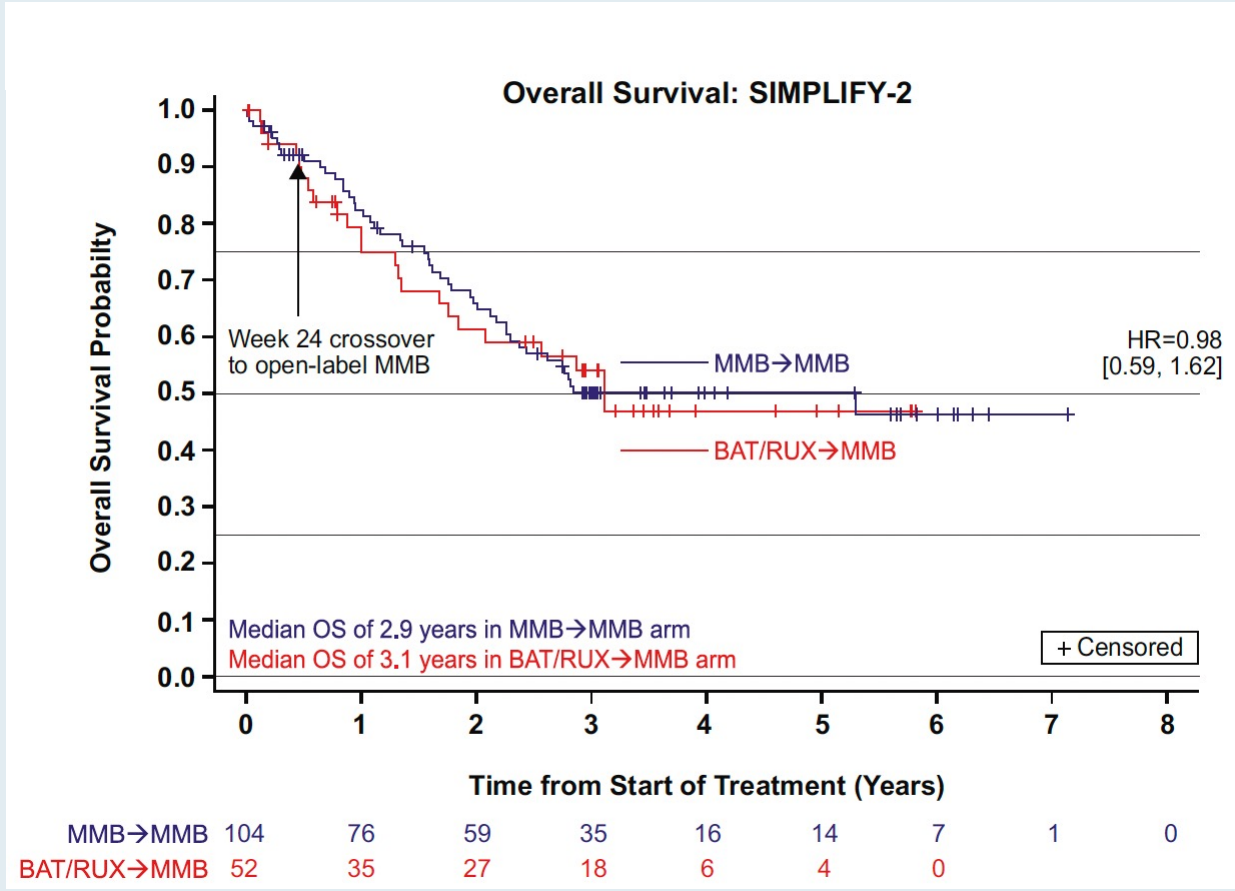
CHRONIC MYELOPROLIFERATIVE NEOPLASMS

Overall survival in the SIMPLIFY-1 and SIMPLIFY-2 phase 3 trials of momelotinib in patients with myelofibrosis

Ruben Mesa[1]✉, Claire Harrison[2], Stephen T. Oh[3], Aaron T. Gerds[4], Vikas Gupta[5], John Catalano[6], Francisco Cervantes[7], Timothy Devos[8], Marek Hus[9], Jean-Jacques Kiladjian[10], Ewa Lech-Maranda[11], Donal McLornan[2], Alessandro M. Vannucchi[12], Uwe Platzbecker[13], Mei Huang[14], Bryan Strouse[14], Barbara Klencke[14] and Srdan Verstovsek[15]

Overall Survival in the SIMPLIFY-1 and SIMPLIFY-2 Phase III Trials of Momelotinib in Myelofibrosis





Mesa R et al. *Leukemia* 2022;36:2261-8.

Momelotinib Yields Statistically Significant Improvement in Symptoms for Myelofibrosis

Press Release: January 27, 2022

"Topline findings from the phase 3 MOMENTUM study indicated that patients with myelofibrosis experienced a statistically significant reduction in symptoms following treatment with momelotinib.

Treatment with momelotinib resulted in a statistically significant reduction in symptoms for patients with myelofibrosis, according to a press release of the topline findings from the pivotal phase 3 MOMENTUM trial (NCT04173494).

In a population of 195 patients, specifically 130 who received momelotinib and 65 who received danazol, 25% and 9% of patients, respectively, had a total symptom score of more than 50% (*P* = .0095). Additionally, 31% of patients in the momelotinib arm and 20% in the control arm were transfusion independent following treatment (one-sided *P* = .0064), indicating non-inferiority. Investigators also reported a splenic response rate of over 35% in 23% of patients in the experimental arm compared with 3% of patients in the control arm (*P* = .0006).

The full data are set to be presented at an upcoming medical meeting and plans have been put in place to submit a new drug application for the agent in the second quarter of 2022."

https://www.cancernetwork.com/view/momelotinib-yields-statistically-significant-improvement-in-symptoms-for-myelofibrosis

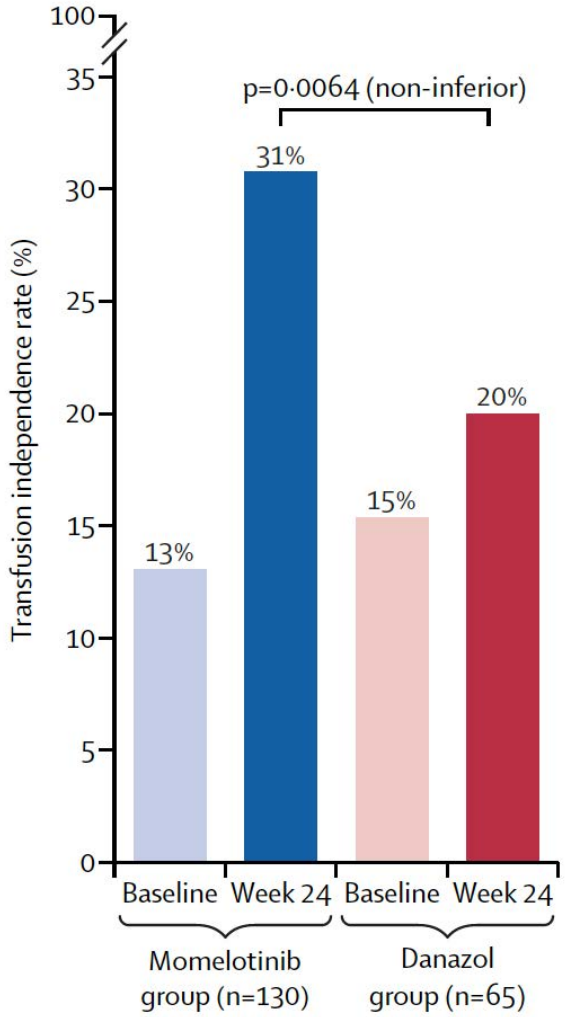
RTP RESEARCH TO PRACTICE

Lancet 2023;401-269-80

Momelotinib versus danazol in symptomatic patients with anaemia and myelofibrosis (MOMENTUM): results from an international, double-blind, randomised, controlled, phase 3 study

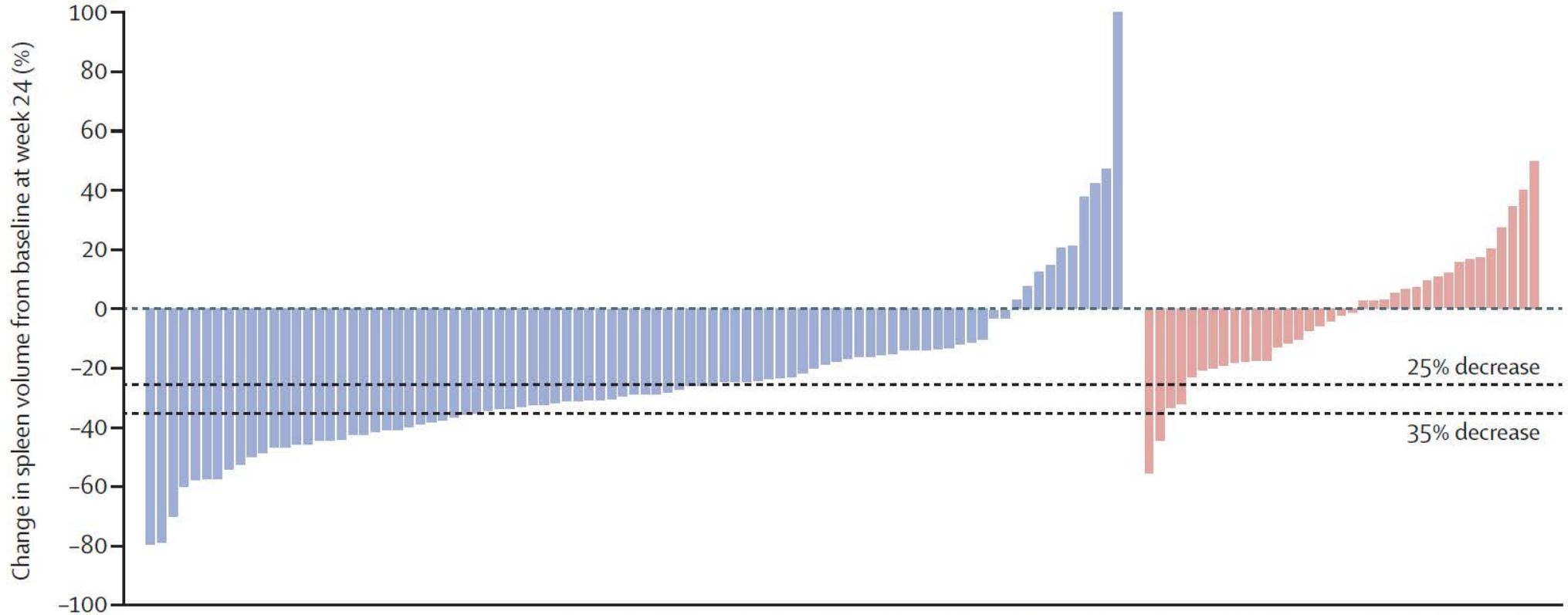
*Srdan Verstovsek, Aaron T Gerds, Alessandro M Vannucchi, Haifa Kathrin Al-Ali, David Lavie, Andrew T Kuykendall, Sebastian Grosicki, Alessandra Iurlo, Yeow Tee Goh, Mihaela C Lazaroiu, Miklos Egyed, Maria Laura Fox, Donal McLornan, Andrew Perkins, Sung-Soo Yoon, Vikas Gupta, Jean-Jacques Kiladjian, Nikki Granacher, Sung-Eun Lee, Luminita Ocroteala, Francesco Passamonti, Claire N Harrison, Barbara J Klencke, Sunhee Ro, Rafe Donahue, Jun Kawashima, Ruben Mesa, on behalf of MOMENTUM Study Investigators**

MOMENTUM: Change in Transfusion Independence Rate from Baseline to Week 24



Verstovsek S et al. *Lancet* 2023;401:269-80.

