

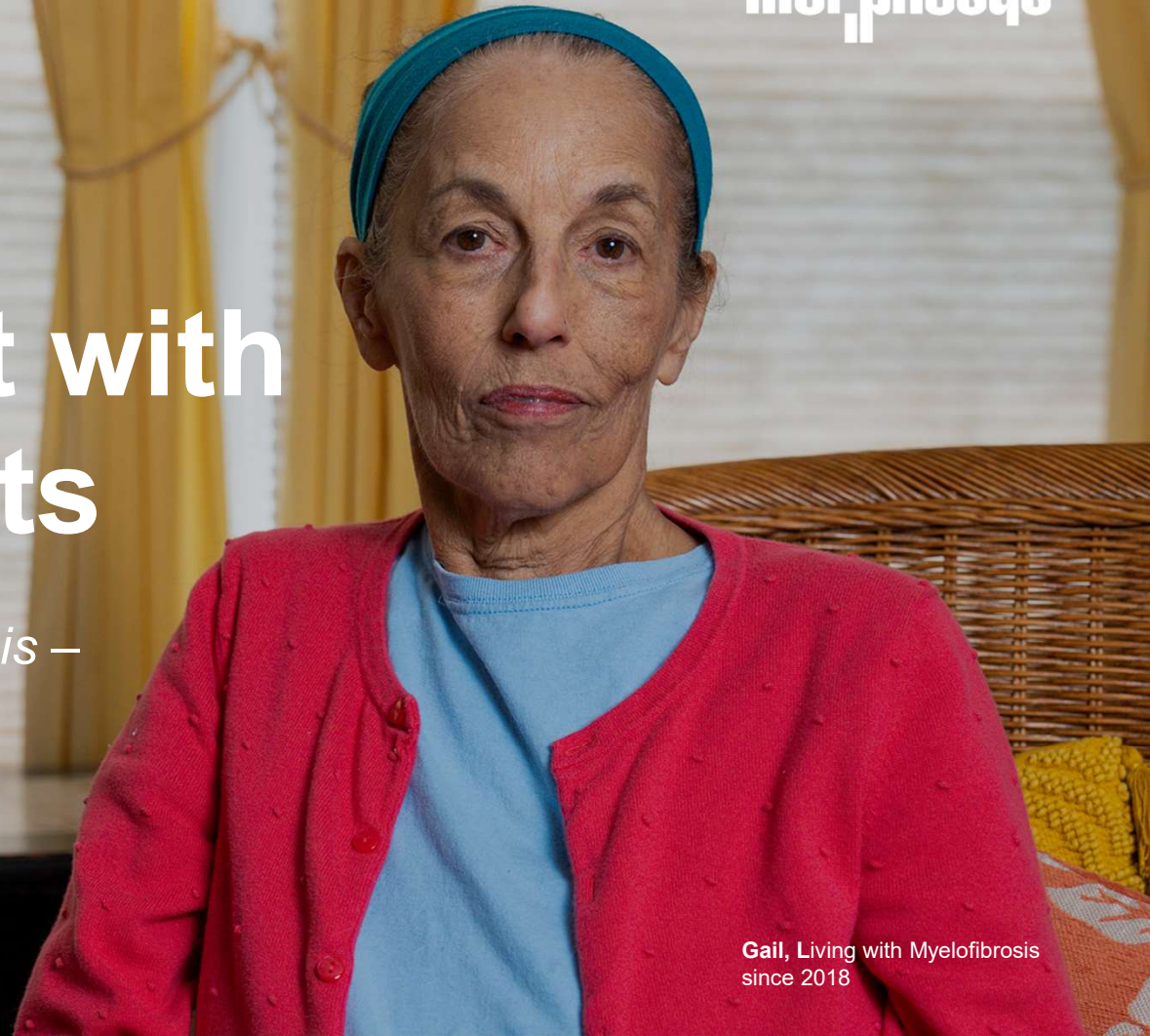


# ASH 2023 Investor Event with Medical Experts

*Pelabresib in First-Line Myelofibrosis –  
Phase 3 MANIFEST-2 Results*

December 11, 2023

**Gail**, Living with Myelofibrosis  
since 2018



## Forward-Looking Statements

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This communication contains certain forward-looking statements concerning the MorphoSys group of companies, including the expectations regarding Monjuvi's ability to treat patients with relapsed or refractory diffuse large B-cell lymphoma ("DLBCL"), the further clinical development of tafasitamab, including ongoing confirmatory trials, additional interactions with regulatory authorities and expectations regarding future regulatory filings and possible additional approvals for tafasitamab as well as the commercial performance of Monjuvi. The words "anticipate", "believe", "estimate", "expect", "intend", "may", "plan", "predict", "project", "would", "could", "potential", "possible", "hope" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. The forward-looking statements contained herein represent the judgment of MorphoSys as of the date of this release and involve known and unknown risks and uncertainties, which might cause the actual results, financial condition and liquidity, performance or achievements of MorphoSys, or industry results, to be materially different from any historic or future results, financial conditions and liquidity, performance or achievements expressed or implied by such forward-looking statements. In addition, even if MorphoSys' results, performance, financial condition and liquidity, and the development of the industry in which it operates are consistent with such forward-looking statements, they may not be predictive of results or developments in future periods. Among the factors that may result in differences are MorphoSys' expectations regarding risks and uncertainties related to the impact of the COVID-19 pandemic to MorphoSys' business, operations, strategy, goals and anticipated milestones, including its ongoing and planned research activities, ability to conduct ongoing and planned clinical trials, clinical supply of current or future drug candidates, commercial supply of current or future approved products, and launching, marketing and selling current or future approved products, the global collaboration and license agreement for tafasitamab, the further clinical development of tafasitamab, including ongoing confirmatory trials, and MorphoSys' ability to obtain and maintain requisite regulatory approvals and to enroll patients in its planned clinical trials, additional interactions with regulatory authorities and expectations regarding future regulatory filings and possible additional approvals for tafasitamab as well as the commercial performance of Monjuvi, MorphoSys' reliance on collaborations with third parties, estimating the commercial potential of its development programs and other risks indicated in the risk factors included in MorphoSys' Annual Report on Form 20-F and other filings with the U.S. Securities and Exchange Commission. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements. These forward-looking statements speak only as of the date of publication of this document. MorphoSys expressly disclaims any obligation to update any such forward-looking statements in this document to reflect any change in its expectations with regard thereto or any change in events, conditions or circumstances on which any such statement is based or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements, unless specifically required by law or regulation.

The compounds discussed in this slide presentation are investigational products being developed by MorphoSys and its partners and are not currently approved by the U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA) or any other regulatory authority (except for tafasitamab/Monjuvi® and tafasitamab/Minjuvi® in relapsed or refractory DLBCL). The safety and efficacy of these investigational products have not been established and there is no guarantee any investigational product will be approved by regulatory authorities.

Monjuvi® and Minjuvi® are registered trademarks of MorphoSys AG.

# 01

## Welcome & Opening Remarks



**JEAN-PAUL KRESS, M.D.**  
Chief Executive Officer, MorphoSys

# Pelabresib and Ruxolitinib Combination Therapy: Potential to Shift Treatment Paradigm in Myelofibrosis

## OUR VISION:

Pelabresib Combination Therapy Becomes New Standard of Care in Myelofibrosis



**MANIFEST-2 Most  
Impressive Benefits  
Seen in Myelofibrosis**



**Excellent Operational  
Execution**  
*(Data readout six months  
ahead of schedule)*



**File for  
Approval in the U.S.  
and Ex-U.S.**



**Multi-Billion  
Dollar Market  
Opportunity**

**STRONG RUNWAY, WITH CASH AVAILABLE INTO 2025**

# Agenda

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01

## Welcome & Opening Remarks

Jean-Paul Kress, M.D., Chief Executive Officer, MorphoSys

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02

## Myelofibrosis Burden of Disease and Medical Need

Claire Harrison, DM, FRCP, FRCPath, Professor and Clinical Director Department of Hematology Guy's and St. Thomas' NHS Foundation Trust, London, United Kingdom

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03

## Phase 3 MANIFEST-2 Trial Results

Claire Harrison, DM, FRCP, FRCPath, Professor and Clinical Director Department of Hematology Guy's and St. Thomas' NHS Foundation Trust, London, United Kingdom

Ruben Mesa, M.D., FACP, President and Executive Director, Atrium Health Levine Cancer Center and Atrium Health Wake Forest Baptist Comprehensive Cancer Center

