



Clinical Data from SUMMIT Part 1b

Investor Webcast
February 23, 2024

Real Challenges. Real Solutions.

Precision therapies for genetically defined diseases

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Agenda and Speakers



Andrew Robbins
President and
Chief Executive Officer



Frank Siebenhaar, M.D.
Head, University Outpatient Clinic,
Institute of Allergology, Charite -
Universitätsmedizin Berlin





Jessica Sachs, M.D.
Chief Medical Officer

• Introduction and Corporate Overview	Andrew Robbins
• Review of SUMMIT Part 1b Data with Bezuclastinib in Nonadvanced Systemic Mastocytosis	Dr. Frank Siebenhaar
• Presentation Summary	Andrew Robbins
• Q&A	All

Multiple Clinical and Preclinical Programs with Upcoming Catalysts

Program	Indication	Early Stage Development	Late Stage Development	Regulatory Submission	Approval
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Clinical Programs

Bezuclastinib (KIT inhibitor)	Advanced Systemic Mastocytosis		• APEX Part 2 (Registration-Directed) • Top-line results by mid-2025
	Nonadvanced Systemic Mastocytosis		• SUMMIT Part 2 (Registration-Directed) • Top-line results by YE 2025
	Gastrointestinal Stromal Tumors		• PEAK Part 2 (Global Phase 3 trial) • Top-line results by YE 2025

Research Programs

Indication	Hit ID	Lead Generation	Lead Optimization	Candidate Selected	IND Submission
ErbB2 mut					
FGFR2					
PI3Kα					
Target 4					
Target 5					
Target 6					

Objectives of Today's Presentation

- Review Summit Part 1b results, with a focus on newly announced RP2D 100 mg optimized formulation:
 - Well tolerated safety profile, generally consistent with placebo
 - Impressive pharmacodynamic effects, evidence of strong target engagement
 - 51% mean change in TSS with 70% of patients achieving $\geq 50\%$ reduction at week 12
 - 49% mean improvement in quality-of-life (McQoL) at week 12
- Introduce MS2D2, a novel, refined patient reported outcomes measure (PROM) for symptomatic severity in NonAdvSM patients
- Announce the initiation of SUMMIT Part 2, a 159-patient, registration-directed clinical trial of bezuclastinib vs. placebo in NonAdvSM patients
- Provide clinical context to the SUMMIT Part 1b findings
- Review upcoming milestones for Cogent Biosciences

Systemic Mastocytosis (SM) is a Rare and Debilitating Disease of Neoplastic Mast Cells with Significant Unmet Medical Need Remaining for New Therapies¹

- Nonadvanced SM (NonAdvSM)² includes smoldering SM (SSM),³ for which no therapies are approved, as well as indolent SM (ISM).
- Patients with NonAdvSM experience a variety of disabling, potentially serious and severe symptoms caused by mast cell degranulation, including life-threatening anaphylaxis.⁴
- Agents targeting KIT D816V are used to treat Advanced SM (AdvSM) and NonAdvSM, but unmet need remains.⁵⁻⁷
- Adverse events, such as cognitive impairment, bleeding, and edema, may limit dosing of other agents.

Development of MS2D2 Total Symptom Score

- Rigorous process, in accordance with FDA guidelines, was followed for the development of a novel patient reported outcomes measure (PROM)
- Literature review, patient and physician interviews, and data from SUMMIT Part 1 were used to design a reliable, valid and fit-for-purpose PROM
- Pending FDA approval, a comparison of week 24 mean absolute change from baseline in MS2D2 score between bezuclastinib and placebo will serve as the primary endpoint of SUMMIT Part 2

MS2D2 TSS Additions based on:

- Literature review
- Patient interviews
- SUMMIT Part 1 psychometric analysis

Itching
Flushing
Spots
Skin redness
Difficulty Concentrating
Difficulty Remembering
Nausea
Abdominal Pain
Headache
Bone Pain
Feeling Tiredness

MS2D2 TSS Exclusions based on:

- FDA feedback
- KOL advice
- SUMMIT Part 1 psychometric analysis

Brain Fog

Dizziness

Diarrhea Severity

Each of these items will be collected as part of MS2D2 secondary analyses in SUMMIT Part 2

MS2D2 TSS comprised of 11 items scored on 0-110 scale

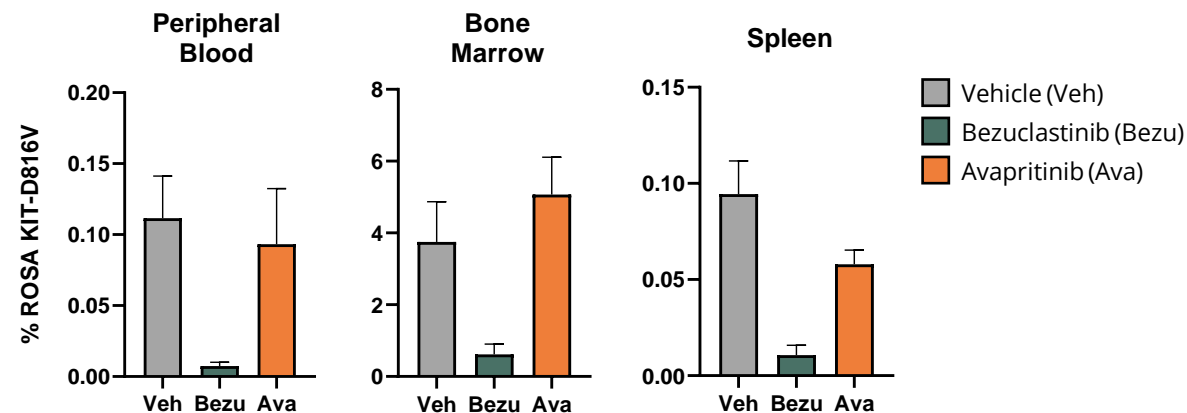
Nonclinical Data Suggests Optimal Activity Against Mastocytosis May Require Higher Exposures Than Clinically Tolerable With Available Therapy

- Mice engrafted with SCF-independent human ROSA^{KIT D816V} cells⁸ were treated daily for 8 weeks with a KIT inhibitor at doses matching clinical exposures observed in NonAdvSM patients
- Only bezuclastinib led to statistically significant decreases ($p < 0.05$) in mutant MC burden compared to vehicle
- At exposures comparable to those achieved in NonAdvSM patients, bezuclastinib led to statistically significant decreases ($P < 0.05$) in bone marrow and spleen compared to avapritinib