MOMENTUM: Percentage Change of Spleen Volume from Baseline to Week 24 for Each Patient



	Momelotinib group (n=130)	Danazol group (n=65)	Proportion difference (95% CI)	p value
Number meeting at least 25% reduction in spleen volume	52 (40% [95% CI 32–49])	4 (6% [95% CI 2–15])	34% (24–45)	<0·0001
Number meeting at least 35% reduction in spleen volume	30 (23% [95% CI 16–31])	2 (3% [95% CI 0–11])	19% (11–28)	0·0006

Verstovsek S et al. *Lancet* 2023;401:269-80.

RTP RESEARCH TO PRACTICE

MOMENTUM: Treatment-Emergent Adverse Events

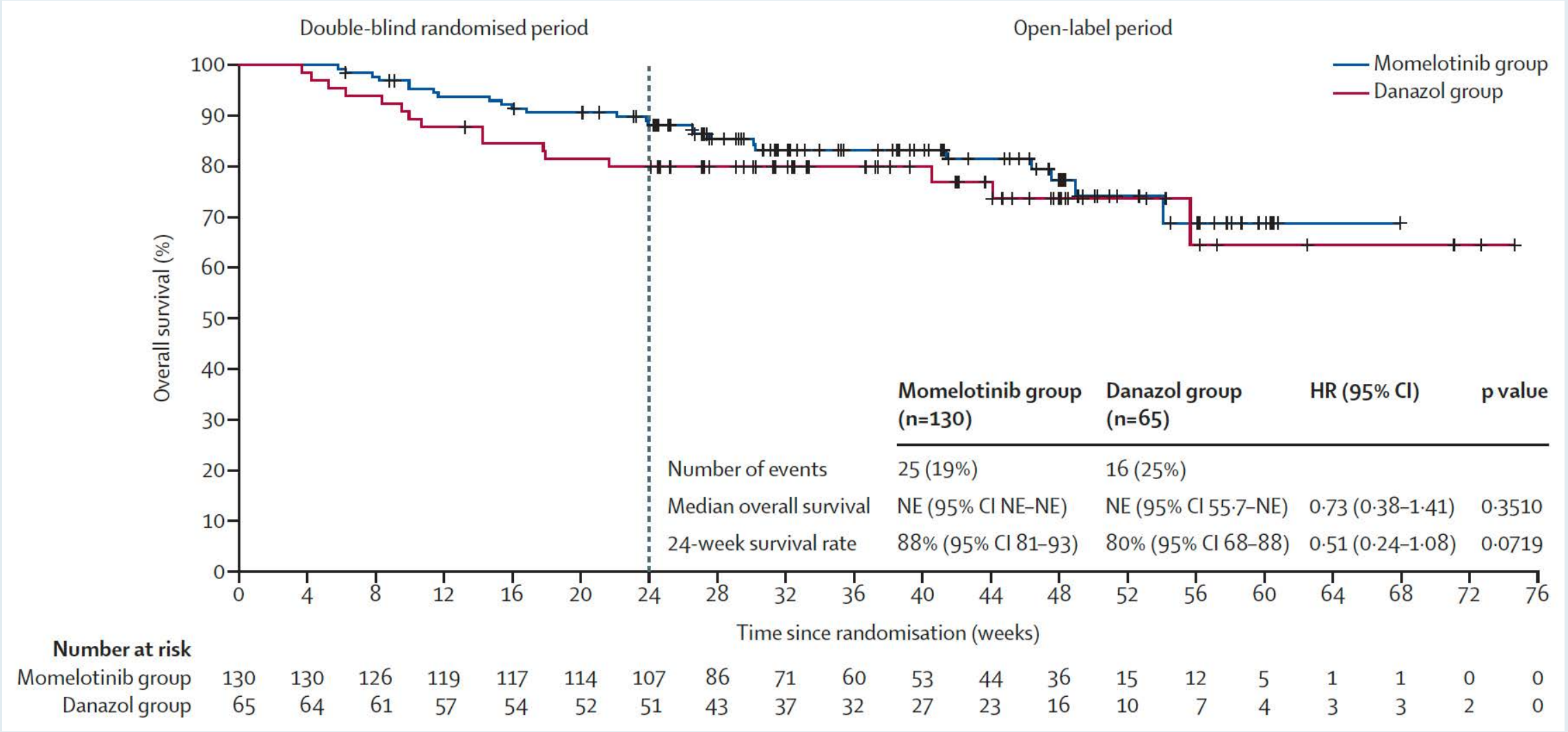
	Momelotinib group (n=130)		Danazol group (n=65)	
	Any grade	Grade ≥3	Any grade	Grade ≥3
Non-haematological abnormalities (preferred term)				
Diarrhoea	29 (22%)	0	6 (9%)	1 (2%)
Nausea	21 (16%)	3 (2%)	6 (9%)	2 (3%)
Asthenia	17 (13%)	1 (1%)	6 (9%)	1 (2%)
Pruritus	14 (11%)	2 (2%)	7 (11%)	0
Weight decreased	14 (11%)	0	4 (6%)	0
Blood creatinine increased	10 (8%)	1 (1%)	10 (15%)	2 (3%)
Dyspnoea	10 (8%)	3 (2%)	9 (14%)	1 (2%)
Peripheral oedema	10 (8%)	2 (2%)	9 (14%)	0
Fatigue	8 (6%)	1 (1%)	7 (11%)	2 (3%)
Acute kidney injury	6 (5%)	4 (3%)	8 (12%)	6 (9%)
Haematological abnormalities*				
Anaemia	129 (99%)	79 (61%)	65 (100%)	49 (75%)
Thrombocytopenia	99 (76%)	36 (28%)	40 (62%)	17 (26%)
Neutropenia	38 (29%)	16 (12%)	17 (26%)	6 (9%)

Data are n (%). *Haematological abnormalities are based on laboratory values. The data shown are for events of the worst grade during the 24-week randomised treatment phase of the study, regardless of whether this grade was a change from baseline.

Verstovsek S et al. *Lancet* 2023;401:269-80.

RTP RESEARCH TO PRACTICE

MOMENTUM: Overall Survival (ITT Population)



	Momelotinib group (n=130)	Danazol group (n=65)	HR (95% CI)	p value
Number of events	25 (19%)	16 (25%)		
Median overall survival	NE (95% CI NE–NE)	NE (95% CI 55·7–NE)	0·73 (0·38–1·41)	0·3510
24-week survival rate	88% (95% CI 81–93)	80% (95% CI 68–88)	0·51 (0·24–1·08)	0·0719

Number at risk

Momelotinib group	130	130	126	119	117	114	107	86	71	60	53	44	36	15	12	5	1	1	0	0
Danazol group	65	64	61	57	54	52	51	43	37	32	27	23	16	10	7	4	3	3	2	0

Verstovsek S et al. *Lancet* 2023;401:269-80.

MDS

N Engl J Med 2020;382:140-51.

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Luspatercept in Patients with Lower-Risk Myelodysplastic Syndromes

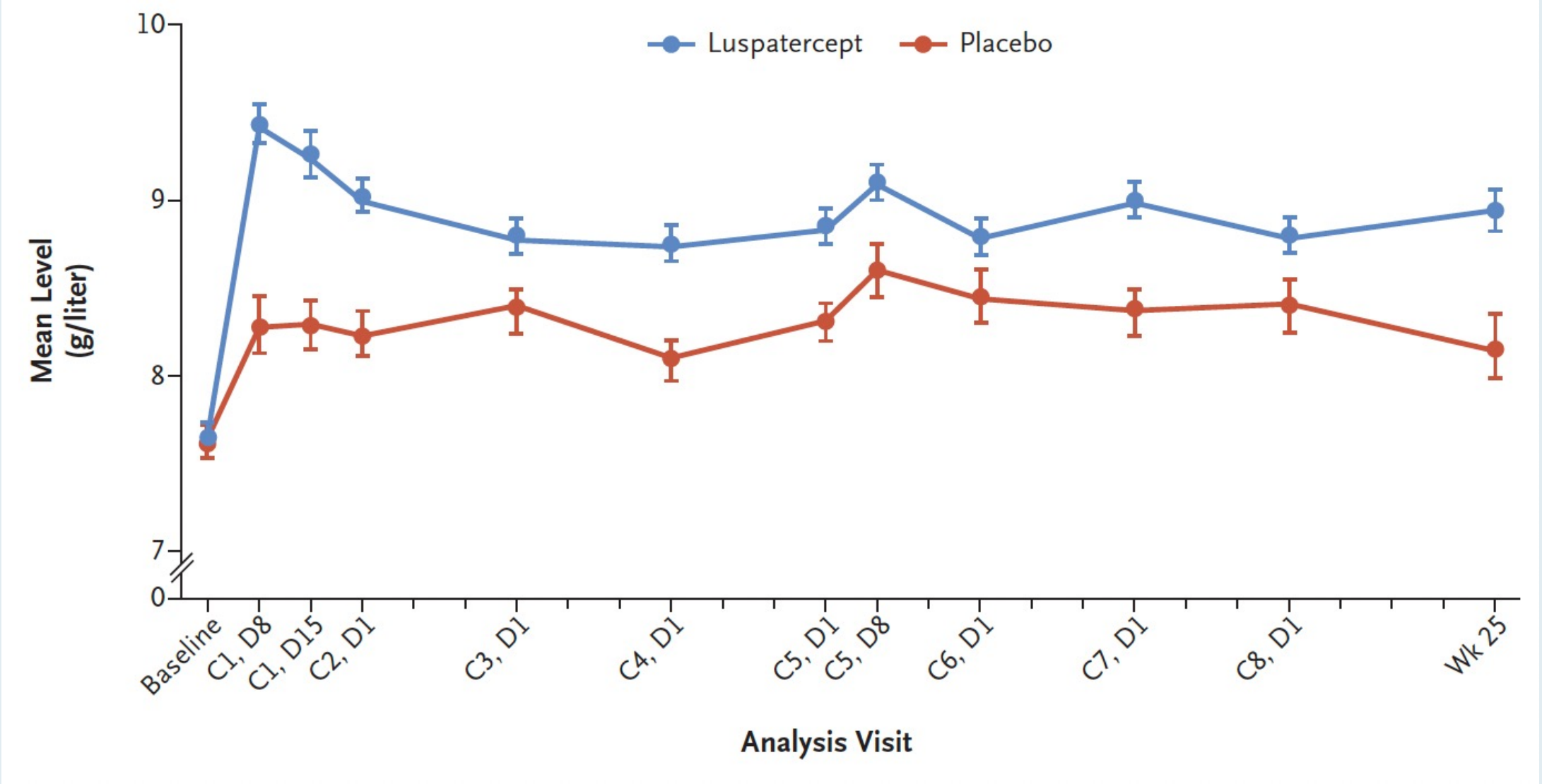
P. Fenaux, U. Platzbecker, G.J. Mufti, G. Garcia-Manero, R. Buckstein, V. Santini, M. Díez-Campelo, C. Finelli, M. Cazzola, O. Ilhan, M.A. Sekeres, J.F. Falantes, B. Arrizabalaga, F. Salvi, V. Giai, P. Vyas, D. Bowen, D. Selleslag, A.E. DeZern, J.G. Jurcic, U. Germing, K.S. Götze, B. Quesnel, O. Beyne-Rauzy, T. Cluzeau, M.-T. Voso, D. Mazure, E. Vellenga, P.L. Greenberg, E. Hellström-Lindberg, A.M. Zeidan, L. Adès, A. Verma, M.R. Savona, A. Laadem, A. Benzohra, J. Zhang, A. Rampersad, D.R. Dunshee, P.G. Linde, M.L. Sherman, R.S. Komrokji, and A.F. List