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04

## Pelabresib Clinical Development Next Steps

Tim Demuth, M.D., Ph.D., Chief Research and Development Officer, MorphoSys

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05

## Q&A

Moderated Q&A

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# 02

## Myelofibrosis Disease Burden and Medical Need



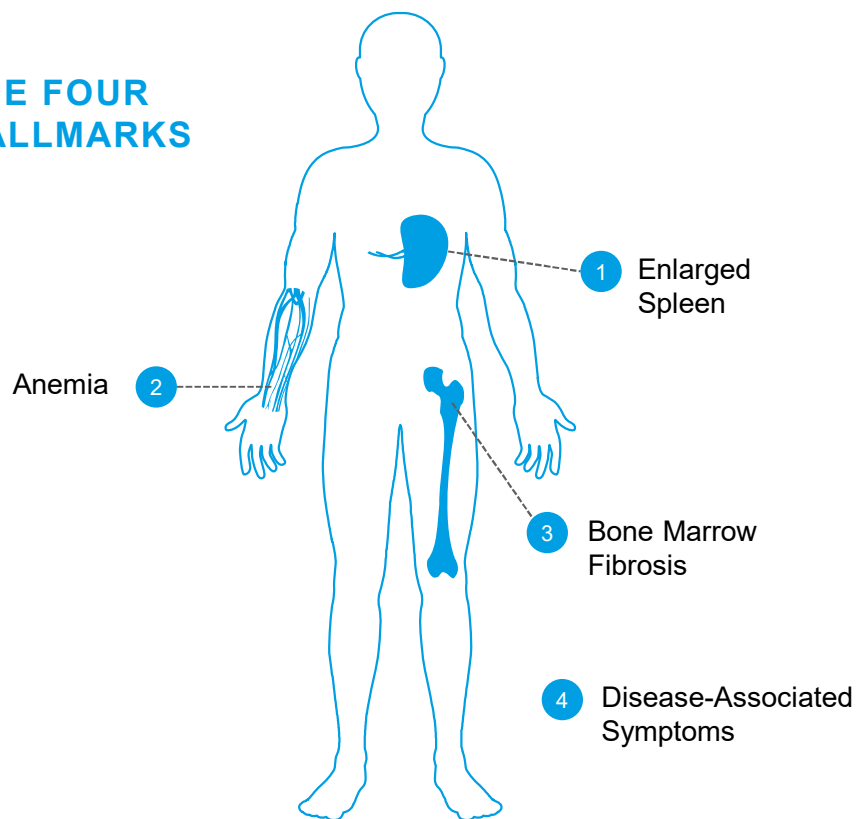
**PROFESSOR CLAIRE HARRISON**

Guy's and St. Thomas' NHS Foundation Trust, London, United Kingdom

# Myelofibrosis is a Debilitating, Progressive and Often Deadly Blood Cancer, Characterized by Four Hallmarks

*No currently approved myelofibrosis treatment addresses all four hallmarks of disease*

## THE FOUR HALLMARKS



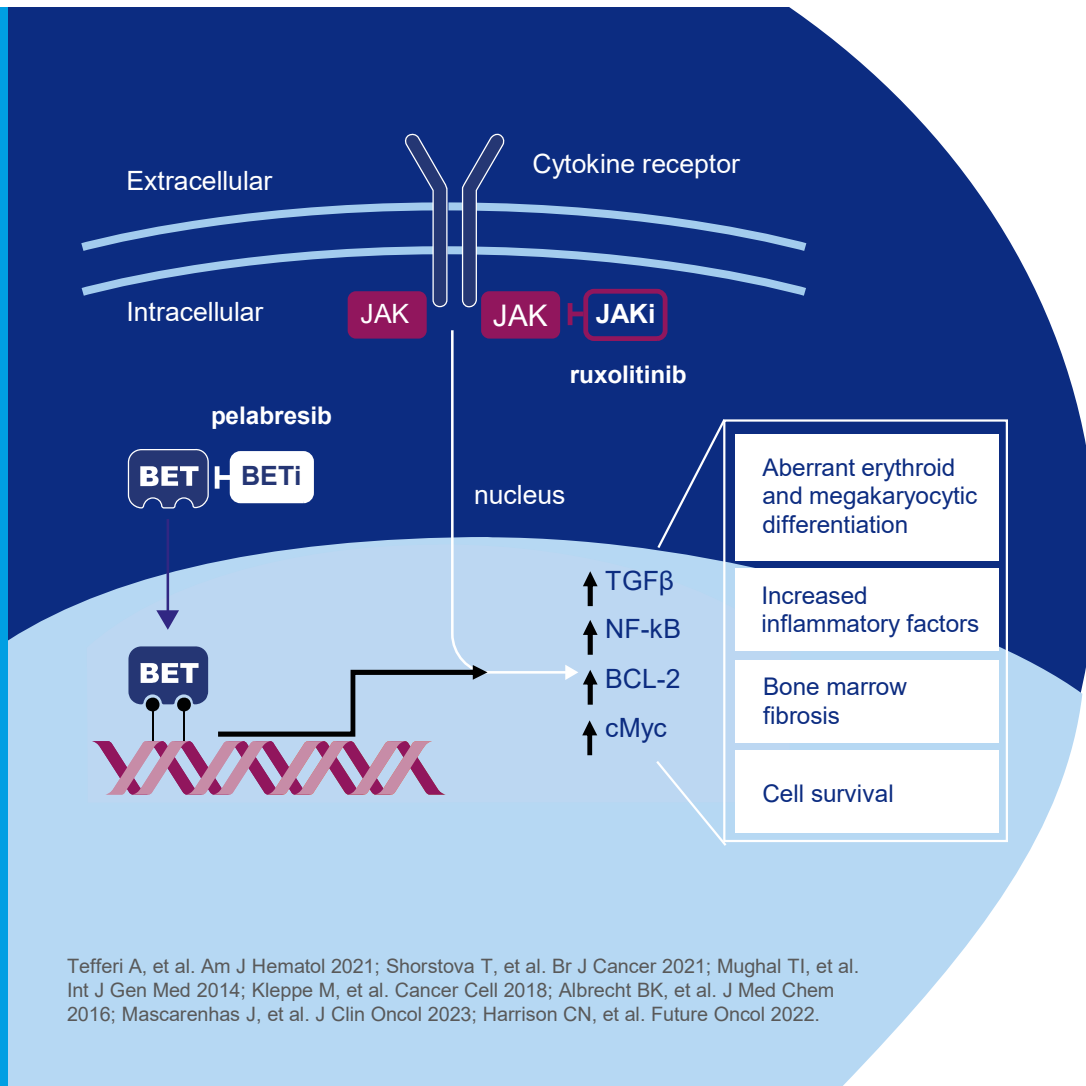
## DIAGNOSIS (DIPSS)\*

- + ~77-86% of patients have intermediate-risk\*\*
- + ~9-11% of patients have high-risk disease

## MEDIAN OVERALL SURVIVAL (DIPSS)\*

- + ~4 – 14.2 years for intermediate-risk\*\*
- + ~1.5 years for high-risk

\*Measured by Dynamic International Prognostic Scoring System (DIPSS) | \*\*Int-1 and Int-2  
Harrison C, et al. Expert Rev Hematol 2013; Al-Ali HK, et al. Haematologica 2016; Primary myelofibrosis. Orphanet. Accessed August 2023; Primary Myelofibrosis. MPN Research Foundation. Accessed August 2023; Mehta J, et al. Leuk Lymphoma 2014; Panda A, et al. Decision Resources Group 2022; Passamonti, F et al. Hematological Oncology 2021; Szuber N, et al. Mayo Clinic 2019; Passamonti, F et al. Blood 2010.



## JAK-STAT Pathway and BET Proteins Are Central to Myelofibrosis Pathology

### The combination of BET and JAK inhibition:

- Showed broad suppression of the proinflammatory molecules involved in bone marrow fibrosis *in vivo*
- Normalized the balance of precursor red blood cells and precursor platelet-forming cells in the bone marrow *in vivo*

Tefferi A, et al. Am J Hematol 2021; Shorstova T, et al. Br J Cancer 2021; Mughal TI, et al. Int J Gen Med 2014; Kleppe M, et al. Cancer Cell 2018; Albrecht BK, et al. J Med Chem 2016; Mascarenhas J, et al. J Clin Oncol 2023; Harrison CN, et al. Future Oncol 2022.



# Phase 2 MANIFEST Arm 3: Strong Improvements in Spleen Volume and Symptoms Across All Patient Risk Groups Form Basis for Phase 3 Study

*Durable responses were seen in SVR35 and TSS50 at 48 and 60 weeks*

STUDY POPULATION	PATIENT POPULATION BY DIPSS RISK CATEGORY	SVR35 (24 Weeks)	TSS50 (24 Weeks)
JAK-inhibitor-naïve myelofibrosis patients	<b>All Patients (N = 84)</b>	68%	56%
<b>ENDPOINTS</b>	Intermediate-1 (DIPSS) (N = 20)	70%	42%
<p><b>Primary:</b> SVR35 at 24 Weeks</p>	Intermediate-2 (DIPSS) (N = 51)	71%	57%
<p><b>Key Secondary:</b> TSS50 at 24 weeks (MFSAF v4.0)</p>	High-Risk (DIPSS) (N = 13)	54%	85%

Mascarenhas J, et al. J Clin Oncol. 2023;41(32): 4993-5004.