Total Drug Exposure Ratio Measured in SM Mouse Model

	Mouse Plasma AUC ₀₋₂₄ (ng·hr/mL) ^a	NonAdvSM Clinical Plasma AUC ₀₋₂₄ (ng·hr/mL) ^b	Total Drug Exposure Ratio (mouse/clinic)
Bezuclastinib	11775	16900	0.7X
Avapritinib	2118	1548	1.4X

MC Burden in SM Mouse Model



Initial Results from Summit: An Ongoing, 3-Part, Multi-Center, Randomized, Double-Blind, Placebo-Controlled Phase 2 Clinical Study of Bezuclastinib in Adult Patients with NonAdvanced Systemic Mastocytosis (NonAdvSM)

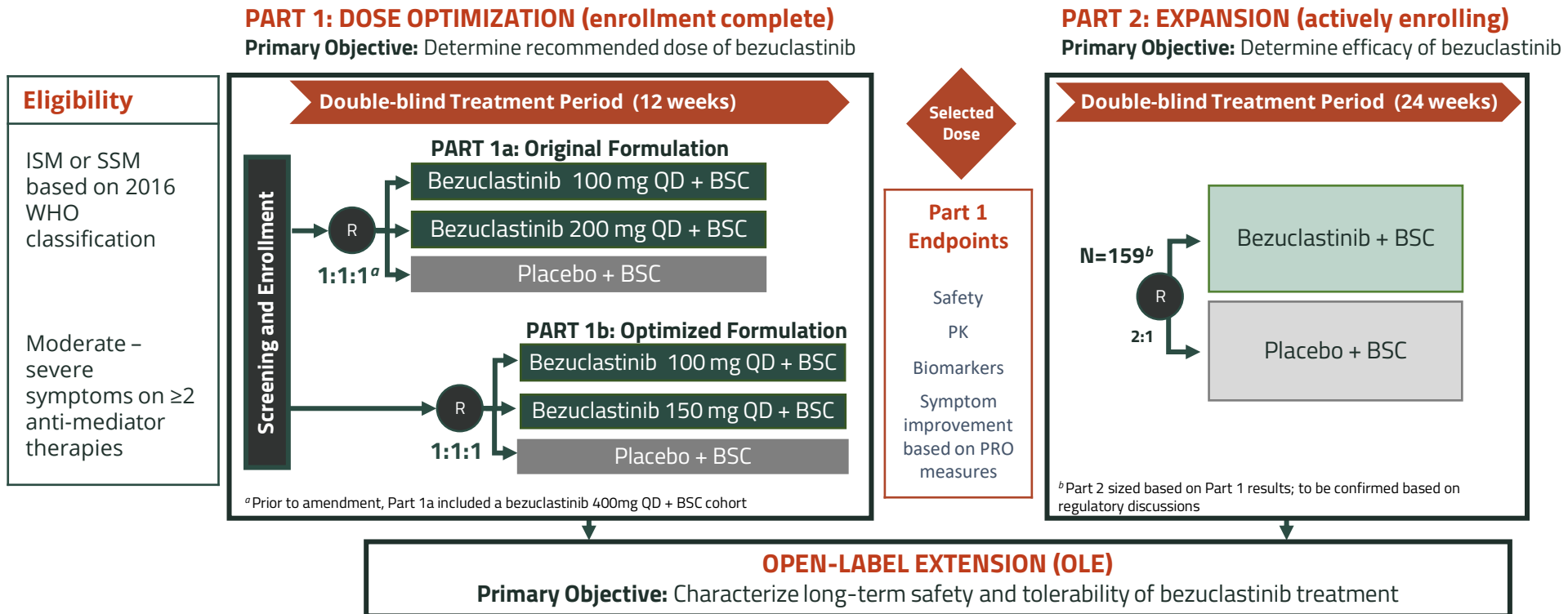
Brian D. Modena¹, Lindsay A. M. Rein², Stephen T. Oh³, Daniel J. DeAngelo⁴, Anthony M. Hunter⁵, Cem Akin⁶, Mariana Castells⁷, Michael Manning⁸, Richard Herrscher⁹, Celalettin Ustun¹⁰, Arnold Kirshenbaum¹¹, Cristina Bulai Livideanu¹², Nathan A. Boggs¹³, Cecilia Arana Yi¹⁴, Frank Siebenhaar^{15,16}, Tracy I. George¹⁷, Jay Patel¹⁷, Lei Sun¹⁸, Benjamin Exter¹⁸, Jenna Zhang¹⁸, Amanda Pilla¹⁸, Hina A. Jolin¹⁸, Rachael Easton¹⁸, Prithviraj Bose¹⁹

1. Modena Allergy & Asthma, San Diego, CA, USA; 2. Duke University, Durham, NC, USA; 3. Washington University School of Medicine, St. Louis, Missouri, USA; 4. Dana-Farber, Boston, MA, USA; 5. Emory University School of Medicine, Atlanta, GA, USA; 6. University of Michigan, Ann Arbor, MI, USA; 7. Brigham and Women's Hospital, Harvard Medical School, Boston, MA, USA; 8. Allergy, Asthma, & Immunology Associates, Scottsdale, AZ, USA; 9. AirCare, Plano, TX, USA; 10. Rush University Medical Center, Chicago, IL; 11. Allervie Clinical Research, Glenn Dale, MD; 12. CEREMAST Toulouse, Dermatology Department, Toulouse University Hospital, Toulouse, France; 13. Walter Reed National Military Medical Center, Bethesda, MD; 14. Mayo Clinic Arizona, Phoenix, AZ; 15. Institute of Allergology, Charité - Universitätsmedizin Berlin, Berlin, Germany; 16. Fraunhofer Institute for Translational Medicine and Pharmacology ITMP, Immunology and Allergology IA, Berlin, Germany. 17. University of Utah, ARUP Laboratories, Salt Lake City, UT, USA; 18. Cogent Biosciences Inc., Waltham, MA, USA; 19. MD Anderson Cancer Center, Houston, Texas, USA



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SUMMIT: Phase 2 Clinical Study Evaluating Bezuclostinib in NonAdvSM



Summit Part 1 Enrolled NonAdvSM Patients with Moderate to Severe Disease

Patient Demographics, Characteristics, and Disposition

Patient Demographics	Part 1a (N=20)	Part 1b (N=34)
Female, n (%)	15 (75)	21 (61.8)
Median Age in years, n (range)	50.5 (38 – 75)	52.0 (27-76)
ECOG PS, n (%)		
0	3 (15)	16 (47.1)
1	15 (75)	17 (50.0)
2	2 (10)	1 (2.9)
Clinical Characteristics	Part 1a (N=20)	Part 1b (N=34)
NonAdv Subtype per PI, n (%)		
Indolent SM (ISM)	18 (90)	33 (97)
Smoldering SM (SSM)	2 (10)	1 (3)
Median (range) MAS Total Score at Eligibility	45.56 (26.3 – 71.6)	43.44 (28.6 – 65.4)
Mast Cell Burden	Part 1a (N=20)	Part 1b (N=34)
KITD816V in Whole Blood, Positive, n (%)	15 (75)	28 (82.4)
Median KIT D816V VAF, % (range)	0.49 (BLD – 32.48)	0.085 (BLD - 19.58)
Median Bone Marrow MC Burden, % (range)	22.5 (1 – 80)	15 (2 – 50)
Median Serum Tryptase, ng/mL (range)	74.35 (10.2- 592.0)	37.15 (9.2 - 206.0)
<20 ng/mL, n (%)	3 (15)	7 (20.6)
≥20 ng/mL, n (%)	17 (85)	27 (79.4)