MEDALIST: Erythroid Response and Increase in Mean Hemoglobin Level

End Point	Luspatercept (N = 153)	Placebo (N = 76)
Erythroid response during wk 1–24*		
No. of patients (% [95% CI])	81 (53 [45–61])	9 (12 [6–21])
Reduction of ≥4 red-cell units/8 wk — no./total no. (%)†	52/107 (49)	8/56 (14)
Mean increase in hemoglobin level of ≥1.5 g/dl — no./total no. (%)‡	29/46 (63)	1/20 (5)
Erythroid response during wk 1–48*		
No. of patients (% [95% CI])	90 (59 [51–67])	13 (17 [9–27])
Reduction of ≥4 red-cell units/8 wk — no./total no. (%)†	58/107 (54)	12/56 (21)
Mean increase in hemoglobin level of ≥1.5 g/dl — no./total no. (%)‡	32/46 (70)	1/20 (5)
Mean increase in hemoglobin level of ≥1.0 g/dl — no. (% [95% CI])§		
During wk 1–24	54 (35 [28–43])	6 (8 [3–16])
During wk 1–48	63 (41 [33–49])	8 (11 [5–20])

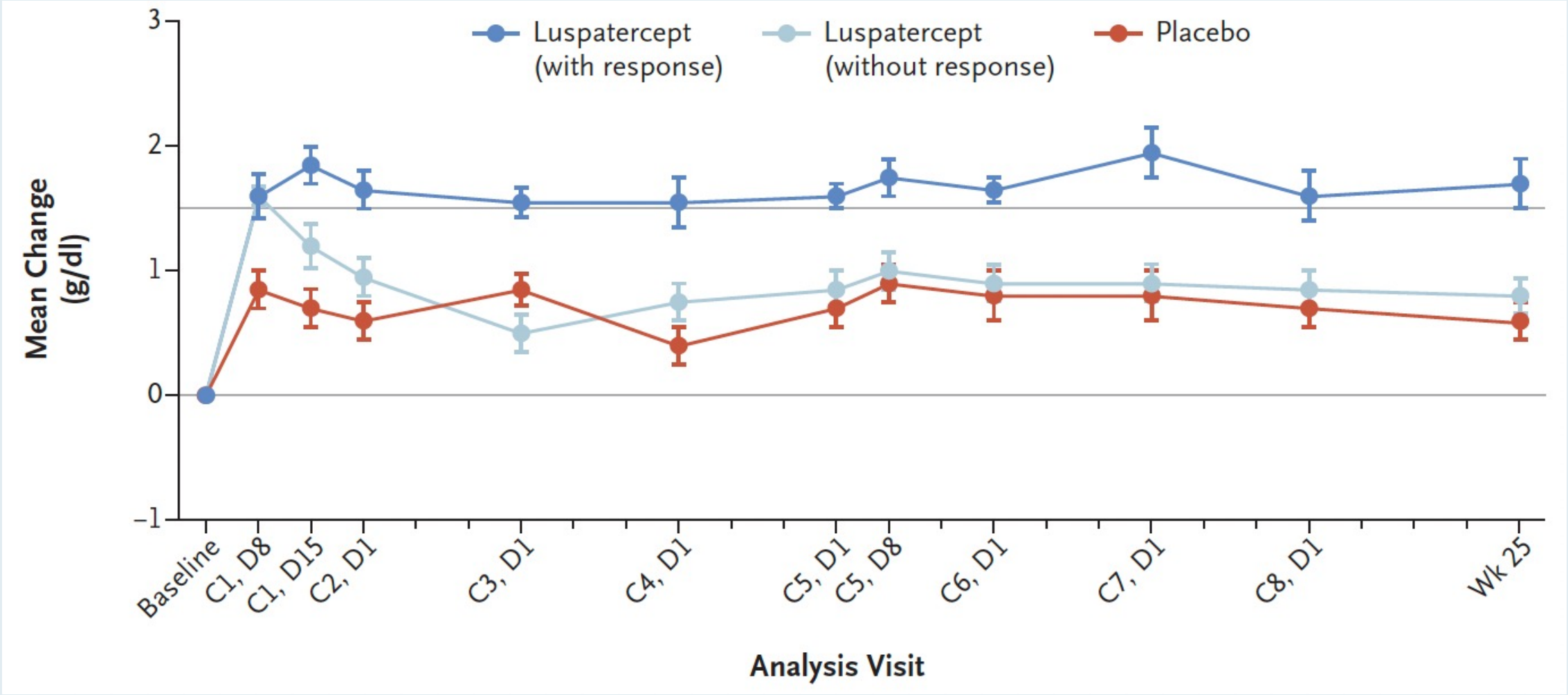
Fenaux P et al. *N Engl J Med* 2020;382:140-51.

MEDALIST: Change in Mean Observed Hemoglobin Level over Time



Fenaux P et al. *N Engl J Med* 2020;382:140-51.

MEDALIST: Change from Baseline in Hemoglobin Level



Fenaux P et al. *N Engl J Med* 2020;382:140-51.

MEDALIST: Adverse Events Occurring in at Least 10% of Patients

Event	Luspatercept (N=153)		Placebo (N=76)	
	Any Grade	Grade 3	Any Grade	Grade 3
	number of patients with event (percent)			
General disorder or administration-site condition				
Fatigue	41 (27)	7 (5)	10 (13)	2 (3)
Asthenia	31 (20)	4 (3)	9 (12)	0
Peripheral edema	25 (16)	0	13 (17)	1 (1)
Gastrointestinal disorder				
Diarrhea	34 (22)	0	7 (9)	0
Nausea†	31 (20)	1 (1)	6 (8)	0
Constipation	17 (11)	0	7 (9)	0
Nervous system disorder				
Dizziness	30 (20)	0	4 (5)	0
Headache	24 (16)	1 (1)	5 (7)	0
Musculoskeletal or connective-tissue disorder				
Back pain†	29 (19)	3 (2)	5 (7)	0
Arthralgia	8 (5)	1 (1)	9 (12)	2 (3)
Respiratory, thoracic, or mediastinal disorder				
Dyspnea†	23 (15)	1 (1)	5 (7)	0
Cough	27 (18)	0	10 (13)	0
Infection or infestation				
Bronchitis†	17 (11)	1 (1)	1 (1)	0
Urinary tract infection†	17 (11)	2 (1)	4 (5)	3 (4)

Fenaux P et al. *N Engl J Med* 2020;382:140-51.

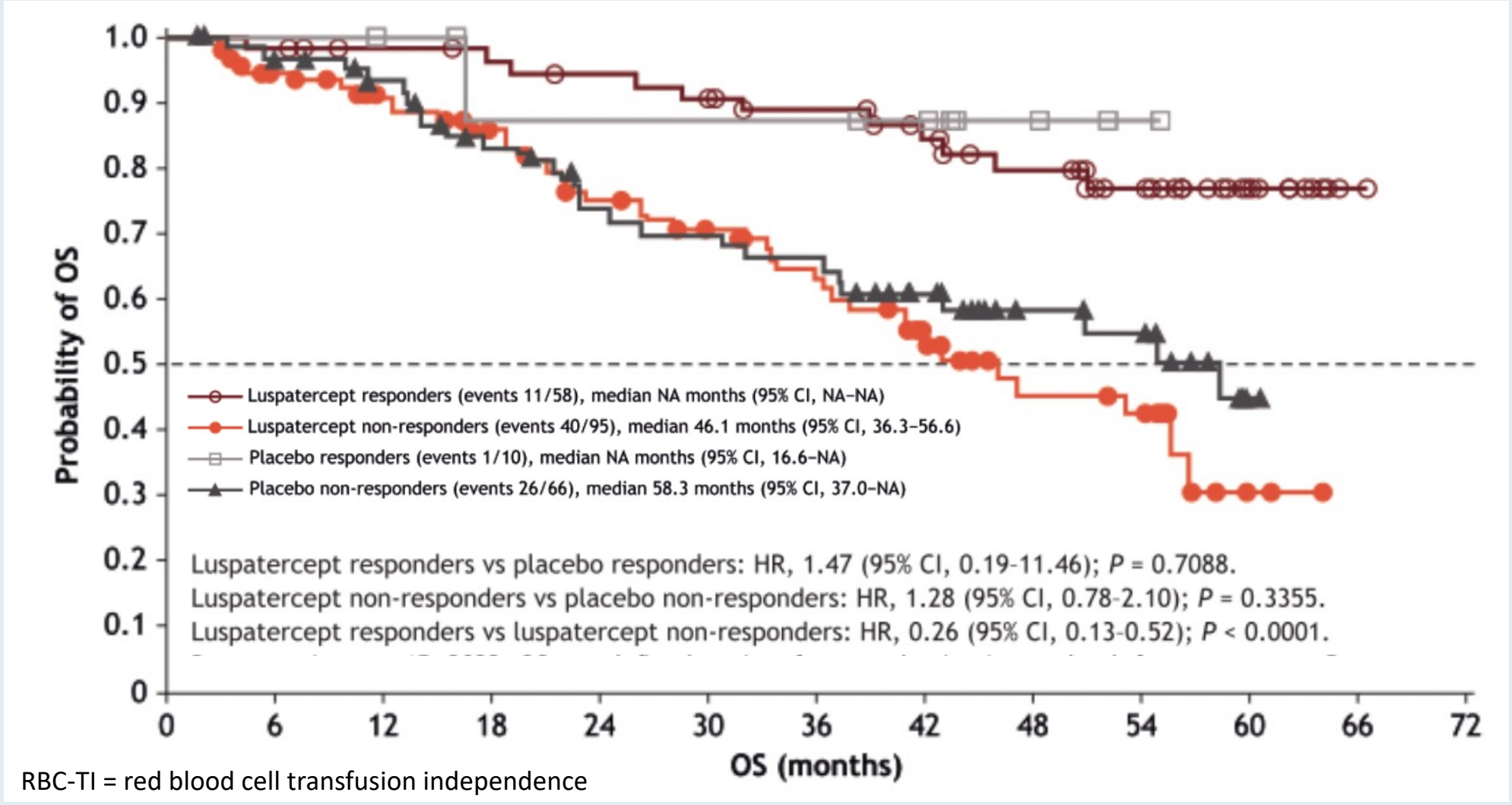
Overall survival and progression-free survival of patients following luspatercept treatment in the MEDALIST trial