# 03

## Phase 3 MANIFEST-2 Trial Results



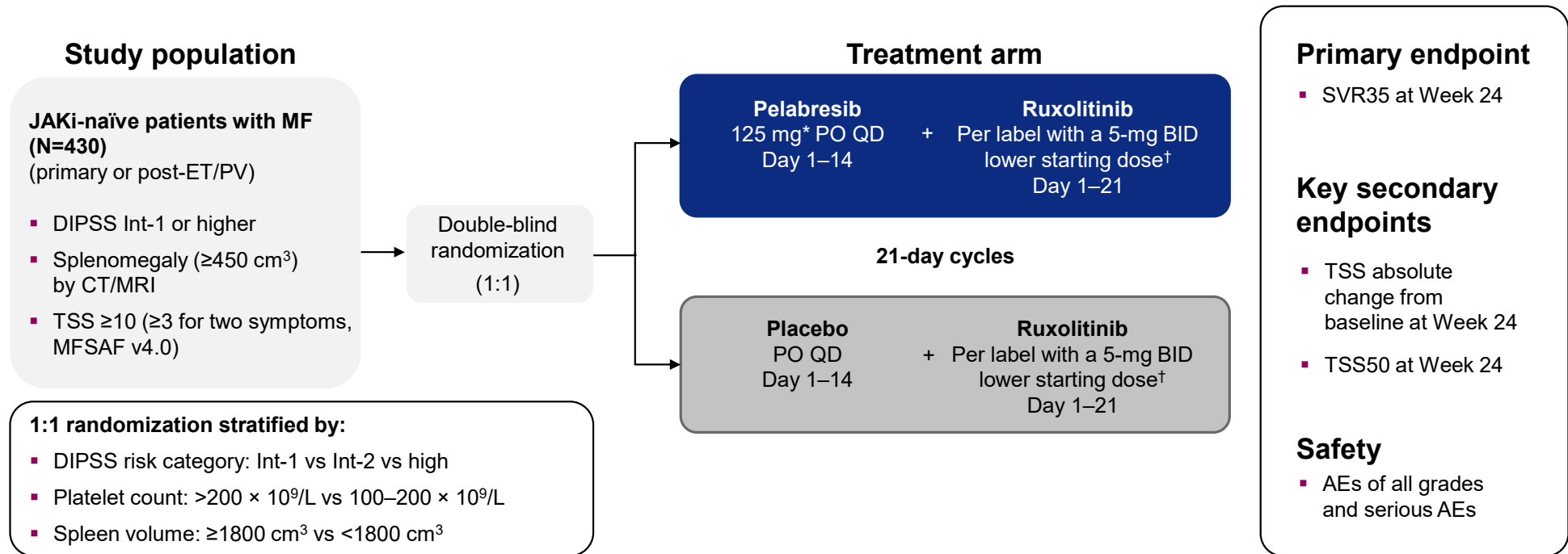
**PROFESSOR  
CLAIRE HARRISON**

Guy's and St. Thomas' NHS  
Foundation Trust, London, United  
Kingdom



**RUBEN MESA, M.D., FACP**  
President and Executive Director,  
Atrium Health Levine Cancer  
Center and Atrium Health Wake  
Forest Baptist Comprehensive  
Cancer Center

# Global, Randomized, Double-Blind, Active-Control, Phase 3 Study



AE, adverse event; BID, twice daily; CT, computed tomography; DIPSS, Dynamic International Prognostic Scoring System; ET, essential thrombocythemia; Int, intermediate; JAKi, Janus kinase inhibitor; MF, myelofibrosis; MFSAF, Myelofibrosis Symptom Assessment Form; MRI, magnetic resonance imaging; PO, orally; PV, polycythemia vera; QD, once daily; SVR35,  $\geq 35\%$  reduction in spleen volume; TSS, total symptom score; TSS50,  $\geq 50\%$  reduction in total symptom score. \*The starting dose for pelabresib was 125 mg QD and protocol-defined dose modifications based on AEs and treatment response allowed a dose range between 50 mg and 175 mg QD; †Ruxolitinib was started at 10 mg BID (baseline platelet count  $100\text{--}200 \times 10^9/\text{L}$ ) or 15 mg BID (baseline platelet count  $>200 \times 10^9/\text{L}$ ) with a mandatory dose increase by 5 mg BID after one cycle and a maximum dose of 25 mg BID per label. Harrison CN, et al. *Future Oncol.* 2022;18(27):2987-29977.

# Patient disposition

	Pelabresib + ruxolitinib	Placebo + ruxolitinib
Randomized	214 (100%)	216 (100%)
Treated	212 (99.1%)	214 (99.1%)
Discontinued double-blind treatment	58 (27.1%)	54 (25.0%)
Adverse event	23 (10.7%)	14 (6.5%)
Physician decision (including lack of benefit)	9 (4.2%)	20 (9.3%)
Disease progression	5 (2.3%)	5 (2.3%)
Eligible for transplant	8 (3.7%)	9 (4.2%)
Other*	13 (6.0%)	6 (2.8%)
Ongoing on double-blind treatment	154 (72.0%)	160 (74.1%)

	Pelabresib + ruxolitinib	Placebo + ruxolitinib
Mean daily dose for pelabresib (14 days per 21-day cycle)	108 mg	N/A
Mean daily dose for ruxolitinib (daily)	29.3 mg	31.3 mg
Median follow-up on study (weeks)	45.4	

**Data cut off: August 31, 2023.** N/A, not applicable. \*Other: non-compliance, withdrawal of consent. The study opened for enrollment in November 2020; the first patient received their initial treatment on April 22, 2021, and the last patient received their first treatment on March 2, 2023. Percentages reported are based on the number of patients randomized (intent-to-treat set).

# Baseline disease characteristics

Characteristic		Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)
Age — years	Median (min, max)	66 (19, 84)	66 (26, 88)
Sex — n (%)	Female / male	85 (39.7) / 129 (60.3)	94 (43.5) / 122 (56.5)
Race — n (%)	White / Asian / Black	160 (74.8) / 35 (16.4) / 2 (0.9)	163 (75.5) / 42 (19.4) / 0
	American Indian or Alaska Native	1 (0.5)	0
	Not reported / Unknown	15 (7.0) / 1 (0.5)	11 (5.1) / 0
Myelofibrosis subtype — n (%)	Primary myelofibrosis	107 (50)	110 (50.9)
	Post-polycythemia vera myelofibrosis	45 (21)	53 (24.5)
	Post-essential thrombocytopenia myelofibrosis	62 (29)	53 (24.5)
Dynamic International Prognostic Scoring System — n (%)	Intermediate-1	128 (59.8)	127 (58.8)
	Intermediate-2	75 (35)	74 (34.3)
	High-risk	11 (5.1)	15 (6.9)
Mutations — n (%)*	JAK2 V617F	125 (67.2)	122 (64.6)
	CALR	45 (24.2)	50 (26.5)
	MPL	11 (5.9)	13 (6.9)
	Triple negative	8 (4.3)	5 (2.6)
	High-molecular risk mutations	72 (38.7)	88 (46.6)
	Missing	28 (13.1)	27 (12.5)
Hemoglobin — g/dL	Median (range)	10.9 (5.8–18.0)	11.0 (6.7–17.9)
	≤10 — n (%)	70 (32.7)	76 (35.2)
Platelets — × 10 <sup>9</sup> /L	Median (min, max)	285 (99, 1303)	287 (66, 1084)
	>200 × 10 <sup>9</sup> /L — n (%)	154 (72)	157 (72.7)
Peripheral blasts	Mean (SD)	0.8 (1.18) <sup>†</sup>	0.8 (1.25) <sup>‡</sup>
RBC transfusions — patient n (%)	Requiring RBC transfusion at baseline	35 (16)	25 (12)
	0	107 (50)	109 (50.5)
ECOG performance status — n (%)	1	97 (45.3)	95 (44.0)
	≥2	10 (4.7)	10 (4.6)
	Missing	0	2 (0.9)
Spleen volume (central read) <sup>§</sup>	Median spleen volume (range) — cc	1308.89 (200.24–7117.03)	1382.97 (277.87–5540.45)
Total symptom score <sup>¶</sup>	Median total symptom score (range)	26.6 (7.3–66.4)	24.7 (9.0–68.4)