SM Therapy	Part 1a (N=20)	Part 1b (N=34)
Prior avapritinib, n (%)	1 (5.0)	1 (2.9)
Baseline Supportive Care Medications, Median (range)	3 (2-7)	2.5 (2 – 9)
H1 blockers, n (%)	19 (95)	30 (88.2)
H2 blockers, n (%)	18 (90)	27 (79.4)
Leukotriene receptor antagonists, n (%)	8 (40)	14 (41.2)
Proton pump inhibitors, n (%)	7 (35)	9 (26.5)
Cromolyn sodium, n (%)	4 (20)	3 (8.8)
Omalizumab, n (%)	3 (15)	1 (2.9)
Corticosteroids, n (%)	1 (5)	1 (2.9)
Patient Disposition	Part 1a (n=20)	Part 1b (N=34)
Months on Study (Part 1 + OLE), median (range)	7.03 (2.8 – 16.0)	4.09 (2.7-6.6)
Completed Part 1 (a or b), n (%)	20 (100)	34 (100)
On Study as of Data Cut-off, n (%)	18 (90)	33 (97.1)
Discontinued study, n (%)	2 (10)	1 (2.9)
AE, n (%)	1 (5)	1 (2.9)
Patient Decision, n (%)	1 (5)	0

Bezuclastinib 100 mg QD Optimized Formulation Selected as Summit Part 2 Dose Based on Part 1 Safety, PK, Biomarker and Efficacy Results



Encouraging Safety and Tolerability Profile for Bezuclastinib 100 mg Dose in Part 1b

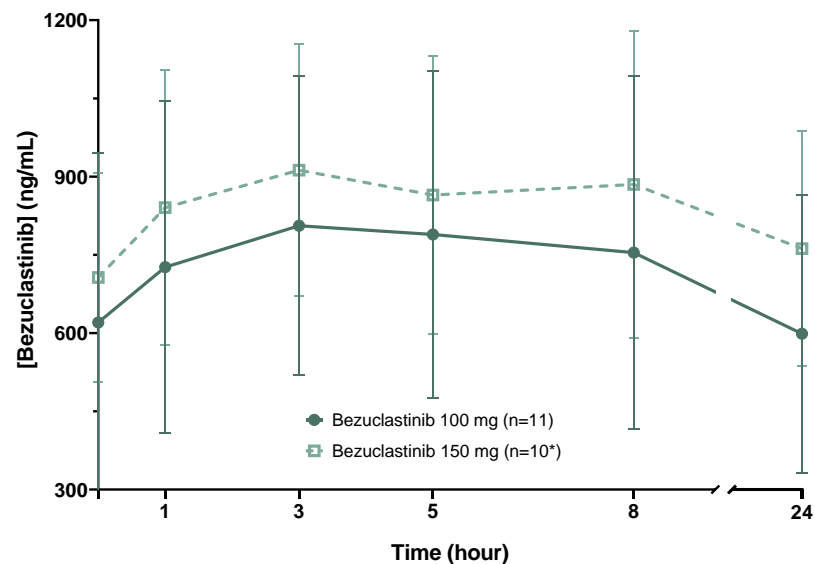
- The majority of TEAEs were low grade and reversible without dose modification
- No bleeding or cognitive impairment events reported across bezuclastinib cohorts
- No dose reductions at 100mg cohort; two dose reductions at 150mg: Gr1 ALT, Gr2 abdominal pain
- Only one SAE reported in bezuclastinib cohorts (150mg patient experienced ALT/AST increase that led to discontinuation)

All TEAEs Occurring >1 Patient in Any Cohort in Part 1b

Preferred Term	Placebo (n=12)		Bezuclastinib			
			100mg QD (n=11)		150mg QD (n=11)	
	Gr 1/2	Gr 3+	Gr 1/2	Gr 3+	Gr 1/2	Gr 3+
Hair color changes	-	-	3	-	7	-
Diarrhea	2	-	2	-	2	-
Nausea	3	-	3	-	1	-
Taste disorder[#]	-	-	1	-	2	-
Dizziness	2	-	-	-	2	-
Fatigue	1	-	-	-	2	-
Noncardiac chest pain	1	-	-	-	2	-
ALT/AST increased[#]	1	-	-	-	1	1*
Neutropenia[#]	-	-	-	-	1	1*
COVID-19	3	-	1	-	-	-
Insomnia	2	-	-	-	-	-
Decreased appetite	2	-	-	-	-	-
Vomiting	2	-	-	-	-	-
Urticaria	2	-	-	-	-	-
Palpitations	2	-	-	-	-	-

Bezuclastinib Demonstrated Dose Dependent Increase in Mean Steady State Exposure

Summit Part 1b: Mean (\pm SD) Concentration on C2D1 (Steady State)



Comparable Exposures for Low and High Dose Across Part 1a and 1b

	Dose (mg), Study Part	N	Mean S.S. AUC _{0-24h} (ng.h/mL)
Low Dose	100, 1a	7	16900
	100, 1b	11	16900
High Dose	200, 1a	5	19200
	150, 1b	10*	19700

Bezuclastinib Elicited Deep Reductions Across Markers of Mast Cell Burden Within 12 Weeks

Serum Tryptase

- Of patients with baseline tryptase $\geq 20\text{ng/mL}$, nearly all patients treated with bezuclastinib achieved $< 20\text{ng/mL}$ (100% on 100 mg, 89% on 150 mg, 0% on placebo)
 - Overall, mean time to tryptase $< 20\text{ng/mL}$ was 4.5 weeks for patients treated with bezuclastinib
- Of patients with baseline tryptase $\geq 11.4\text{ng/mL}$: 70% on 100mg, 90% on 150mg and 0% on placebo achieved $< 11.4\text{ng/mL}$