Valeria Santini,[1] Pierre Fenaux,[2] Amer M. Zeidan,[3] Rami S. Komrokji,[4] Rena Buckstein,[5] Esther N. Oliva,[6] Xianwei Ha,[7] Dimana Miteva,[8] Aylin Yucel,[7] Jose Alberto Nadal,[8] Uwe Platzbecker[9]

ASH 2022;Abstract 1774

RTP RESEARCH TO PRACTICE

MEDALIST: Overall Survival by RBC-TI ≥8 Weeks During Weeks 1 to 24 Response and Treatment Arm (ITT Population)

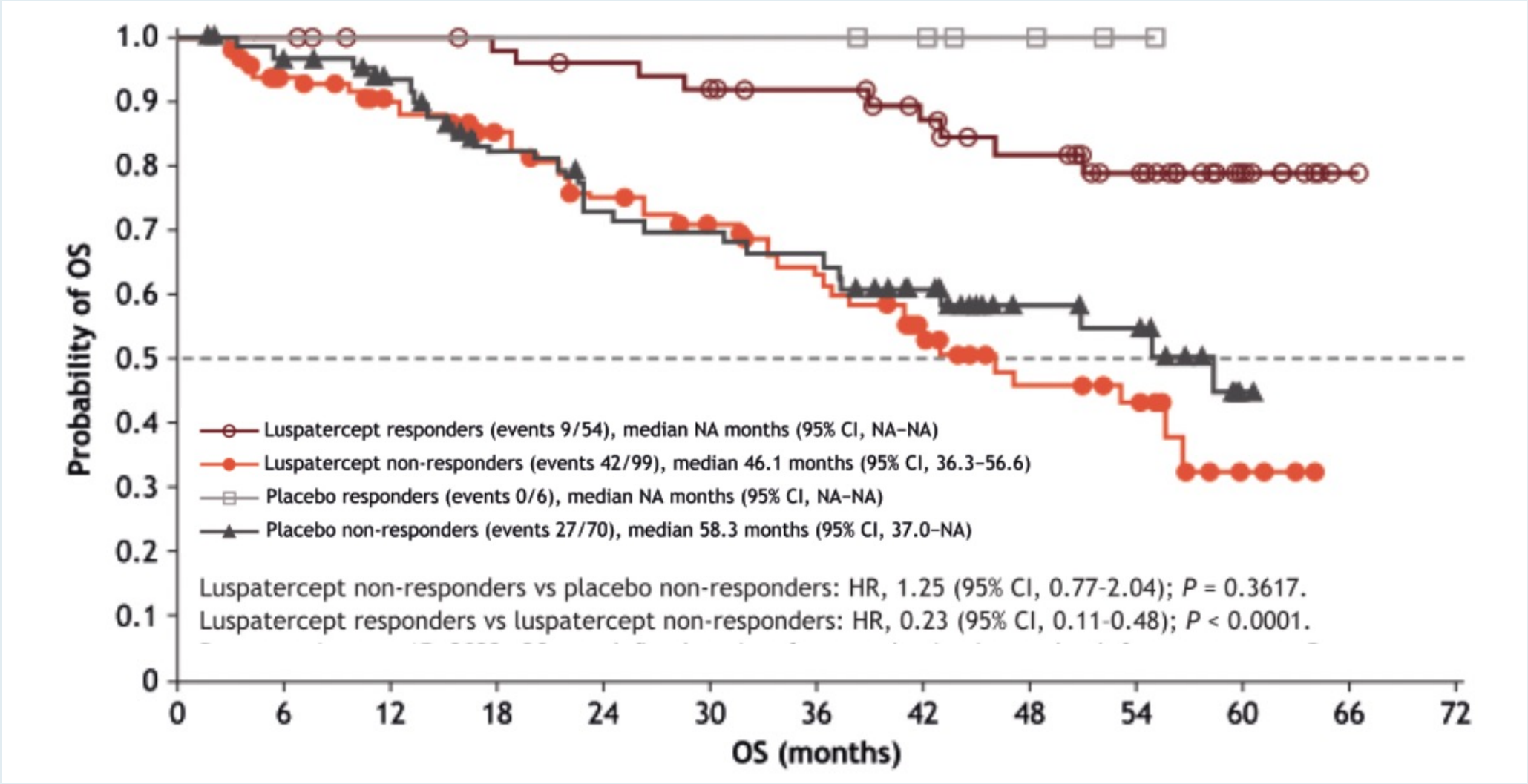


RBC-TI = red blood cell transfusion independence

Santini V et al. ASH 2022;Abstract 1774.

RTP RESEARCH TO PRACTICE

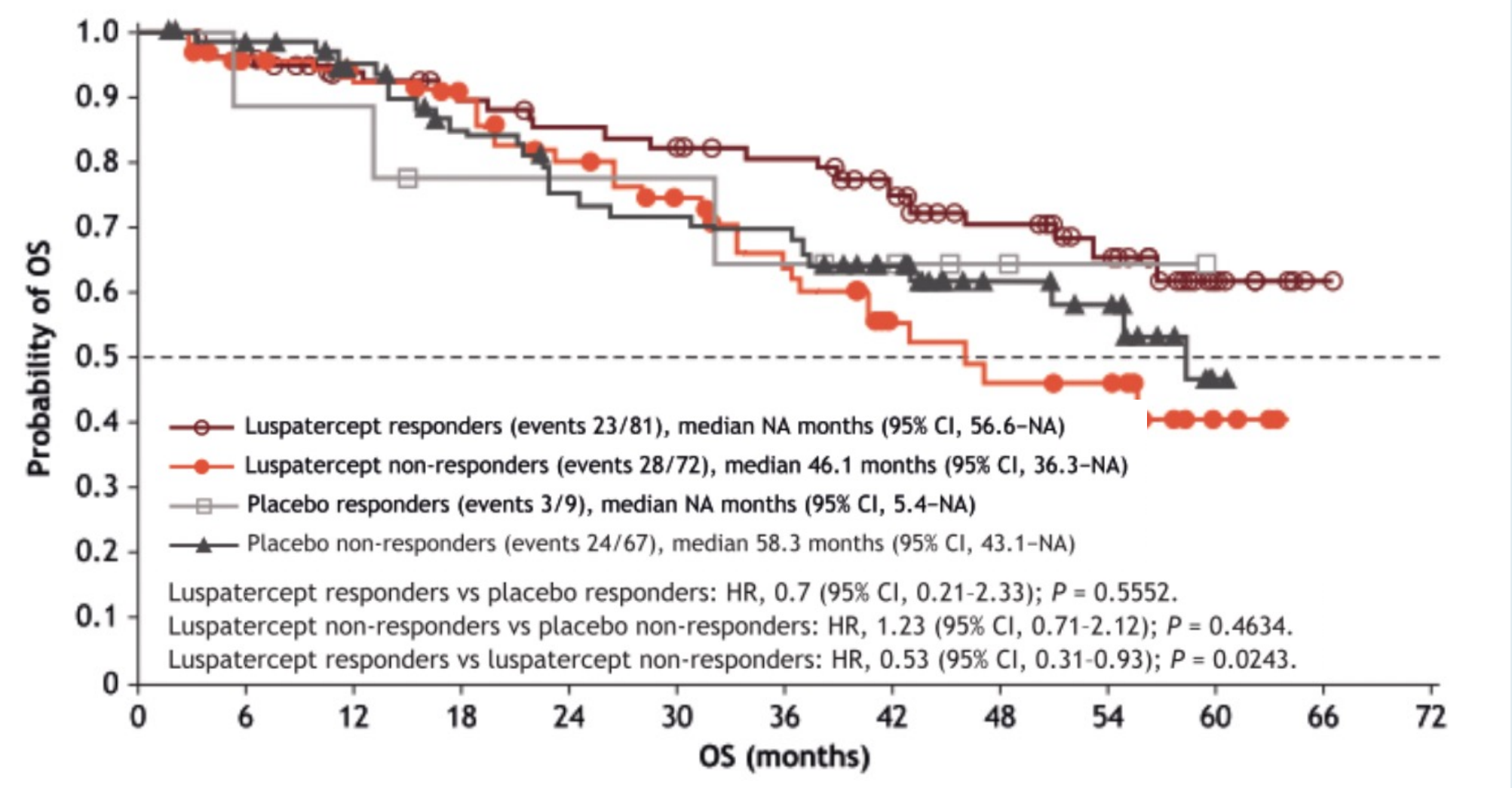
MEDALIST: Overall Survival by Mean Increase in Hb ≥1 g/dL Response During Weeks 1 to 24



Santini V et al. ASH 2022;Abstract 1774.

RTP RESEARCH TO PRACTICE

MEDALIST: Overall Survival by Modified Hematologic Improvement – Erythroid Response During Weeks 1 to 24



Santini V et al. ASH 2022;Abstract 1774.