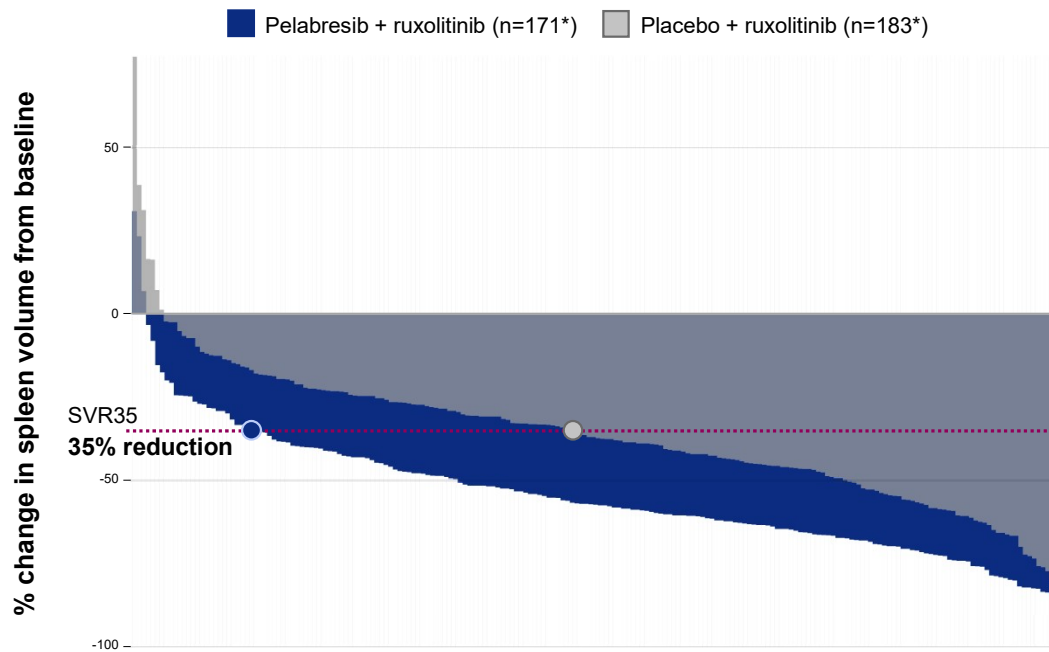
**Data cut off: August 31, 2023.** CALR, calreticulin; ECOG, Eastern Cooperative Oncology Group; JAK, Janus kinase; max, maximum; min, minimum; MPL, MPL proto-oncogene, thrombopoietin receptor; RBC red blood cell; SD, standard deviation. \*Results do not originate from a validated programming environment. <sup>†</sup>n=208. <sup>‡</sup>n=207. <sup>§</sup>Randomization of patients was based on local read. <sup>¶</sup>Patients with baseline TSS values of <10 have at least 2 individual symptoms score ≥ 3 at baseline.

Rampal R, et al. ASH 2023. Oral 628

Pelabresib (CPI-0610) is an investigational new drug and has not been approved by any regulatory authority

# MANIFEST-2 study achieved its primary endpoint: SVR35 at Week 24

Significantly greater response in patients treated with pelabresib + ruxolitinib vs placebo + ruxolitinib



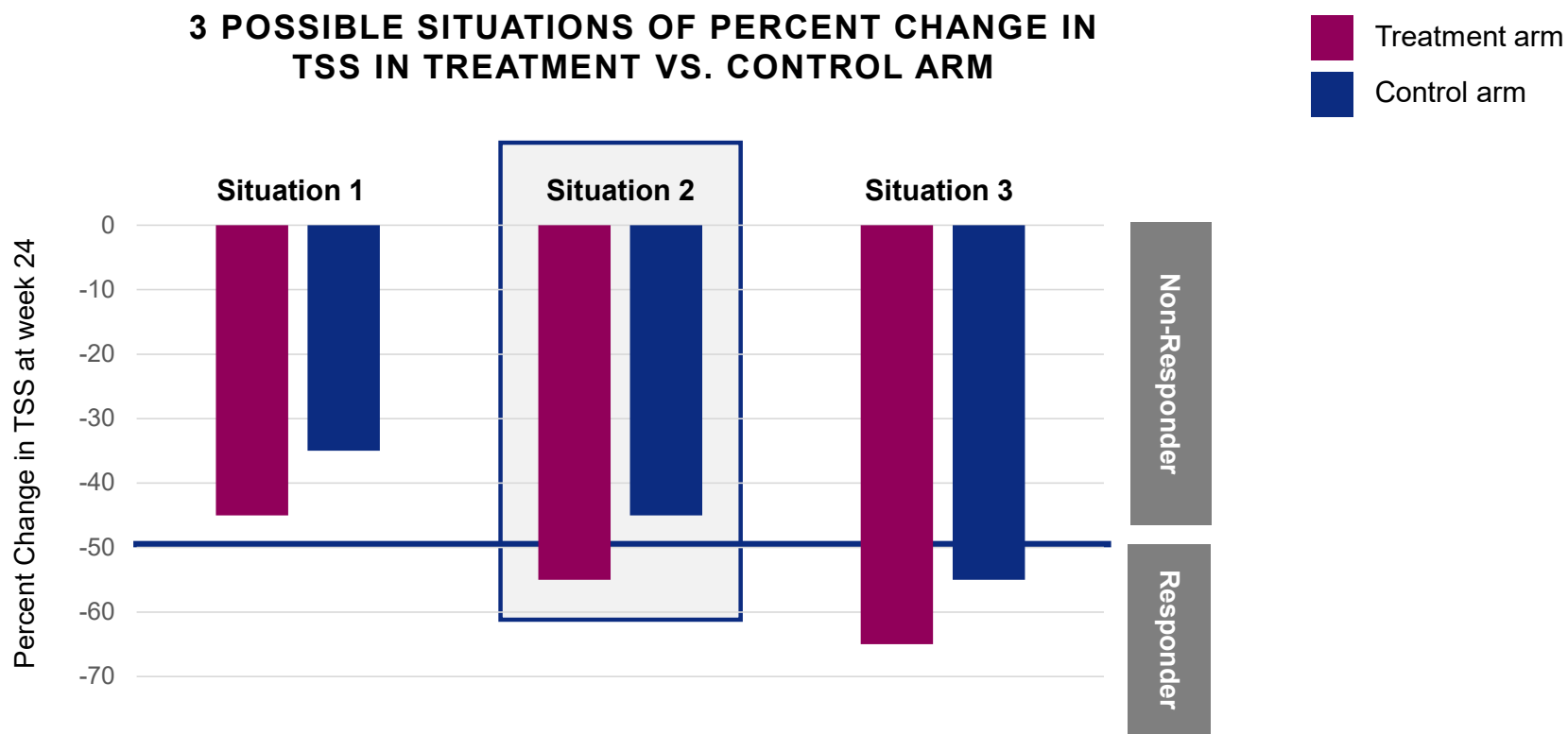
ITT population

	Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)	p-value
<b>SVR35 at Week 24</b>	65.9%	35.2%	
<b>Difference<sup>†</sup> (95% CI)</b>	30.4 (21.6, 39.3)		<0.001

<b>Mean % change in spleen volume at Week 24<sup>‡</sup></b>	-50.6 (n=171)	-30.6 (n=183)	
<b>95% CI</b>	-53.2, -48	-33.7, -27.5	

Data cut off: August 31, 2023. CI, confidence interval; ITT, intent-to-treat; SVR35,  $\geq 35\%$  reduction in spleen volume. Spleen volume assessed by central read. \*Waterfall plots represent patients who have baseline and Week 24 data. <sup>†</sup>Calculated by stratified Cochran–Mantel–Haenszel test; <sup>‡</sup>Patients without Week 24 assessment are considered non-responders.

# TSS50 Limits Detection of Symptom Benefit, Absolute Change More Comprehensive View of Symptom Improvement

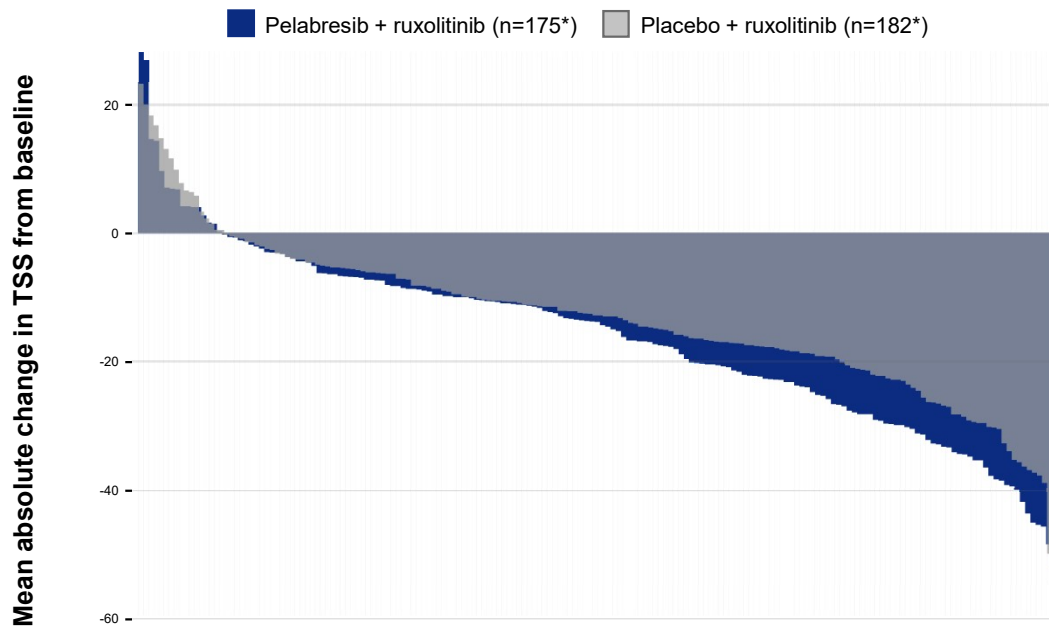


## CONCLUSIONS:

- Continuous TSS change will detect the difference between the arms in ALL 3 situations
- TSS50 will only be able to detect a difference between the arms in Situation 2