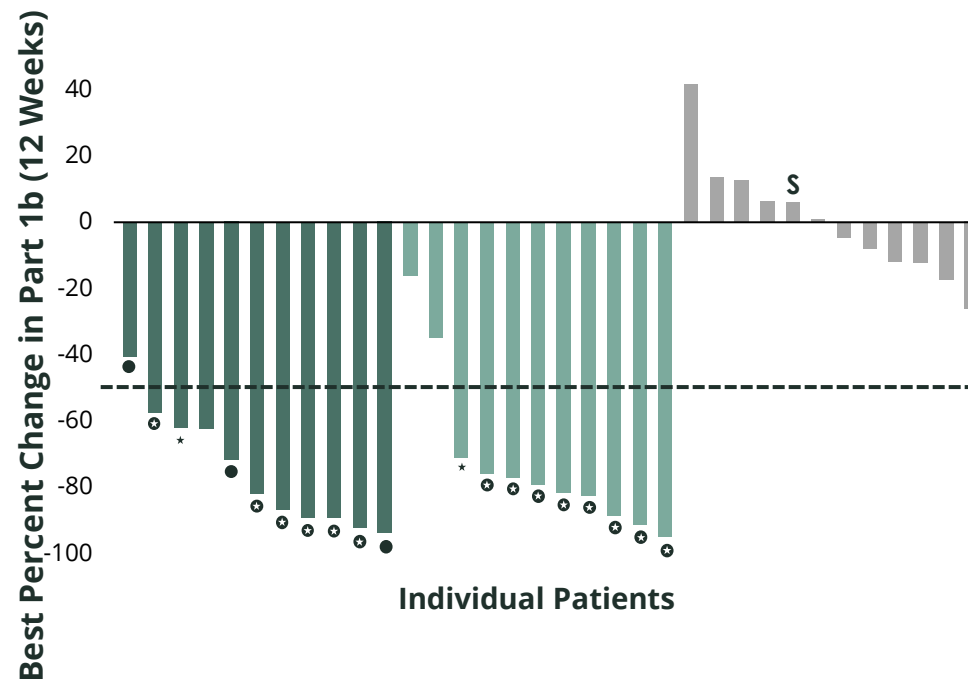
KIT D816V VAF

- Among patients with detectable KIT D816V at baseline: 100% on 100mg, 89% on 150mg and 0% on placebo achieved at least 50% reduction or undetectable KIT D816V at Week 12

Bone Marrow Mast Cells (BM MC)

- Among patients with evaluable BM: 86% on 100mg, 78% on 150mg and 40% on placebo achieved $\geq 50\%$ reduction in BM MC at Week 12
 - Mean % change from baseline in BM MC at Week 12 for patients treated with bezuclastinib 100mg was -70% vs -30% on placebo

Serum Tryptase (n=34)



■ Bezuclastinib 100 mg
■ Bezuclastinib 150 mg
■ Placebo
s Smoldering SM

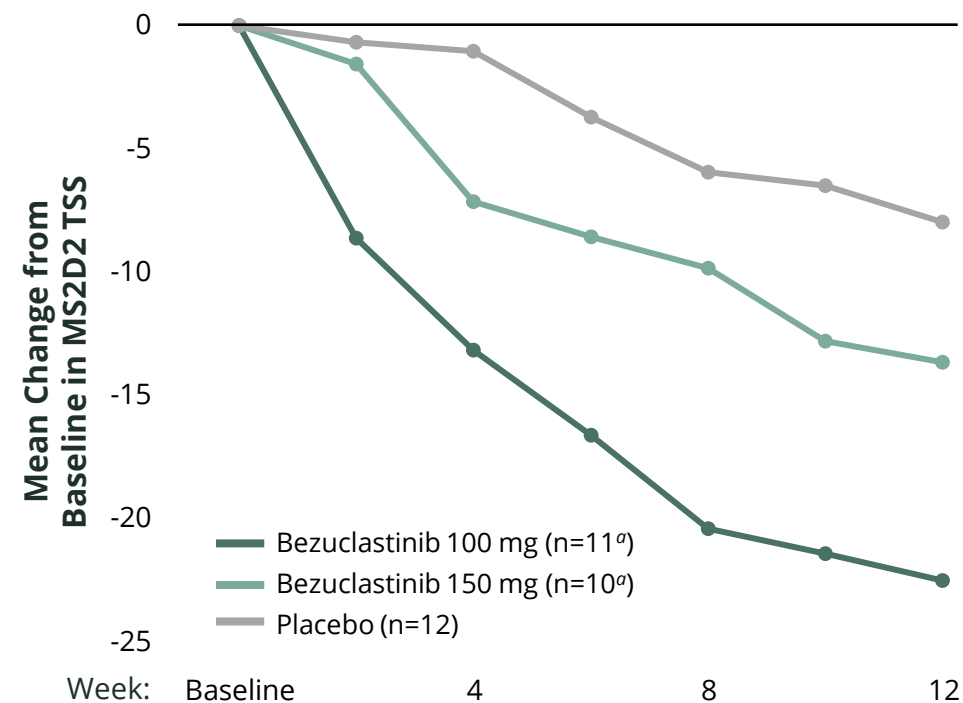
Serum Tryptase Outcomes

● Achieved $< 20\text{ng/mL}^{\mu}$
★ Achieved $< 11.4\text{ng/mL}^{\mu}$
⊗ Achieved both^μ

Patients Treated With Bezuclastinib 100 mg Reported Rapid and Significant Improvement in Symptom Severity

- 51% mean improvement in overall symptom severity (MS2D2 TSS) from baseline at Week 12 for patients receiving 100 mg bezuclastinib vs. 18% improvement for placebo patients
- Patients treated with 100 mg bezuclastinib reported a significant reduction in total symptom severity vs. placebo at Week 12 (-23.78 vs. -9.03; $p=0.0003$)
- 70% of patients treated with 100 mg bezuclastinib achieved $\geq 50\%$ reduction in MS2D2 TSS at Week 12 vs. 8% placebo patients