RTP RESEARCH TO PRACTICE

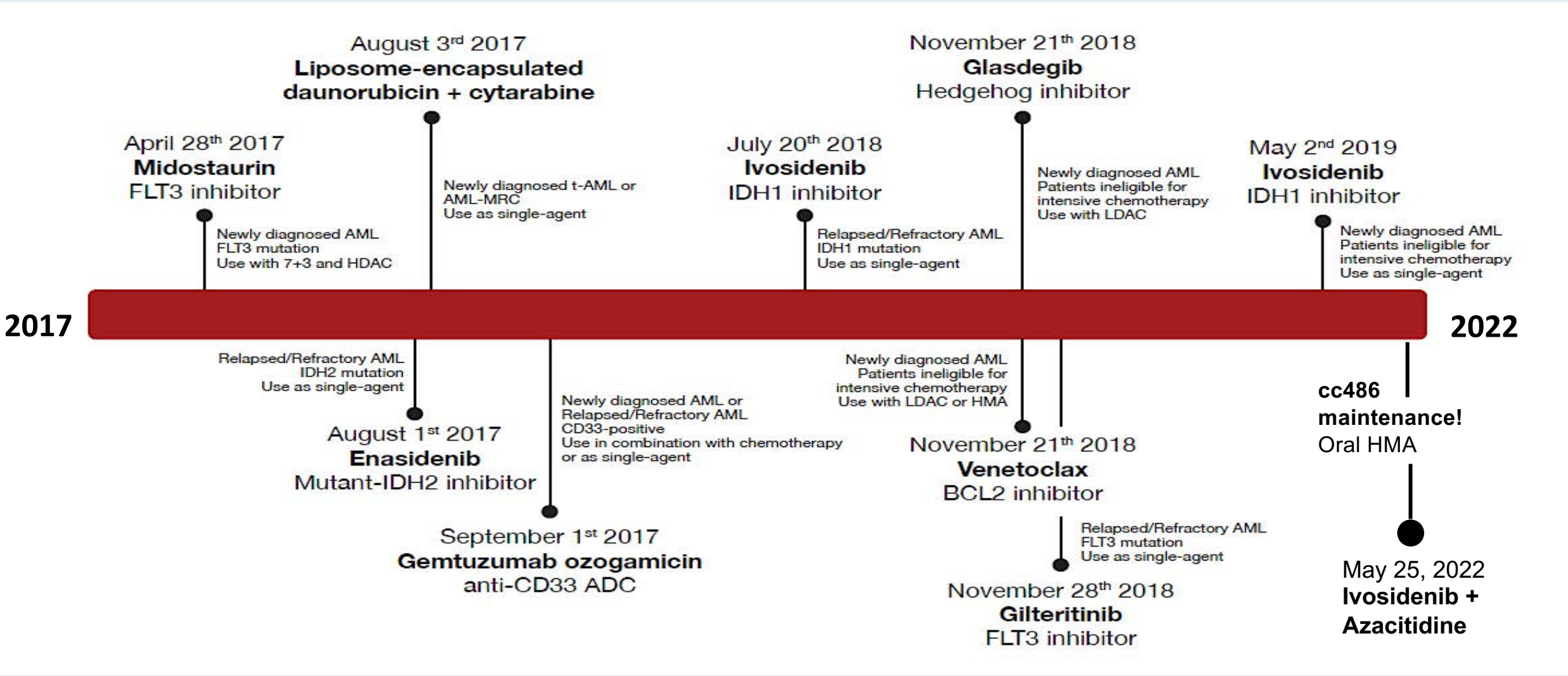
MEDALIST Conclusions

- Patients who achieved a response had an increased probability of extended OS
  - Patients treated with luspatercept had a 5 times greater probability of response, defined as RBC-TI ≥ 8 weeks during weeks 1-24, than patients treated with placebo
  - Patients treated with luspatercept who achieved an increase in Hb ≥ 1 g/dL in the first 24 weeks of treatment had extended OS
  - Similarly, achievement of RBC-TI ≥ 8 weeks or mHI-E in the first 24 weeks correlated with significant extension of OS
  - Responders (RBC-TI) in the placebo group were more likely to have a low baseline transfusion burden (< 4 units/8 weeks) than patients treated with luspatercept
- There were no significant differences in PFS between patients treated with luspatercept or placebo in the ITT population, measured as time from MDS diagnosis to AML progression
- Patients with LR-MDS with certain baseline characteristics associated with greater OS and PFS may derive greater survival benefit from luspatercept than placebo
  - Patients treated with luspatercept with IPSS-R Very low-risk MDS were more likely to achieve OS > 36 months
  - Patients treated with luspatercept with a baseline serum EPO level of 100 to < 200 U/L were more likely to achieve PFS > 36 months

AML

RTP
RESEARCH
TO PRACTICE

The Rapidly Evolving Treatment Landscape of AML: FDA Approvals



Richard-Carpentier G, DiNardo CD. *Hematology Am Soc Educ Program* 2019(1):548-56.

Content Courtesy of Courtney D DiNardo, MD, MSCE

RTP RESEARCH TO PRACTICE

Long-term overall survival with oral azacitidine in patients with acute myeloid leukemia in first remission after intensive chemotherapy: updated results from the phase 3 QUAZAR AML-001 trial

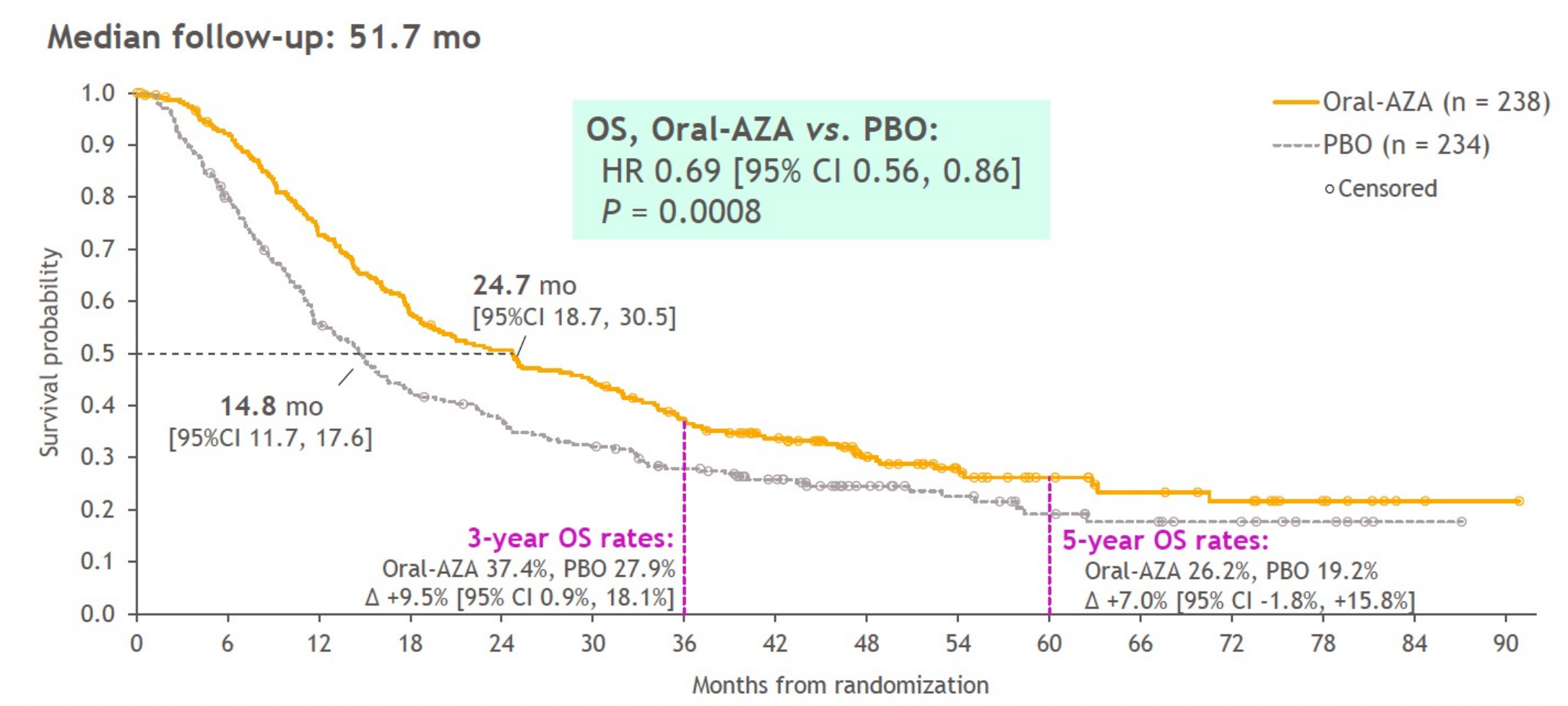
Andrew H Wei,[1,2] Hartmut Döhner,[3] Hamid Sayar,[4] Farhad Ravandi,[5] Pau Montesinos,[6] Hervé Dombret,[7,8] Dominik Selleslag,[9] Kimmo Porkka,[10,11] Jun-Ho Jang,[12] Barry Skikne,[13,14] CL Beach,[14] Olivia Yu Tian,[14] and Gail J Roboz[15,16]

[1]The Alfred Hospital, Melbourne, Australia; [2]Australian Centre for Blood Diseases, Monash University, Melbourne, Australia; [3]Ulm University Hospital, Ulm, Germany; [4]Indiana University Cancer Center, Indianapolis, IN; [5]The University of Texas MD Anderson Cancer Center, Houston, TX; [6]Hospital Universitario La Fe de Valencia, Valencia, Spain; [7]Institut de Recherche Saint Louis, Université de Paris, Paris, France; [8]Hôpital Saint-Louis, Assistance Publique - Hôpitaux de Paris (AP-HP), Paris, France; [9]AZ Sint-Jan Brugge-Oostende AV, Brugge, Belgium; [10]iCAN Digital Precision Cancer Medicine Flagship, University of Helsinki, Helsinki, Finland; [11]Helsinki University Hospital Comprehensive Cancer Center, Helsinki, Finland; [12]Samsung Medical Center, Seoul, Republic of Korea; [13]University of Kansas Medical Center, Kansas City, KS; [14]Bristol Myers Squibb, Princeton, NJ; [15]Weil Cornell Medical College, New York, NY; [16]New York Presbyterian Hospital, New York, NY

Presentation 871

ASH 2021

QUAZAR AML-001: Long-Term Overall Survival with Oral Azacitidine in AML in First Remission After Intensive Chemotherapy



Wei AH et al. ASH 2021;Abstract 871.

RTP RESEARCH TO PRACTICE

QUAZAR AML-001: Safety Summary and Select Adverse Events (AEs)

	CC-486 (N = 236)		Placebo (N = 233)	
	Any grade	Grade 3 or 4	Any grade	Grade 3 or 4
AEs leading to dose interruptions	43%	—	17%	—
AEs leading to dose reductions	16%	—	3%	—
AEs leading to discontinuation	13%	—	4%	—
Nausea	65%	3%	24%	<1%
Vomiting	60%	3%	10%	0
Diarrhea	50%	5%	21%	1%
Neutropenia	44%	41%	26%	24%
Thrombocytopenia	33%	22%	27%	21%
Anemia	20%	14%	18%	13%

Wei AH et al. *N Engl J Med* 2020;383:2526-37.