# Absolute TSS at Week 24

Strong numerical improvements for patients treated with pelabresib + ruxolitinib vs placebo + ruxolitinib



ITT population

	Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)	p-value
TSS change <sup>†</sup> from baseline at Week 24	-15.99	-14.05	
Mean difference <sup>‡</sup> (95% CI)	-1.94 (-3.92, 0.04)		0.0545

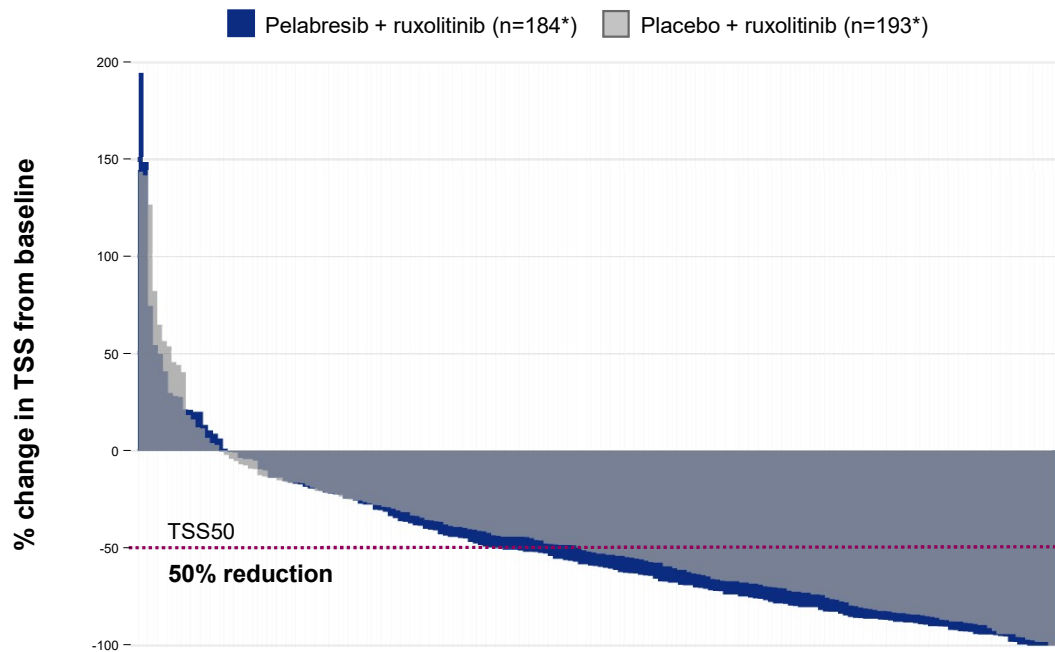
- **Absolute change in TSS is a continuous endpoint** that estimates magnitude of symptom burden reduction with enhanced precision

**Data cut off: August 31, 2023.** ANCOVA, analysis of covariance; CI, confidence interval; ITT, intent-to-treat; TSS, total symptom score. \*Waterfall plots represent patients who have baseline and Week 24 data. <sup>†</sup>Change from baseline determined by ANCOVA model using Multiple Imputation. <sup>‡</sup>Least square mean difference from ANCOVA model using baseline DIPSS, baseline platelet count and baseline spleen volume as factors, and baseline TSS as covariate.



# TSS50 Response at Week 24

Numerically greater in patients treated with pelabresib + ruxolitinib vs placebo + ruxolitinib



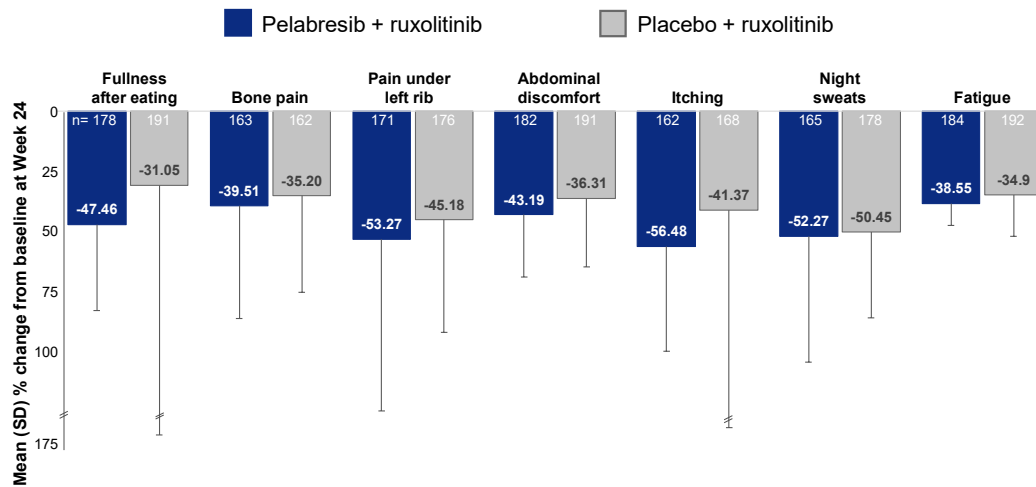
ITT population

	Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)	p-value
<b>TSS50 at Week 24</b>	52.3%	46.3%	
<b>Difference† (95% CI)</b>	6.0 (-3.5, 15.5)		0.216

**Data cut off: August 31, 2023.** CI, confidence interval; ITT, intent-to-treat; TSS, total symptom score; TSS50, ≥50% reduction in total symptom score. Patients are evaluable for TSS50 at Week 24 if they have had Week 24 TSS assessment by the data cutoff date or discontinued without Week 24 assessment at any time. \*Waterfall plots represent patients who have baseline and Week 24 data. †Difference in treatment groups analyzed by stratified Cochran–Mantel–Haenszel test (weighted 95% CI adjusted across strata).

# TSS Domains at Week 24

Numerically greater in patients treated with pelabresib + ruxolitinib vs placebo + ruxolitinib with benefit balanced across all TSS domains



ITT population

	Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)	p-value
TSS50 at Week 24	52.3%	46.3%	
Difference <sup>†</sup> (95% CI)	6.0 (-3.5, 15.5)		0.216

Data cut off: August 31, 2023. CI, confidence interval; ITT, intent-to-treat; TSS, total symptom score; TSS50, ≥50% reduction in total symptom score. Patients are evaluable for TSS50 at Week 24 if they have had Week 24 TSS assessment by the data cutoff date or discontinued without Week 24 assessment at any time. <sup>†</sup>Difference in treatment groups analyzed by stratified Cochran–Mantel–Haenszel test (weighted 95% CI adjusted across strata).

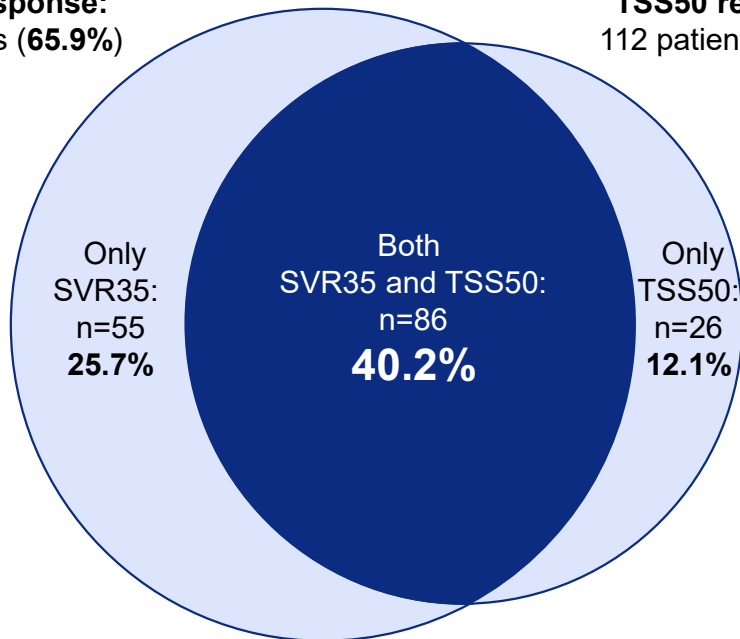
# Dual SVR35 / TSS50 Responders at Week 24

Two-fold increase in patients achieving both SVR35 and TSS50 with pelabresib + ruxolitinib vs placebo + ruxolitinib

**Pelabresib + ruxolitinib (N=214)**

**SVR35 response:**  
141 patients (65.9%)

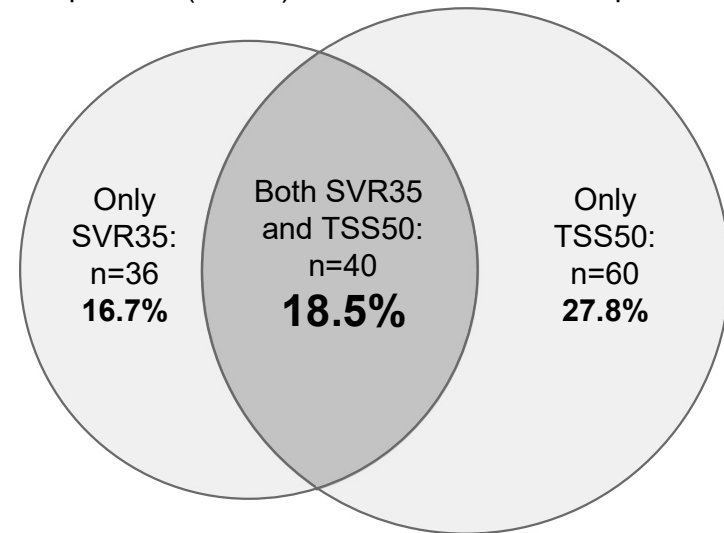
**TSS50 response:**  
112 patients (52.3%)