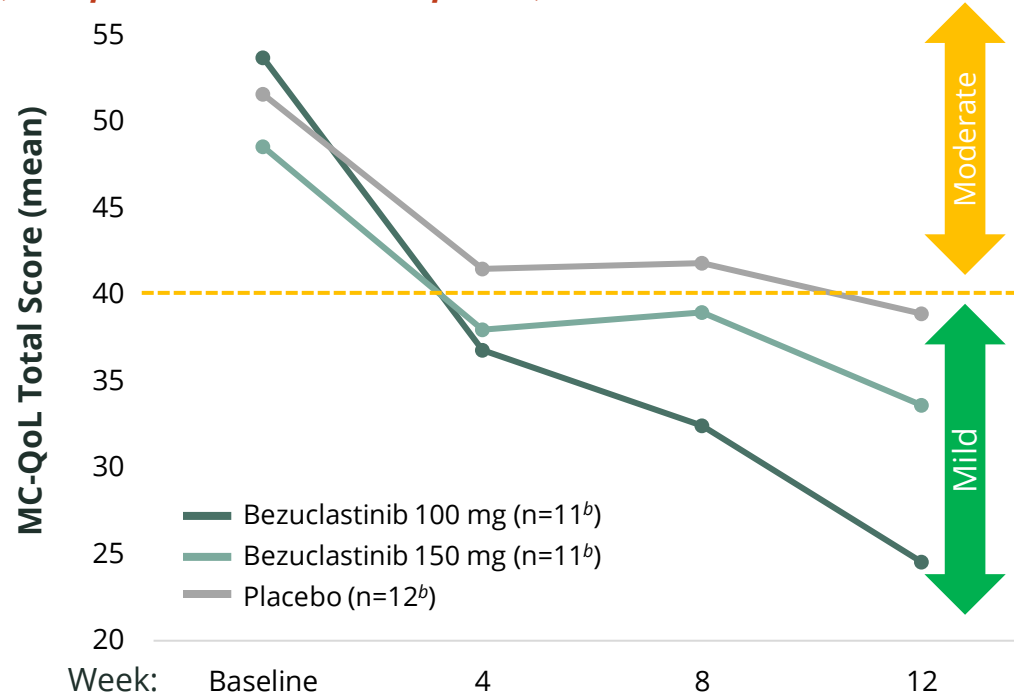
Symptom Severity Measured by MS2D2



Patients Treated With Bezuclastinib 100 mg Reported Rapid and Significant Improvement in Quality of Life

- 49% mean improvement in quality of life (MC-QoL) from baseline at Week 12 in patients treated with 100 mg bezuclastinib vs 24% for placebo
- Patients reported a significant improvement in quality of life after 12 weeks of bezuclastinib 100mg QD compared to placebo (-24.86 vs. -12.39, $p=0.046$)

Quality-of-Life Measured by MC-QoL^a



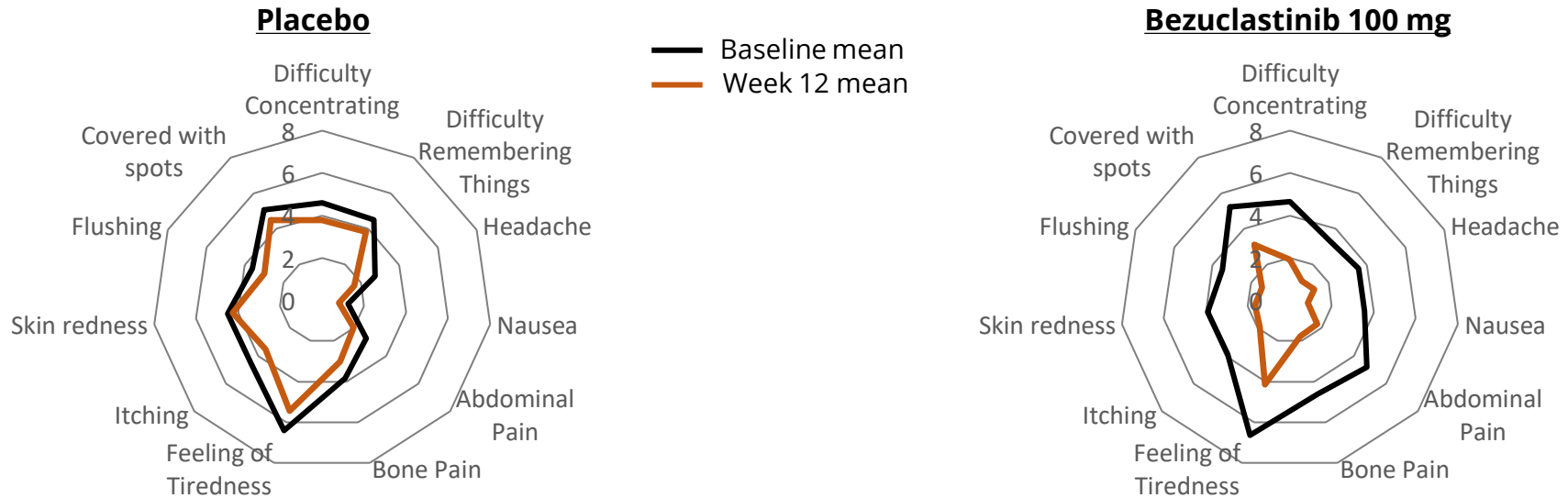
^aMC-QoL is a disease-specific HRQoL questionnaire with 27 items in 4 domains. Total score is linearly transformed to a 0 to 100 scale.¹⁰

^bData are unavailable for 2 patients at selected timepoints

10. Siebenhaar F, Sander B, Tram H, Ellrich A, Maurer M, Weller K. Development and validation of the mastocytosis activity score. Allergy. 2018;00:1-8.

Bezuclastinib 100mg Demonstrated Improvement Compared to Placebo Across Symptoms of SM

Greater Improvement Observed in the MS2D2 TSS With 12 Weeks of Bezuclastinib 100 mg vs Placebo



Health-Related QoL Across All MC-QoL^a Domains Improved With 12 Weeks of Bezuclastinib 100mg vs Placebo