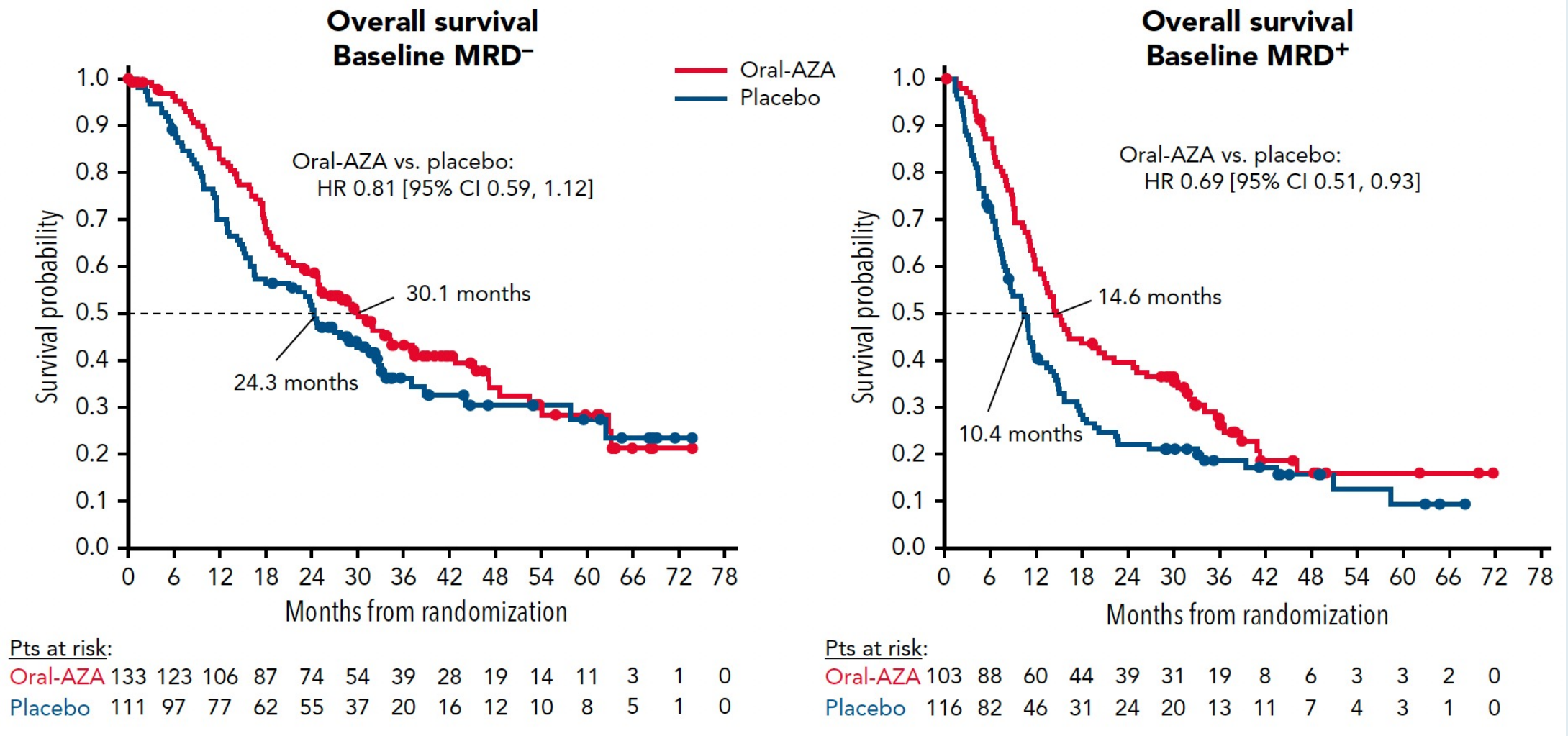
blood
Regular Article

CLINICAL TRIALS AND OBSERVATIONS

Oral azacitidine prolongs survival of patients with AML in remission independently of measurable residual disease status

Gail J. Roboz,[1,2] Farhad Ravandi,[3] Andrew H. Wei,[4,5] Hervé Dombret,[6,7] Felicitas Thol,[8] Maria Teresa Voso,[9] Andre C. Schuh,[10] Kimmo Porkka,[11] Ignazia La Torre,[12] Barry Skikne,[13,14] Jianhua Zhong,[13] C. L. Beach,[13] Alberto Risueño,[15] Daniel L. Menezes,[13] Gert Ossenkoppele,[16] and Hartmut Döhner[17]

QUAZAR AML-001: Overall Survival by Baseline MRD Status



MRD = minimal residual disease

Roboz GJ et al. *Blood* 2022 April;139(14):2145-55.

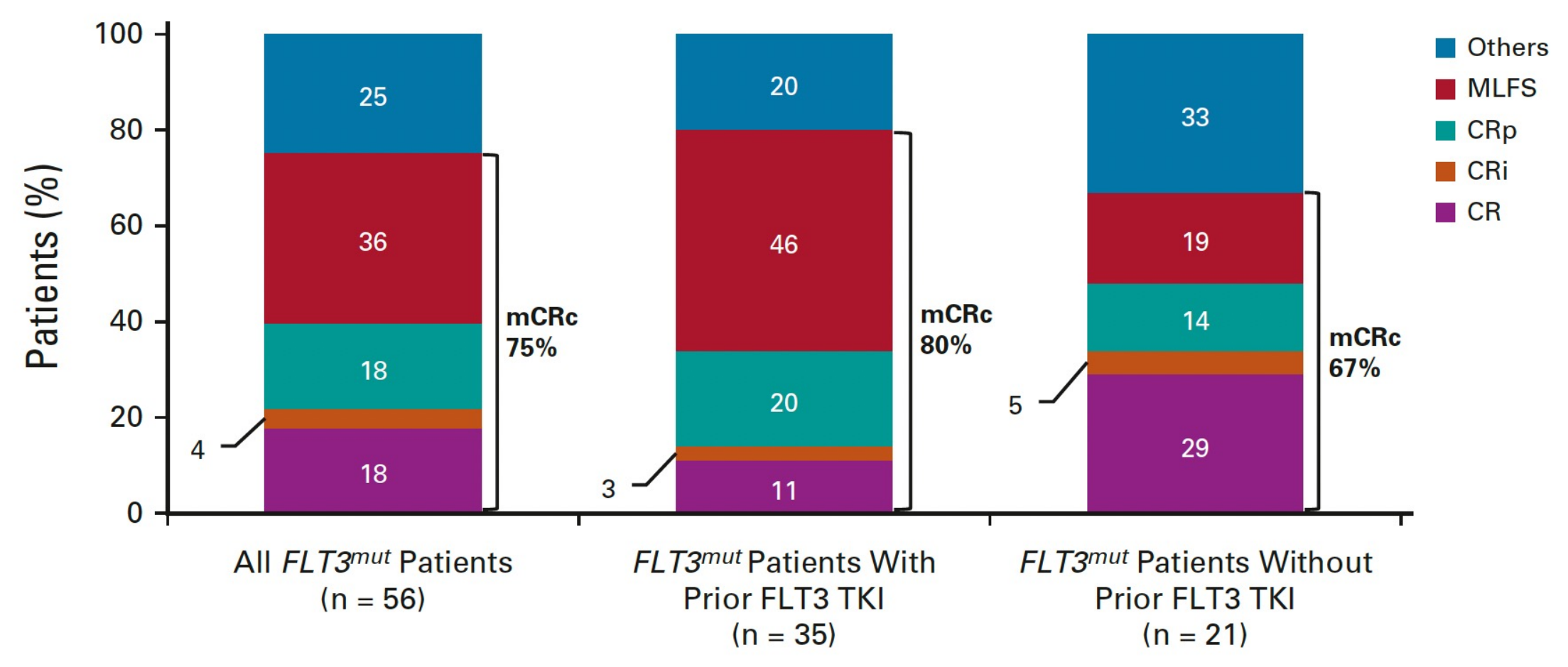
original reports

Venetoclax Plus Gilteritinib for *FLT3*-Mutated Relapsed/Refractory Acute Myeloid Leukemia

Naval Daver, MD[1]; Alexander E. Perl, MD[2]; Joseph Maly, MD[3]; Mark Levis, MD, PhD[4]; Ellen Ritchie, MD[5]; Mark Litzow, MD[6]; James McCloskey, MD[7]; Catherine C. Smith, MD[8]; Gary Schiller, MD[9]; Terrence Bradley, MD[10,11]; Ramon V. Tiu, MD[12]; Kiran Naqvi, MD[13]; Monique Dail, PhD[13]; Deanna Brackman, PhD[14]; Satya Siddani, PhD[14]; Jing Wang, PhD[14]; Brenda Chyla, PhD[14]; Paul Lee, MD, PhD[14]; and Jessica K. Altman, MD[15]

J Clin Oncol 2022 December;40:4048-59

Modified Complete Reponse (mCRc) Rate to Venetoclax and Gilteritinib



Daver N et al. *J Clin Oncol* 2022 December;40:4048-59.

blood

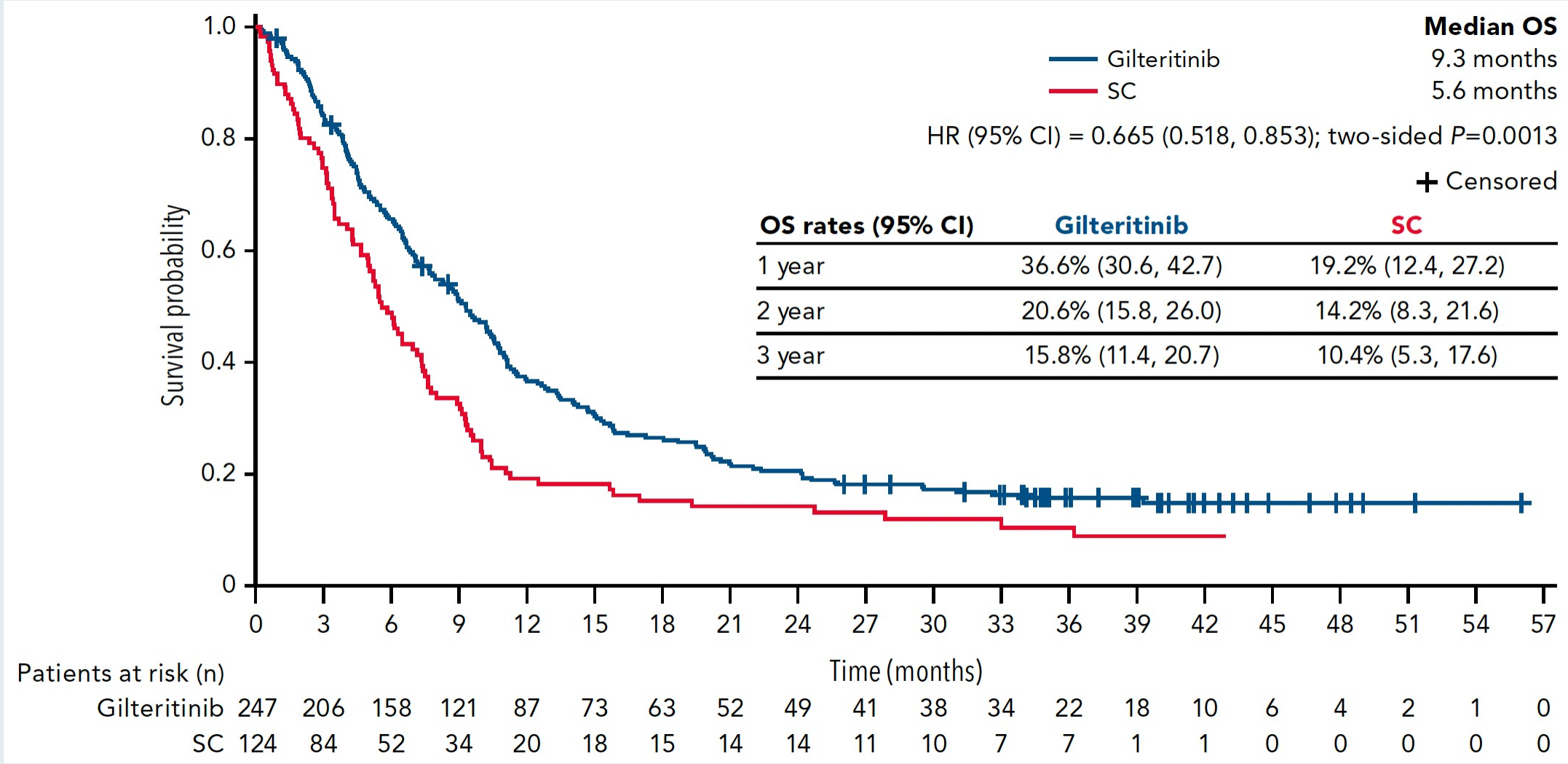
Regular Article

CLINICAL TRIALS AND OBSERVATIONS

Follow-up of patients with R/R *FLT3*-mutation–positive AML treated with gilteritinib in the phase 3 ADMIRAL trial