**Placebo + ruxolitinib (N=216)**

**SVR35 response:**  
76 patients (35.2%)

**TSS50 response:**  
100 patients (46.3%)



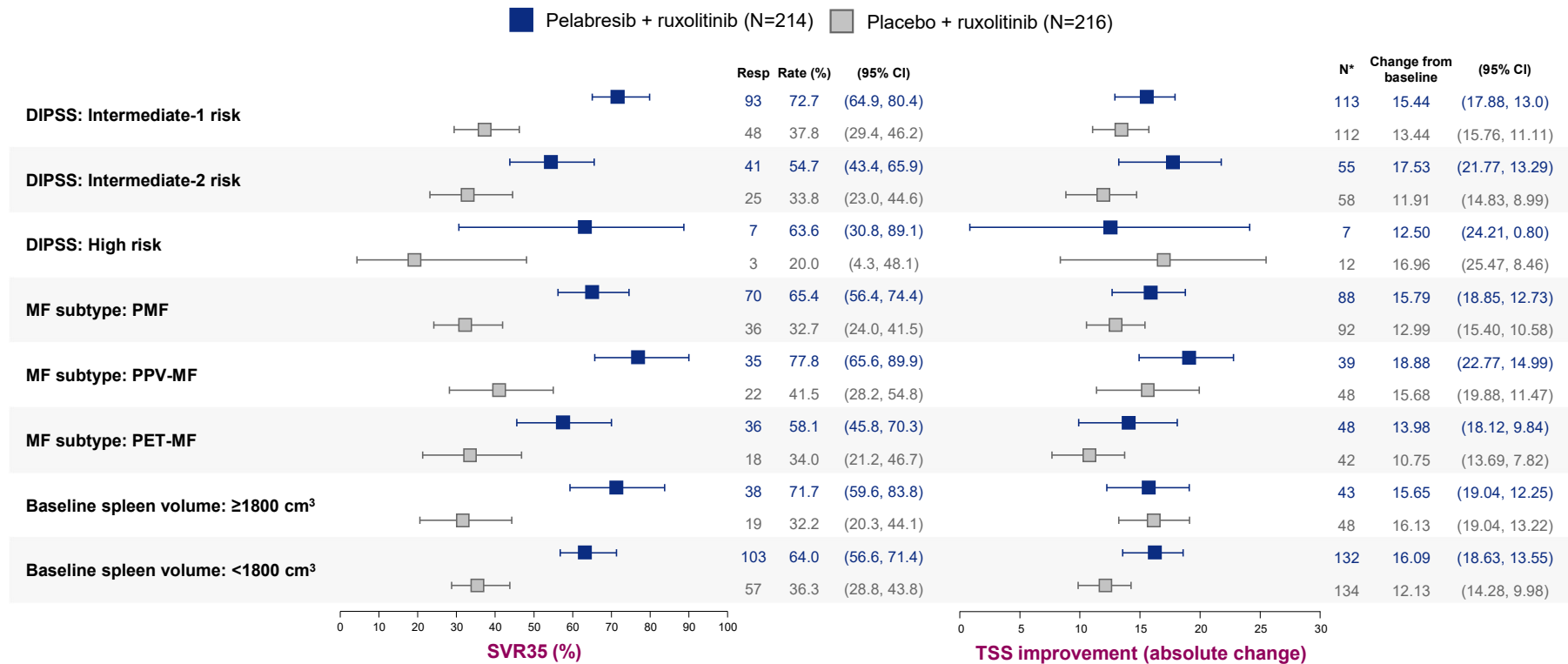
Data cut off: August 31, 2023. N, number of patients; SVR35,  $\geq 35\%$  reduction in spleen volume; TSS50,  $\geq 50\%$  reduction in total symptom score. Diagrams are not drawn to scale.

Rampal R, et al. ASH 2023. Oral 628

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# Prespecified Subgroup Analyses at Week 24

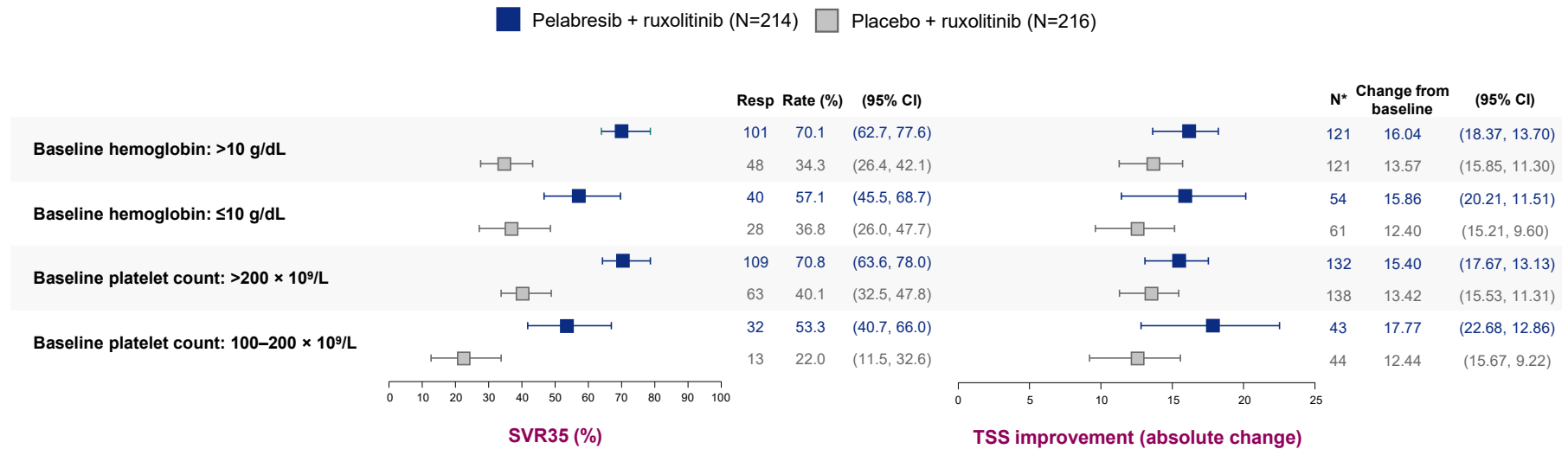
SVR35 response consistently higher in pelabresib + ruxolitinib vs placebo + ruxolitinib across all predefined subgroups



Data cut off: August 31, 2023. CI, confidence interval; DIPSS, Dynamic International Prognostic Scoring System; MF, myelofibrosis; PET-MF, post-essential thrombocythemia myelofibrosis; PMF, primary myelofibrosis; PPV-MF, post-polycythemia vera myelofibrosis; Resp, number of responders; SVR35, ≥35% reduction in spleen volume; TSS, total symptom score. \*Number of patients with Week 24 observations.

# Prespecified Hematologic Subgroup Analyses at Week 24

SVR35 response consistently higher in pelabresib + ruxolitinib combination vs placebo + ruxolitinib across hematologic subgroups



Data cut off: August 31, 2023. CI, confidence interval; Resp, number of responders; SVR35, ≥35% reduction in spleen volume; TSS, total symptom score. \*Number of patients with Week 24 observations.