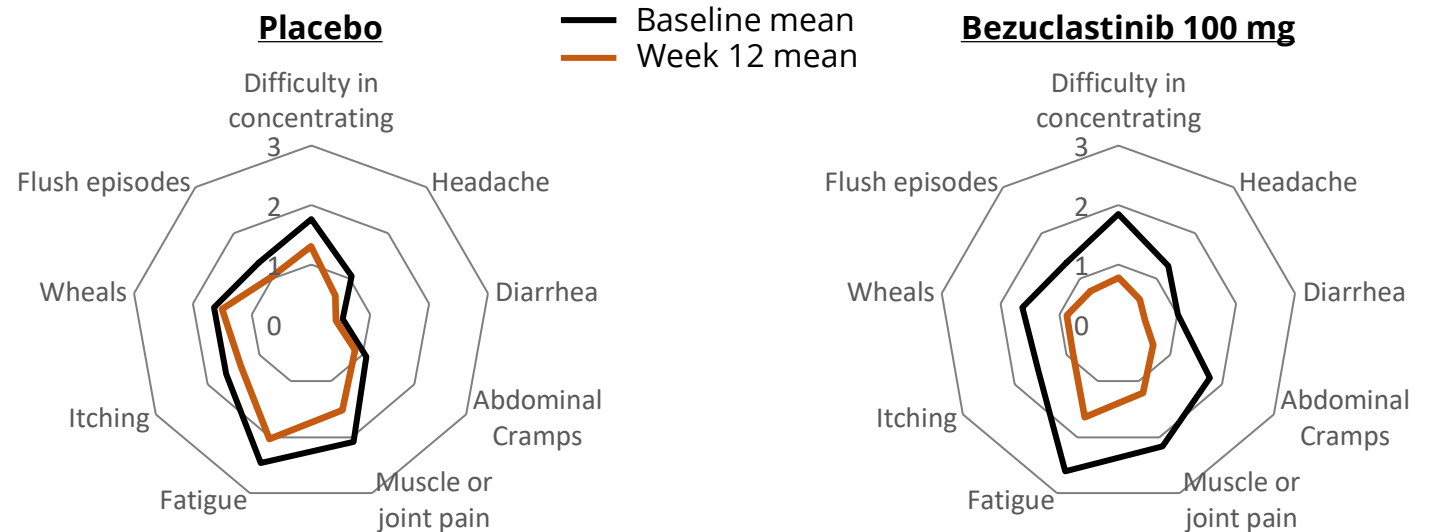


^aMC-QoL is a disease-specific HRQoL questionnaire with 27 items in 4 domains. Total score is linearly transformed to a 0 to 100 scale.⁹
9. Siebenhaar et al. Allergy 2016 71(6):869-77.

Bezuclastinib 100 mg Demonstrated Improvement Compared to Placebo Across Symptoms of SM

Bezuclastinib 100 mg Improved Symptom Severity, As Measured by the Mastocytosis Activity Score^a (MAS), Compared to Placebo

- 41% mean improvement from baseline in MAS at Week 12 for patients receiving 100 mg bezuclastinib vs. 21% improvement for placebo
- 50% of patients treated with 100 mg bezuclastinib achieved $\geq 50\%$ improvement in MAS at week 12 vs. 0% placebo patients



Conclusions: Totality of Results from Summit Part 1 Support 100 mg QD as the Optimal Dose of Bezuclastinib for Patients With NonAdvSM

In Part 1b, bezuclastinib 100mg QD resulted in:

- Safety and tolerability profile generally consistent with placebo results
 - No bleeding, cognitive impairment, or edema AEs reported
 - No dose reductions or discontinuations due to AEs
- Rapid reductions across markers of mast cell burden, supported by KIT D816V mechanism and exposure evidence from nonclinical studies
- Significant improvement versus placebo at 12 weeks in both symptom severity and quality of life, based on mean change from baseline in MS2D2 and MC-QoL total scores which corresponds to:
 - 51% reduction in symptom severity (measured by MS2D2)
 - 49% improvement in health-related quality-of-life (measured by MC-QoL)
- 70% of patients achieving $\geq 50\%$ improvement in symptom severity versus 8% on placebo, as measured by MS2D2

Summit Part 2 is expected to include 159 patients and is actively enrolling

A silhouette of a person standing on a rocky peak with their arms raised in a gesture of triumph or achievement. The background consists of a range of mountains under a dark, overcast sky. The overall color palette is dark, with shades of teal and grey.

Additional Thoughts on SUMMIT Results

Real Challenges. Real Solutions.

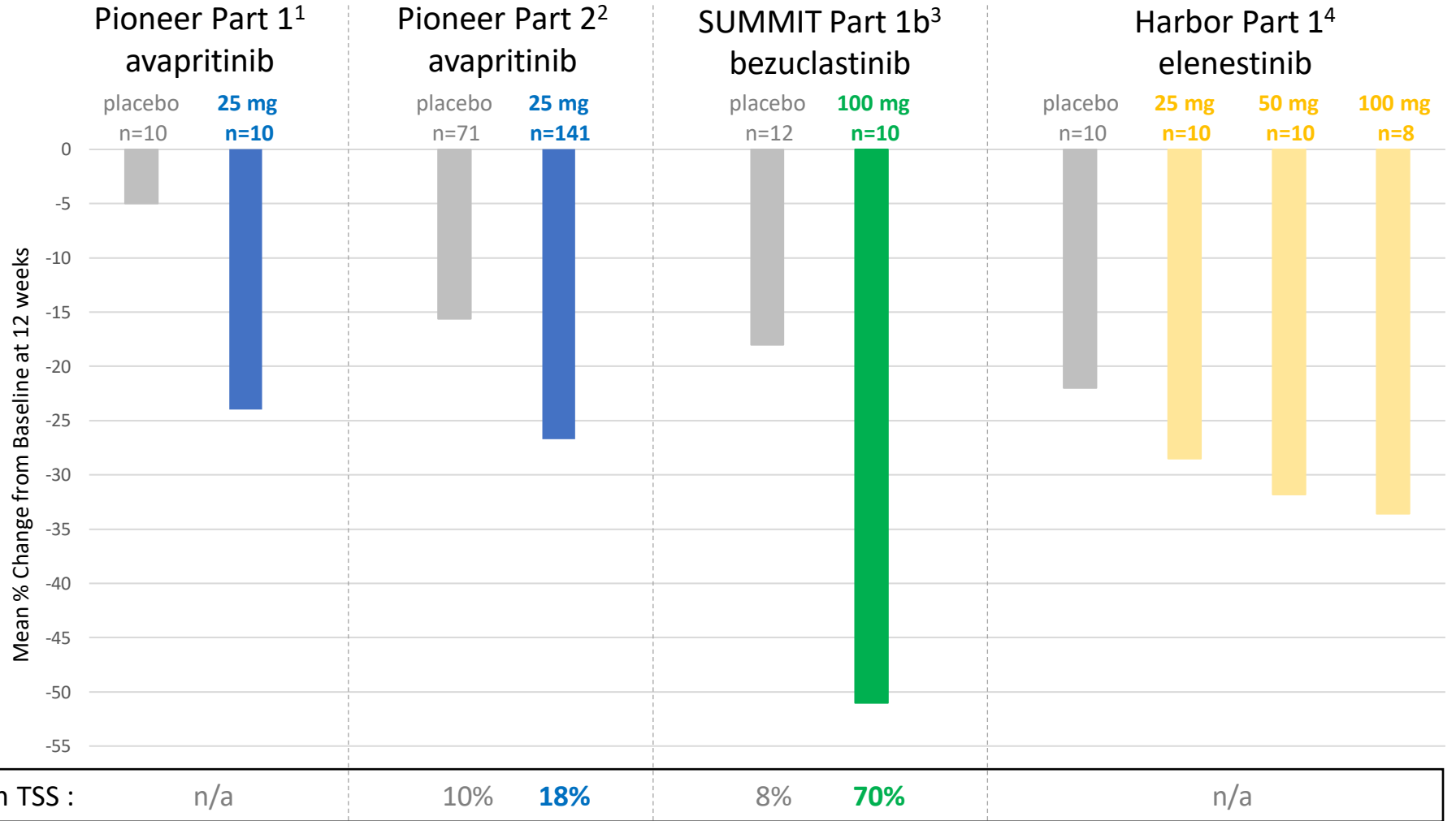
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NonAdvSM Clinical Results: Symptomatic Severity Reduction – Week 12 Means