Alexander E. Perl,[1] Richard A. Larson,[2] Nikolai A. Podoltsev,[3] Stephen Strickland,[4] Eunice S. Wang,[5] Ehab Atallah,[6] Gary J. Schiller,[7] Giovanni Martinelli,[8] Andreas Neubauer,[9] Jorge Sierra,[10] Pau Montesinos,[11] Christian Récher,[12] Sung-Soo Yoon,[13] Naoko Hosono,[14] Masahiro Onozawa,[15] Shigeru Chiba,[16] Hee-Je Kim,[17] Nahla Hasabou,[18] Qiaoyang Lu,[18] Ramon Tiu,[18] and Mark J. Levis[19]

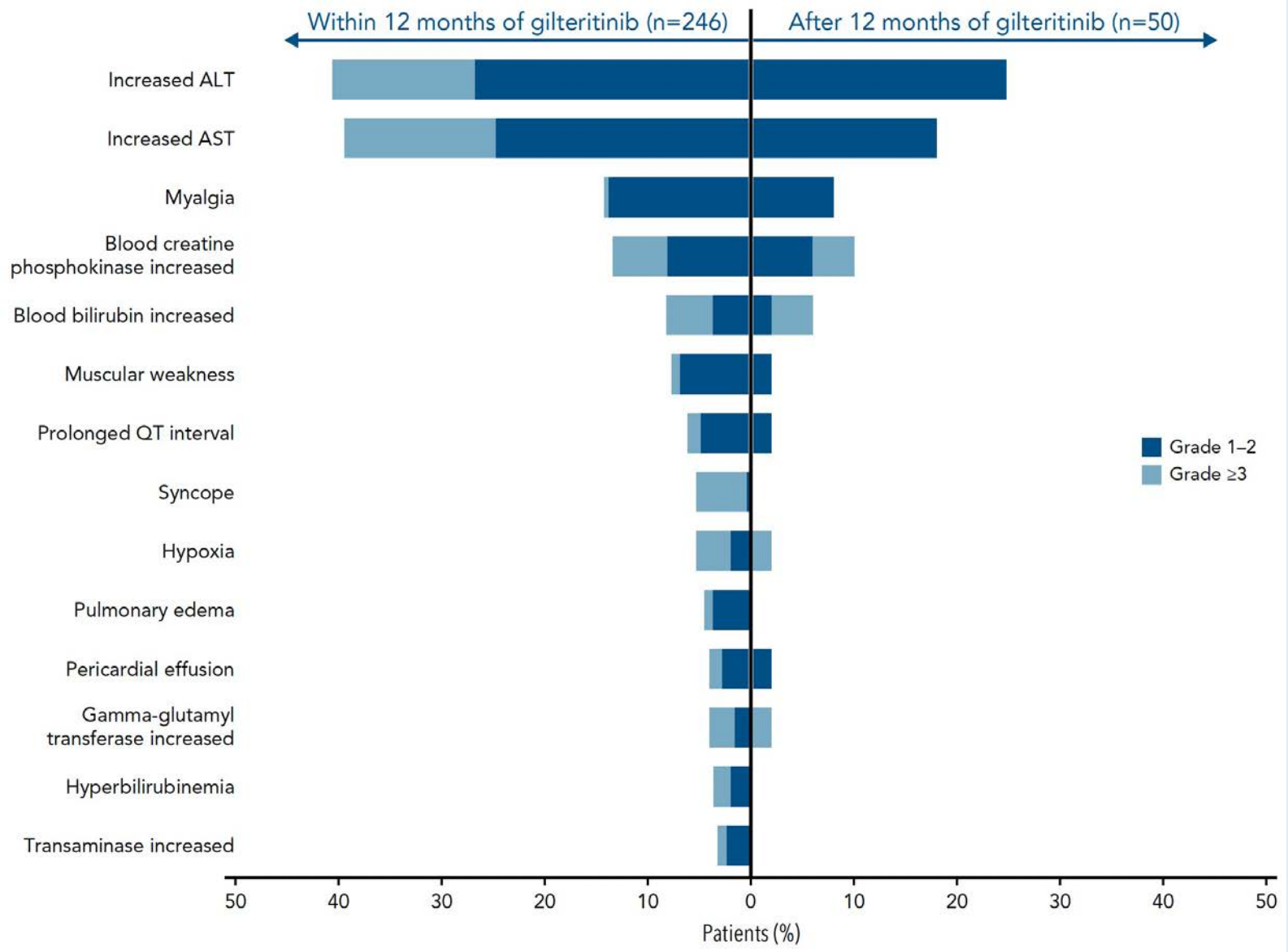
ADMIRAL: Overall Survival (ITT Population)



Perl AE et al. *Blood* 2022 June;139(23):3366-75

RTP RESEARCH TO PRACTICE

ADMIRAL: Adverse Events of Interest



Perl AE et al. *Blood* 2022 June;139(23):3366-75

RTP RESEARCH TO PRACTICE

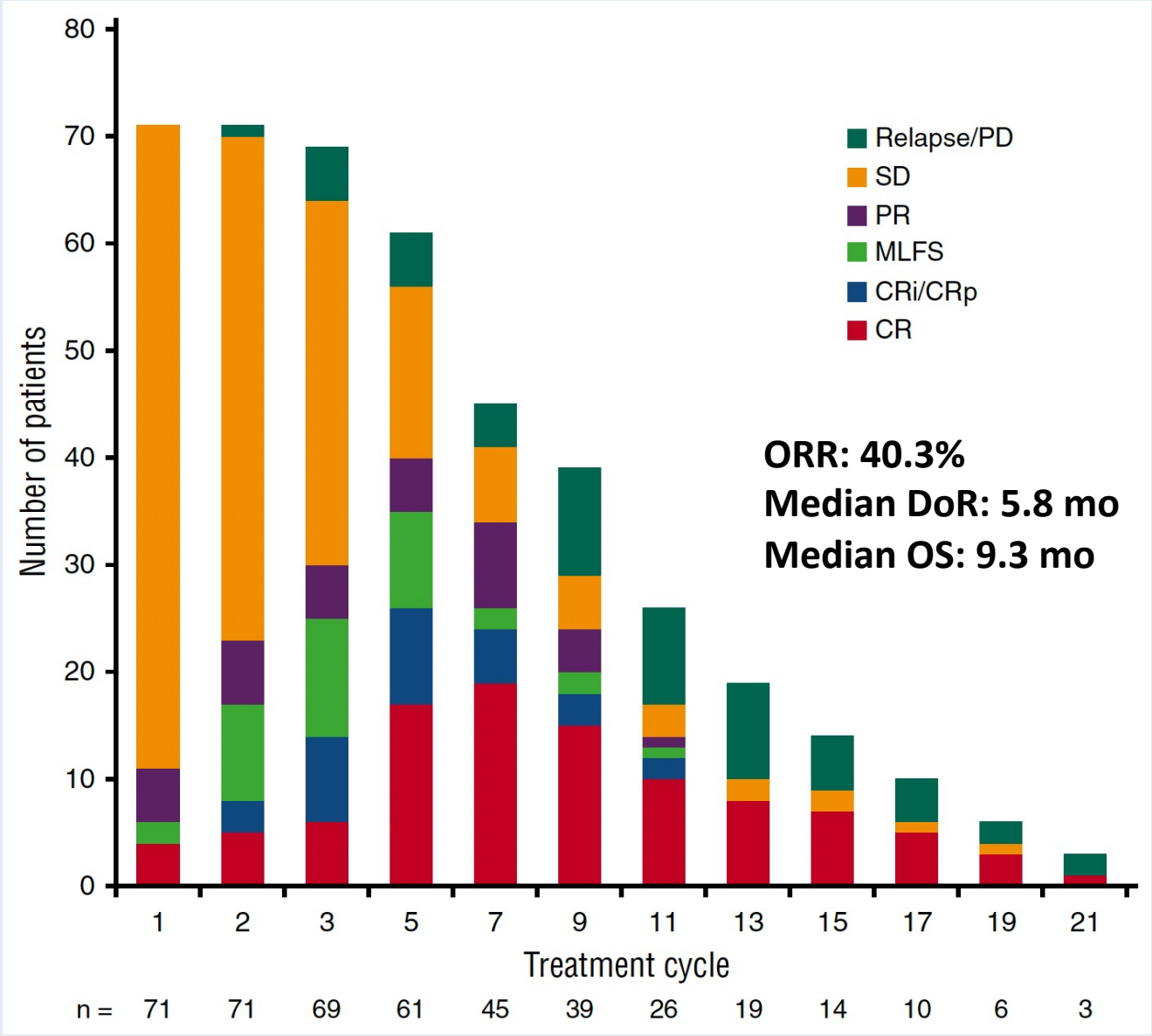
CLINICAL TRIALS AND OBSERVATIONS

Blood 2017;130(6):722-31

Enasidenib in mutant *IDH2* relapsed or refractory acute myeloid leukemia

Eytan M. Stein,[1,2,*] Courtney D. DiNardo,[3,*] Daniel A. Pollyea,[4] Amir T. Fathi,[5,6] Gail J. Roboz,[2,7] Jessica K. Altman,[8] Richard M. Stone,[9] Daniel J. DeAngelo,[9] Ross L. Levine,[1] Ian W. Flinn,[10] Hagop M. Kantarjian,[3] Robert Collins,[11] Manish R. Patel,[12] Arthur E. Frankel,[11] Anthony Stein,[13] Mikkael A. Sekeres,[14] Ronan T. Swords,[15] Bruno C. Medeiros,[16] Christophe Willekens,[17,18] Paresh Vyas,[19,20] Alessandra Tosolini,[21] Qiang Xu,[21] Robert D. Knight,[21] Katharine E. Yen,[22] Sam Agresta,[22] Stephane de Botton,[17,18,†] and Martin S. Tallman[1,2,†]

Evolution of Response with Enasidenib in R/R AML with IDH2 Mutation



Stein EM et al. *Blood* 2017;130(6):722-31.