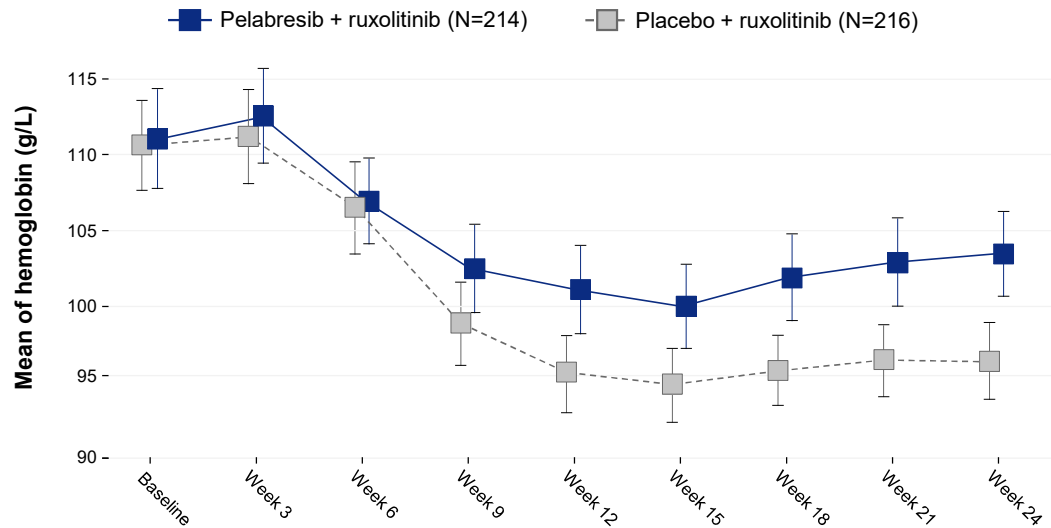
# High-Risk Patient SVR35 and TSS50 Response: Phase 2 MANIFEST Arm 3 vs. Phase 3 MANIFEST-2

Symptom response seen in the high-risk patient group (5% of pelabresib/ruxolitinib MANIFEST-2 arm)  
is a numerical anomaly

PATIENT POPULATION (PELABRESIB + RUXOLITINIB)	SVR35 (24 WEEKS)	TSS50 (24 WEEKS)
<b>Phase 3 MANIFEST-2</b>		
All Patients (N = 214)	66%	52%
High-Risk DIPSS (N = 11)	64%	21%
<b>Phase 2 MANIFEST Arm 3</b>		
All Patients (N = 84)	68%	56%
High-Risk DIPSS (N = 13)	54%	85%

# Hemoglobin Response

A numerically greater proportion of patients achieved hemoglobin response with pelabresib + ruxolitinib vs placebo + ruxolitinib



Number of patients

	Baseline	Week 3	Week 6	Week 9	Week 12	Week 15	Week 18	Week 21	Week 24
Pelabresib + ruxolitinib	212	204	209	199	193	189	186	185	184
Placebo + ruxolitinib	214	206	211	209	207	205	204	199	196

ITT population

	Pelabresib + ruxolitinib (N=214)	Placebo + ruxolitinib (N=216)
Hemoglobin response* $\geq 1.5$ g/dL mean increase (95% CI)	9.3% (5.45, 13.25)	5.6% (2.50, 8.61)
Patients requiring RBC transfusion during screening, n (%)	35 (16.4)	25 (11.6)
Patients requiring RBC transfusion during first 24 weeks of study treatment, n (%)	66 (30.8)	89 (41.2)

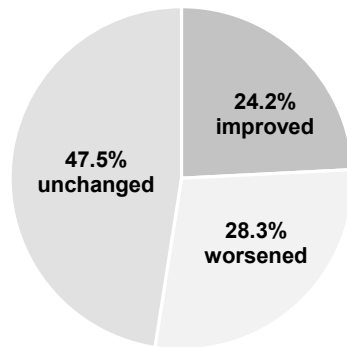
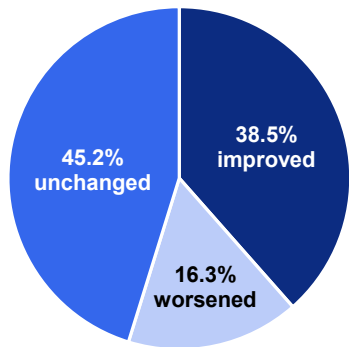
**Preliminary Analyses from Data cut off: August 31, 2023.** CI, confidence interval; RBC, red blood cell. \*Hemoglobin response is defined as a  $\geq 1.5$  g/dL mean increase in hemoglobin from baseline in the absence of transfusions during the previous 12 weeks. Baseline hemoglobin defined as the last assessment prior to or on Cycle 1 Day 1, regardless of blood transfusions. A similar effect was observed across DIPSS categories.

# Reduction in Bone Marrow Fibrosis and Inflammatory Cytokines

## Improvement of reticulin fibrosis grade in central read by Week 24

Pelabresib + ruxolitinib (n=104\*)

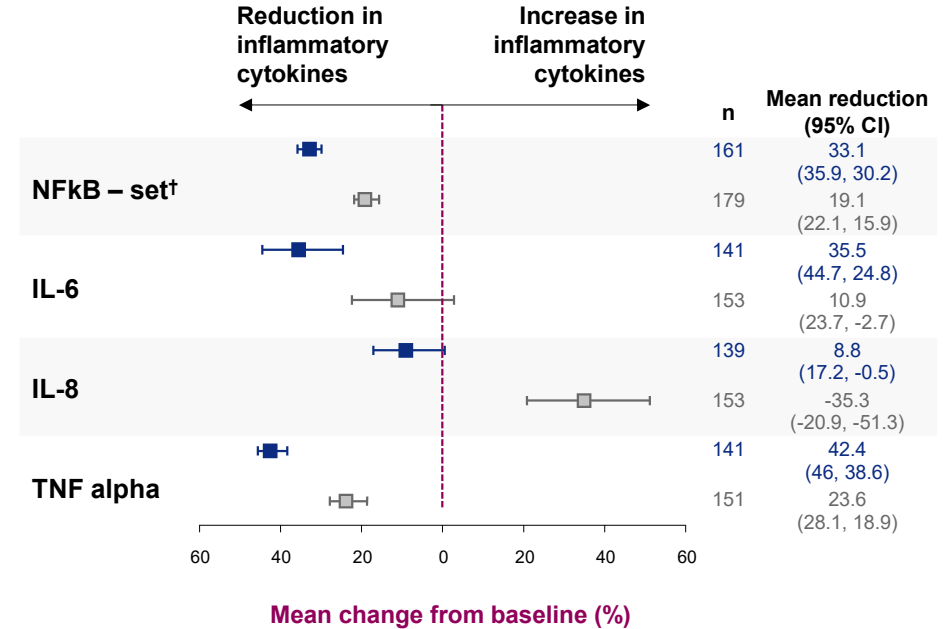
Placebo + ruxolitinib (n=99\*)



	Pelabresib + ruxolitinib	Placebo + ruxolitinib	Odds ratio
Worsened ≥1 grade (%)	16.3	28.3	0.47 (0.23-0.92)
Improved ≥1 grade (%)	38.5	24.2	2.09 (1.14-3.93)

## Reduction of inflammatory cytokines by Week 24

■ Pelabresib + ruxolitinib (N=214) □ Placebo + ruxolitinib (N=216)

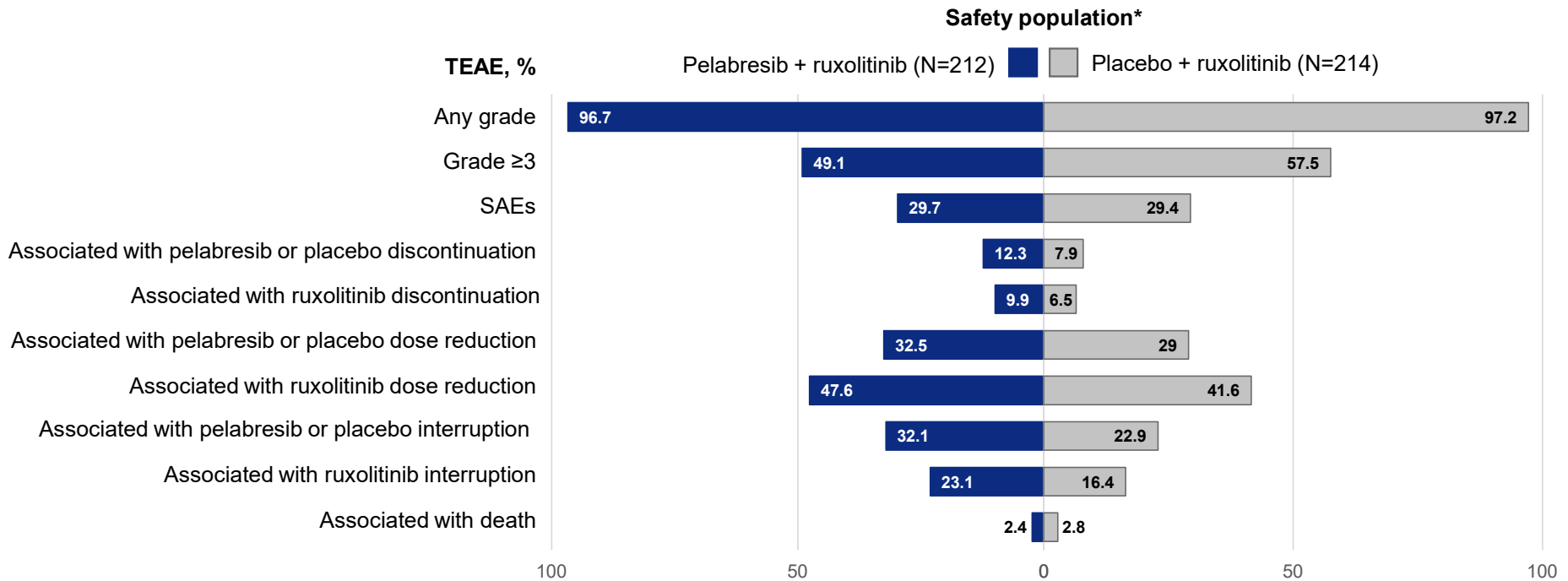


**Preliminary Analyses from Data cut off: August 31, 2023.** IL-6, interleukin 6; IL-8, interleukin 8; NFκB, Nuclear factor kappa-light-chain-enhancer of activated B cells; TNF, tumor necrosis factor. \*n=203 evaluable patients (baseline & C9D1). †NFκB - set includes: B2M, CRP, CD40-L, HEPCIDIN, IL-6, IL-12p40, MIP-1 BETA, MPIF-1, RANTES, TNFR2, TNF alpha, VCAM-1.