- Results shown from RP2D for avapritinib and bezuclastinib; elenestininb RP2D yet to be announced

- Symptomatic severity assessed using ISM-SAF (avapritinib & elenestininb) and MS2D2 (bezuclastinib), each fit-for-purpose PROMs designed to measure NonAdvSM symptoms

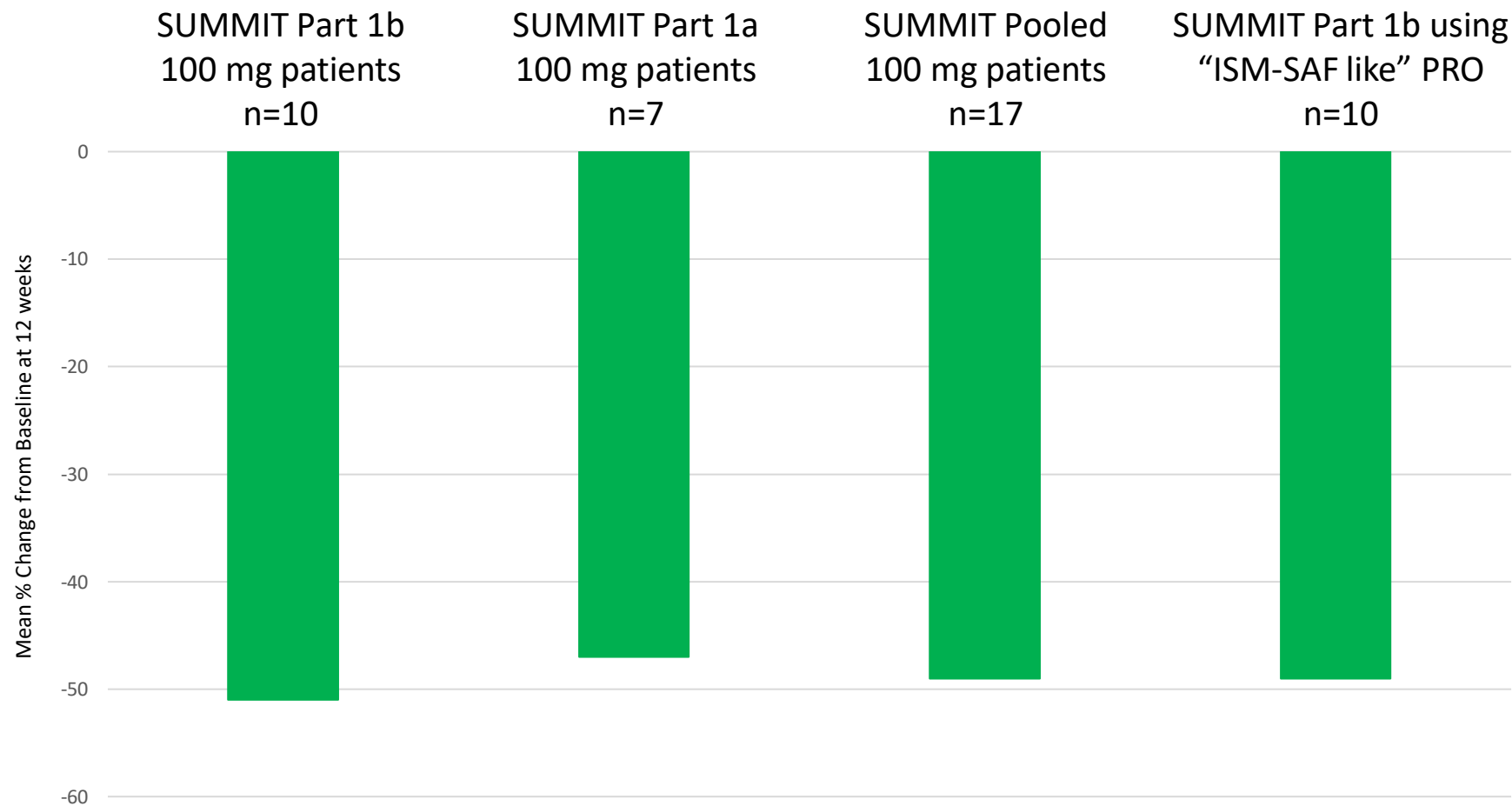
- Pioneer 2, SUMMIT and Harbor Part 1 conducted with patients blinded to laboratory values (eg. serum tryptase) – placebo effect reported appears consistent across studies



1 – ASH 2020 presentation, Blueprint Medicines; estimated from line graph presented; 2 – AAAAAI 2023 presentation, Blueprint Medicines, estimated from line graphs presented and imputed from mean absolute change from baseline using baseline severity; 3 – AAAAAI 2024 Presentation, Cogent Biosciences; 4 – ASH 2023 presentation, Blueprint Medicines

Consistent Magnitude of Symptomatic Improvement for Patients Receiving 100 mg Bezuclostinib

- 100 mg bezuclostinib patients reported very similar symptomatic improvement (week 12 mean change TSS) across SUMMIT 1a and 1b
- Constructing a scoring system using the same symptoms as ISM-SAF results in consistent week 12 mean change in TSS vs. MS2D2
- Improvements across domains in SUMMIT Part 1 support finding that magnitude of effect is not sensitive to item selection in TSS