Grade 3/4 Treatment-Related Treatment-Emergent AEs with Enasidenib

	Enasidenib 100 mg per day (n = 153)	
TEAE	**No.**	**%**
Hyperbilirubinemia*	13	8
IDH differentiation syndrome†	11	7
Anemia	10	7
Thrombocytopenia‡	8	5
Tumor lysis syndrome	5	3
Decreased appetite	3	2
Leukocytosis	2	1
Fatigue	2	1
Nausea	2	1
Lipase increased	2	1

Stein EM et al. *Blood* 2017;130(6):722-31.

N Engl J Med 2018;378(25):2386-98

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Durable Remissions with Ivosidenib in *IDH1*-Mutated Relapsed or Refractory AML

C.D. DiNardo, E.M. Stein, S. de Botton, G.J. Roboz, J.K. Altman, A.S. Mims, R. Swords, R.H. Collins, G.N. Mannis, D.A. Pollyea, W. Donnellan, A.T. Fathi, A. Pigneux, H.P. Erba, G.T. Prince, A.S. Stein, G.L. Uy, J.M. Foran, E. Traer, R.K. Stuart, M.L. Arellano, J.L. Slack, M.A. Sekeres, C. Willekens, S. Choe, H. Wang, V. Zhang, K.E. Yen, S.M. Kapsalis, H. Yang, D. Dai, B. Fan, M. Goldwasser, H. Liu, S. Agresta, B. Wu, E.C. Attar, M.S. Tallman, R.M. Stone, and H.M. Kantarjian

Response to Ivosidenib in R/R AML with IDH1 Mutation

Clinical endpoint	Primary efficacy population (N = 125)
Complete remission (CR)	21.6%
CR + CR with partial hematologic recovery	30.4%
Overall response rate	41.6%
Median DoR	8.2 months
Attained transfusion independence	35%

DiNardo CD et al. *N Engl J Med* 2018;378(25):2386-98.

Grade ≥3 Treatment-Related Adverse Events

Event	Relapsed or Refractory AML and Starting Dose of Ivosidenib of 500 mg Daily (N=179)	Overall Population (N=258)
	no. of patients (%)	
≥1 Treatment-related adverse event of grade 3 or higher	37 (20.7)	66 (25.6)
Prolongation of the QT interval on ECG	14 (7.8)	18 (7.0)
IDH differentiation syndrome†	7 (3.9)	12 (4.7)
Anemia	4 (2.2)	6 (2.3)
Thrombocytopenia	3 (1.7)	5 (1.9)
Leukocytosis	3 (1.7)	3 (1.2)
Febrile neutropenia	1 (0.6)	3 (1.2)
Diarrhea	1 (0.6)	3 (1.2)
Platelet count decreased	3 (1.7)	3 (1.2)
Hypoxia	2 (1.1)	3 (1.2)

DiNardo CD et al. *N Engl J Med* 2018;378(25):2386-98.

Blood 2020;135(7):463-71

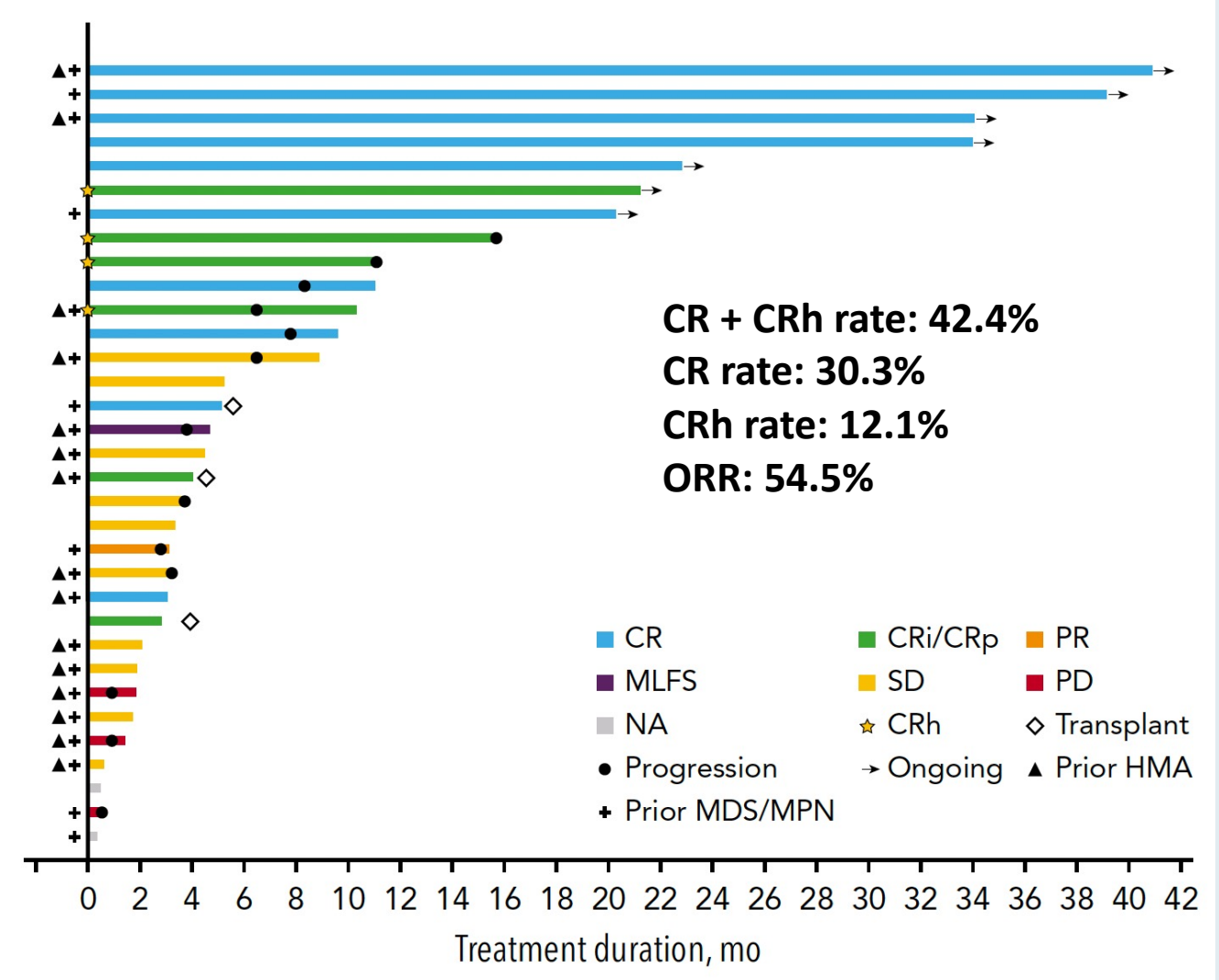
Plenary Paper

MYELOID NEOPLASIA

Ivosidenib induces deep durable remissions in patients with newly diagnosed *IDH1*-mutant acute myeloid leukemia

Gail J. Roboz,[1,*] Courtney D. DiNardo,[2,*] Eytan M. Stein,[3] Stéphane de Botton,[4] Alice S. Mims,[5] Gabrielle T. Prince,[6] Jessica K. Altman,[7] Martha L. Arellano,[8] Will Donnellan,[9] Harry P. Erba,[10] Gabriel N. Mannis,[11] Daniel A. Pollyea,[12] Anthony S. Stein,[13] Geoffrey L. Uy,[14] Justin M. Watts,[15] Amir T. Fathi,[16] Hagop M. Kantarjian,[2] Martin S. Tallman,[3] Sung Choe,[17] David Dai,[17] Bin Fan,[17] Hongfang Wang,[17] Vickie Zhang,[17] Katharine E. Yen,[17] Stephanie M. Kapsalis,[17] Denice Hickman,[17] Hua Liu,[17] Samuel V. Agresta,[17] Bin Wu,[17] Eyal C. Attar,[17] and Richard M. Stone[18]

Response and Duration of Response to Ivosidenib



Roboz GJ et al. *Blood* 2020;135(7):463-71.

Treatment-Emergent Adverse Events with Ivosidenib

Patients with AE, n (%)	Ivosidenib 500 mg, N = 34	
	Any grade	Grade ≥3
Any AE	34 (100)	27 (79)
Diarrhea	18 (53)	2 (6)
Fatigue	16 (47)	4 (12)
Nausea	13 (38)	2 (6)
Decreased appetite	12 (35)	1 (3)
Thrombocytopenia	9 (26)	5 (15)
Anemia	9 (26)	4 (12)
Leukocytosis	9 (26)	1 (3)
Peripheral edema	9 (26)	0

Patients with AE, n (%)	Ivosidenib 500 mg, N = 34	
	Any grade	Grade ≥3
Dyspnea	8 (24)	1 (3)
Dizziness	8 (24)	0
Hypomagnesemia	8 (24)	0
Abdominal pain	7 (21)	1 (3)
Arthralgia	7 (21)	1 (3)
Constipation	7 (21)	1 (3)
Epistaxis	7 (21)	0
Hypokalemia	7 (21)	1 (3)
Insomnia	7 (21)	0

Roboz GJ et al. *Blood* 2020;135(7):463-71.

What I Tell My Patients:
Faculty Physicians and Nurses Discuss Patient Education About New Treatments and Clinical Trials

Fifteenth Annual RTP Symposium Series Held During the Annual ONS Congress

Acute Myeloid Leukemia, Myelodysplastic Syndromes and Myelofibrosis

Saturday, April 29, 2023
6:00 AM – 7:30 AM

Faculty

Ilene Galinsky, NP
Ruben A Mesa, MD (Virtual)
Daniel A Pollyea, MD, MS
Richard M Stone, MD
Sara M Tinsley-Vance, PhD, APRN, AOCN

Moderator

Neil Love, MD

RTP RESEARCH TO PRACTICE

What I Tell My Patients:
Faculty Physicians and Nurses Discuss Patient Education About New Treatments and Clinical Trials

Fifteenth Annual RTP Symposium Series Held During the Annual ONS Congress

Prostate Cancer

Saturday, April 29, 2023
12:15 PM – 1:45 PM

Faculty

Neeraj Agarwal, MD, FASCO
Kathy D Burns, RN, MSN, AGACNP-BC, OCN
Susan K Roethke, MSN, CRNP, AOCNP, ANP-BC
Sandy Srinivas, MD

Moderator

Neil Love, MD

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Thank you for joining us! Please take a moment to complete the survey currently up on Zoom. Your feedback is very important to us. The survey will remain open up to 5 minutes after the meeting ends.

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Virtual attendees: The NCPD credit link is posted in the chat room.

NCPD/ONCC credit information will be emailed to each participant within 1 to 2 business days.

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