# Summary of Safety

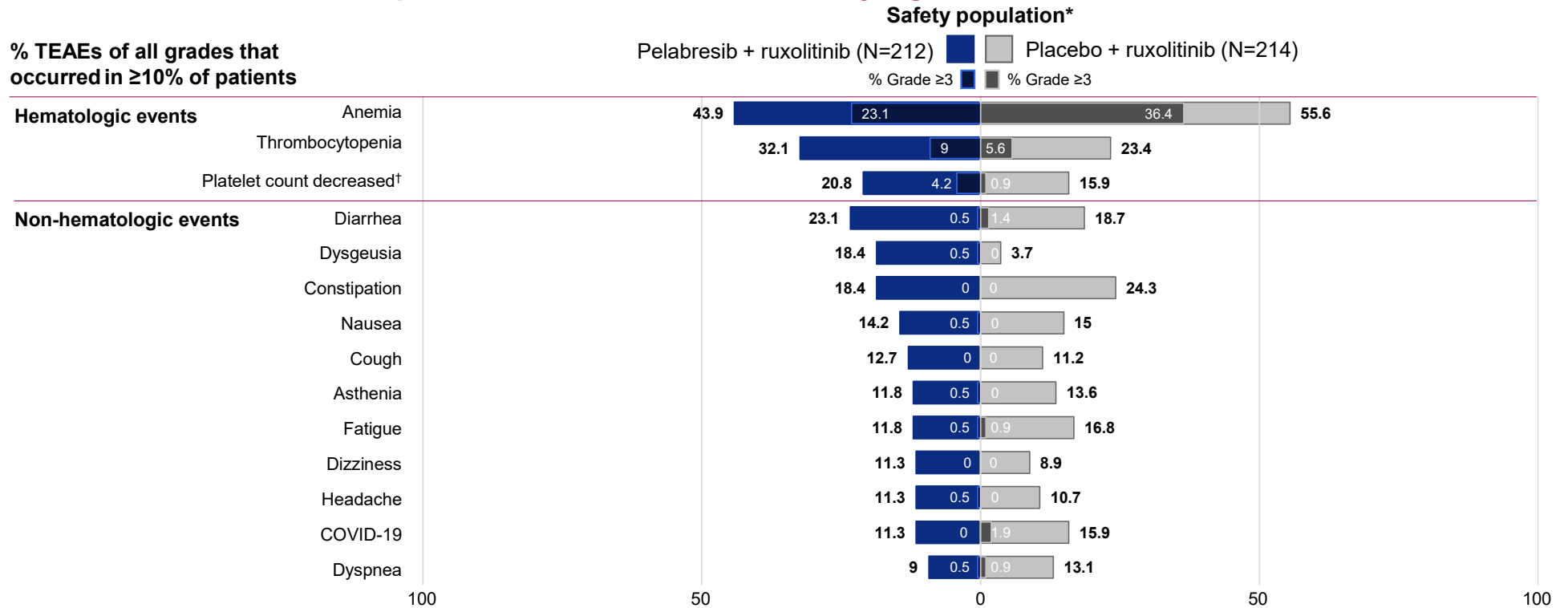
The safety profile of the pelabresib + ruxolitinib combination was consistent with prior trials



**Preliminary Analyses from Data cut off: August 31, 2023.** TEAE, treatment-emergent adverse event; SAE, serious adverse event. \*Safety population: received at least one dose of study drug. TEAEs are regardless of relationship to study drug. A TEAE for the double-blinded treatment period is defined as an adverse event that has a start date on or after the first dose of the pelabresib/placebo and before 30 days after the last dose of pelabresib/placebo or before the start of alternative (off-study) treatment for MF, whichever occurs first. MF, myelofibrosis.

# Treatment-Emergent Adverse Events

Adverse events of anemia were reported less frequently with pelabresib + ruxolitinib combination than with placebo + ruxolitinib; no new safety signals were observed



**Preliminary Analyses from Data cut off: August 31, 2023.** TEAE, treatment-emergent adverse event. \*Safety population: received at least one dose of study drug. †Platelet count decreased was classified under the system organ class of investigation. TEAEs are regardless of relationship to study drug. A TEAE for the double-blinded treatment period is defined as an adverse event that has a start date on or after the first dose of the pelabresib/placebo and before 30 days after the last dose of pelabresib/placebo or before the start of alternative (off-study) treatment for MF, whichever occurs first. MF, myelofibrosis; COVID-19, coronavirus disease 2019.

## Conclusions

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- **Pelabresib in combination with ruxolitinib compared with placebo in combination with ruxolitinib in JAK inhibitor treatment-naïve patients at Week 24:**
  - Significantly reduced splenomegaly (SVR35: 66% vs 35%;  $p < 0.001$ )
  - Demonstrated strong trends in reducing the mean absolute TSS ( $p = 0.0545$ ) and improving TSS50 response
  - Doubled the percentage of patients with dual SVR35 / TSS50 response
- **Fewer anemia adverse events, higher rates of hemoglobin responses and fewer patients with transfusion requirement**
- **The safety profile appeared generally comparable to the established safety profile of ruxolitinib with fewer grade  $\geq 3$  events**
- **Pelabresib in combination with ruxolitinib showed reduction of pro-inflammatory cytokines, and improvement in bone marrow fibrosis and anemia response, addressing the four hallmarks of myelofibrosis**

**These results support a potential paradigm shift in the treatment of patients with myelofibrosis**

JAK, Janus kinase; SVR35,  $\geq 35\%$  reduction in spleen volume; TSS50,  $\geq 50\%$  reduction in total symptom score.

Rampal R, et al. ASH 2023. Oral 628

Pelabresib (CPI-0610) is an investigational new drug and has not been approved by any regulatory authority

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# 04

## Pelabresib First-line Myelofibrosis Clinical Development Next Steps



**TIM DEMUTH, M.D., Ph.D.**

Chief Research & Development Officer, MorphoSys

# Majority of U.S. Physicians View Combination Therapy as the “Way of the Future” in Myelofibrosis

## MAJORITY IMPRESSED BY THE IMPROVED EFFICACY OF COMBINATION THERAPY

**HESITANT TO UTILIZE**  
(13%)

**OPEN TO UTILIZE**  
(87%)



## PELABRESIB RANKED AMONG THE HIGHEST IN TOP ATTRIBUTES DRIVING TREATMENT DECISIONS

**IMPRESSIVE EFFICACY**  
(Spleen Volume Reduction, Symptom Improvement)  
Mentioned by ~85% of HCPs

**HEMATOLOGIC FUNCTION**  
(Transfusion Dependency, Hemoglobin Count, Quality of Life)  
Mentioned by ~70% of HCPs

**LOW RATES OF HEMATOLOGIC ADVERSE EVENTS**  
(Anemia, Thrombocythemia, Neutropenia)  
Mentioned by ~70% of HCPs

MF Drivers and Barriers Qualitative Market Research, Aug 2023 | N=23 MF treating US Hem Oncs & Med Oncs; Product attributes rated based on Target Product Profile for pelabresib

# Execution and Next Steps of Pivotal MANIFEST-2 Results



## Prepare and File Regulatory Submissions

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- Intend to file for approval in U.S. and Europe in mid-2024
- Submit comprehensive MANIFEST-2 data package



## Advance Scientific Publications and Medical Education

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- Collect longer-term data, including quality of life and duration of treatment
- Leverage experienced U.S. Medical Science Liaison team with established physician relationships

## Committed to Bringing Pelabresib to Myelofibrosis Patients in Dire Need Of New Treatment Options

“ The combination of pelabresib and ruxolitinib, for the **first time demonstrates**, with strong clinical data, a **potential paradigm shift to combination therapy.** ”



**JOHN MASCARENHAS, M.D.**

*Director of the Adult Leukemia Program at The Tisch Cancer Institute at Mount Sinai, New York*

“ **MANIFEST-2 demonstrated clear benefits** across the four hallmarks, including a significant reduction in **spleen size** – a key finding given the **known association** between spleen volume reduction and **patient survival.** ”



**RAAJIT K. RAMPAL, M.D., PH.D.**

*Director, Center for Hematologic Malignancies, and Director, Myeloproliferative Neoplasms Program, Memorial Sloan Kettering Cancer Center*

# 05

## Q&A



**JEAN-PAUL  
KRESS, M.D.**



**TIM DEMUTH,  
M.D., Ph.D.**



**LUCINDA CRABTREE,  
Ph.D.**



**PROFESSOR CLAIRE  
HARRISON**



**RUBEN MESA,  
M.D., FACP**





**morphosys**

**Thank you!**

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