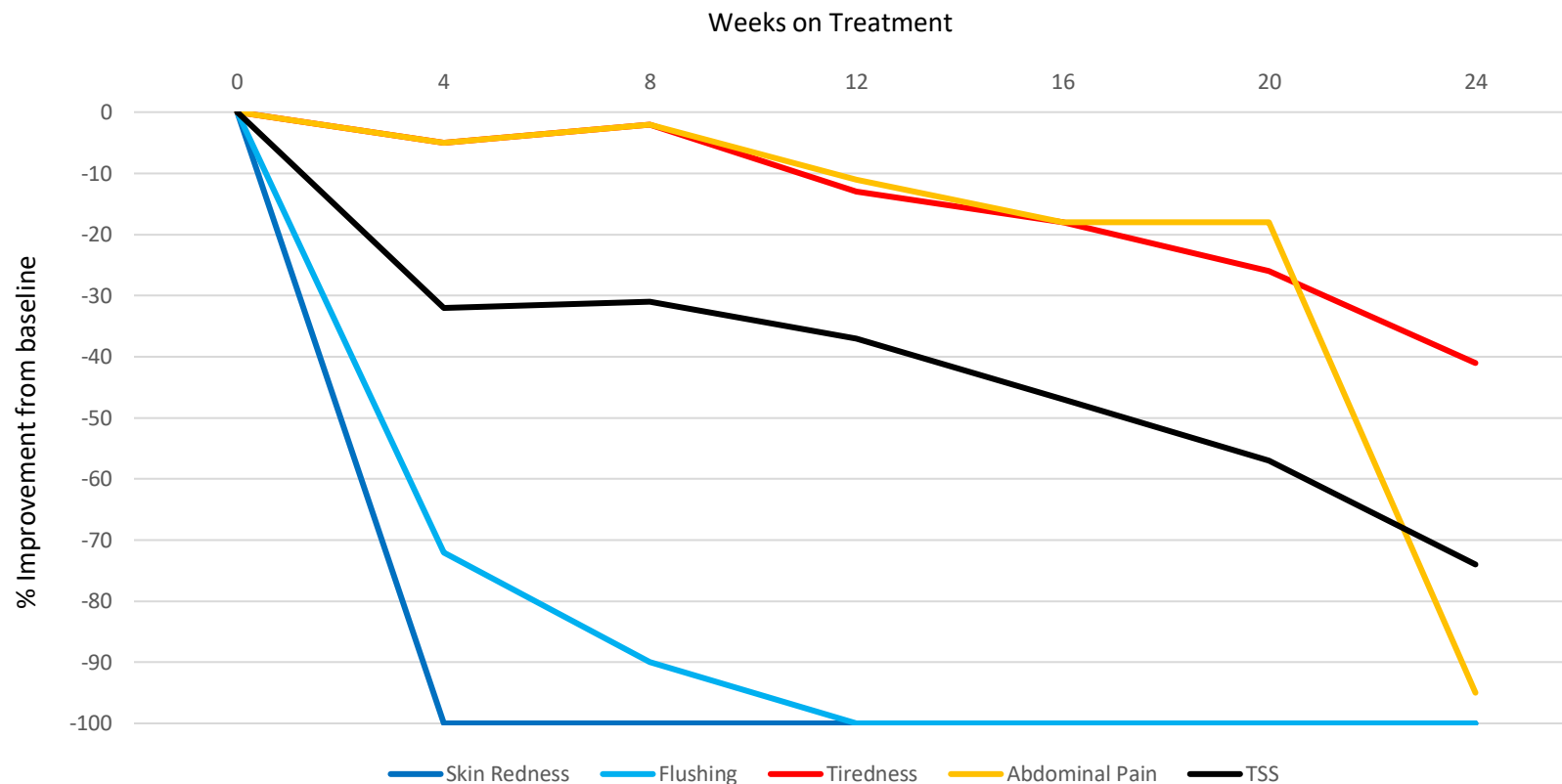
Optimizing Dose in NonAdvSM is Critical as Adverse Events May Confound TSS

- 52yr old patient receiving 150 mg bezuclastinib in Part 1b

- Serum tryptase reduced from 74.1 ng/ml baseline to 8.6 ng/ml at week 12

- Skin symptoms resolved quickly, but TSS at week 12 only -37% due to persistent tiredness and Gr 2 abdominal pain

- Dose reduced to 100 mg at week 20. Following dose reduction, rapid elimination of abdominal pain, improvement in tiredness and resulting TSS of -73% by week 24



A silhouette of a person standing on a mountain peak with their arms raised in a gesture of triumph or achievement. The background shows a range of mountains under a dark, overcast sky.

Cogent Summary

Real Challenges. Real Solutions.

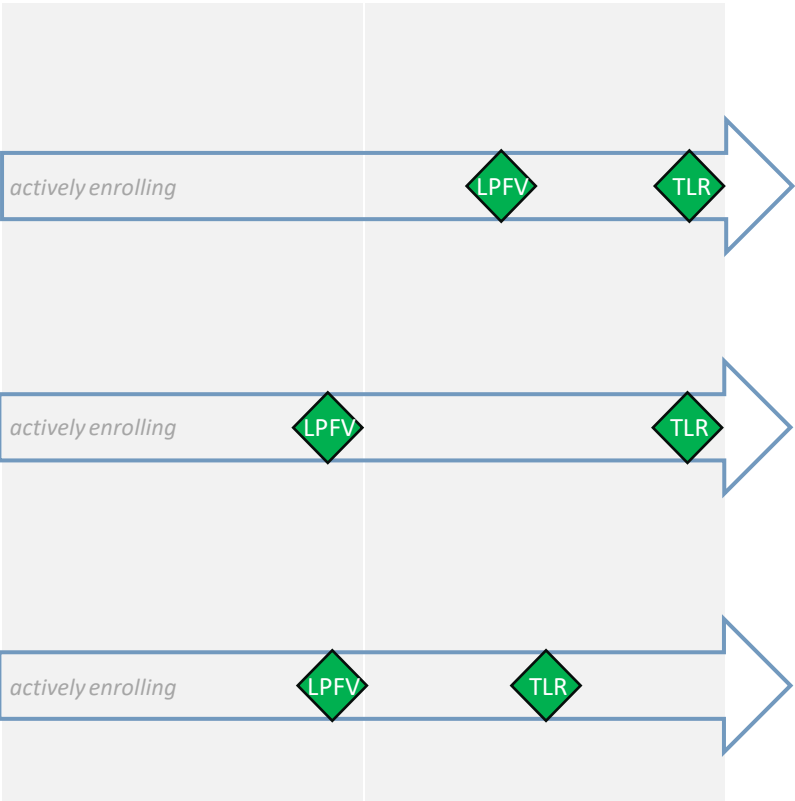
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Bezuclastinib Offers Best-in-Class KIT Inhibitor Opportunity



Registration-directed study in ISM
bezuclastinib vs. placebo
n=159, 24 week MS2D2 primary endpoint

← 2024 → ← 2025 →



\$1.5 billion US annual market opportunity; SUMMIT Part 1b results provide path to market leadership



Phase 3 study in 2nd-line GIST
bezuclastinib +/- sunitinib
n=388, mPFS primary endpoint

\$700 million US annual market opportunity, no competition for broad 2nd-line GIST population



Registration-directed study in ASM
bezuclastinib monotherapy
n=65, ORR primary endpoint

\$300 million US annual market opportunity; avapritinib safety/tolerability concerns provides path to market leadership

Recent \$225M fundraising provides cash runway into 2027, more than 12 months after results from all three registration trials expected; aggregate US annual sales opportunity \$2.5 billion with limited competition



LPFV: last patient, first visit signifies end of enrollment period
TLR: top-line results from primary endpoint of trial



Q&A

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Precision therapies for genetically defined diseases