Medical Treatment of Hypercortisolism with Relacorilant: Final Results of the Phase 3 GRACE Study

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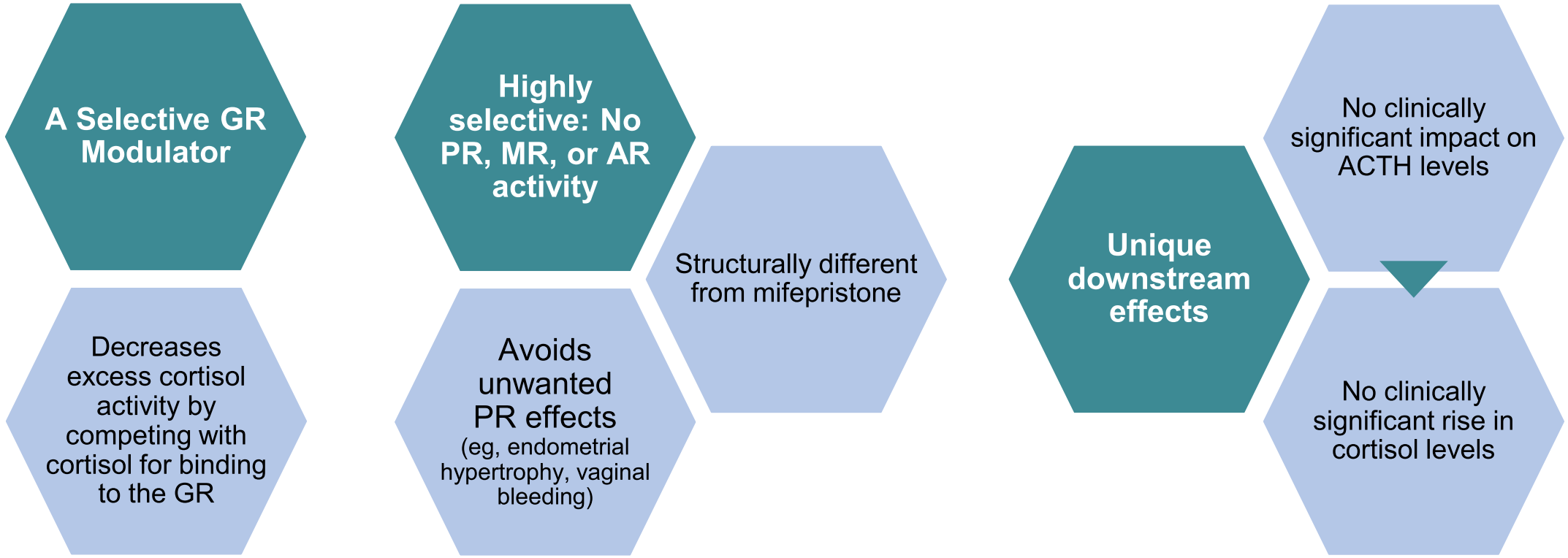
Relacorilant: In Development for the Treatment of Cushing Syndrome



ACTH, adrenocorticotropic hormone; AR, androgen receptor; GR, glucocorticoid receptor; MR, mineralocorticoid receptor; PR, progesterone receptor.

The GRACE Phase 3 Study

Patient population
- 18–80 years old
- Cushing syndrome
- Hypertension, hyperglycemia (impaired glucose tolerance or diabetes mellitus), or both

N=152

grace STUDY

Relacorilant

Dose titration: 100 mg QD to 400 mg QD based on tolerability and efficacy

Randomization (1:1)

Relacorilant

Placebo

Extension study *(optional)*

Primary endpoints
- Loss of hypertension control
- Safety

Eligibility for randomization

Patients who completed the open-label phase and met response criteria at visit OL22*:

Hypertension control (patients with hypertension)
- ≥5 mm Hg decrease in mean SBP and/or DBP, without worsening of either, based on 24-h ABPM

Hyperglycemia control

Patients with **impaired glucose tolerance**
- 2-h oGTT glucose normalization (<140 mg/dL)

Patients with **diabetes** (at least one of)
- HbA1c decrease by ≥0.5%,
- 2-h oGTT glucose normalization (<140 mg/dL) or decrease by ≥50 mg/dL, and/or
- Total daily insulin dose decrease by ≥25% and HbA1c unchanged or decreased

NCT03697109. ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; HbA1c, hemoglobin A1c; oGTT, oral glucose tolerance test; OL, open label; QD, every day; RW, randomized withdrawal; SBP, systolic blood pressure. *Patients with hypertension and hyperglycemia who do not meet the response criteria for both must meet the respective response criteria without worsening of the other comorbidity.

Open Label

Patient Demographics & Baseline Characteristics

Mean (SD)	Hypertension only (n=31)	Hyperglycemia only (n=50)	Hypertension & hyperglycemia (n=71)	Overall (N=152)
Age, yrs	43.5 (11.6)	54.1 (13.7)	50.9 (12.6)	50.4 (13.2)
Female, n (%)	24 (77.4)	42 (84.0)	61 (85.9)	127 (83.6)
Weight, kg	95.2 (25.5)	91.1 (21.4)	95.0 (26.6)	93.8 (24.7)
BMI, kg/m²	33.4 (7.5)	34.8 (7.9)	35.3 (9.6)	34.7 (8.6)
Waist circumference, cm	112.8 (17.4)	114.4 (14.7)	116.1 (20.4)	114.9 (18.0)
ACTH-dependent, n (%)	23 (74.2)	39 (78.0)	56 (78.9)	118 (77.6)
Plasma ACTH, pg/mL [n] 24-h UFC, µg/d [n]	67.7 (34.0) [23] 191.2 (221.8) [18]	74.9 (85.0) [39] 148.0 (136.3) [26]	78.1 (69.9) [56] 257.9 (407.1) [39]	74.9 (69.8) [118] 209.0 (308.3) [83]
ACTH-independent, n (%)	8 (25.8)	11 (22.0)	15 (21.1)	34 (22.4)
Plasma ACTH[a], pg/mL [n] 24-h UFC, µg/d [n]	7.3 (4.8) [8] 108.3 (88.9) [6]	20.0 (26.6) [11] 68.7 (67.9) [7]	10.0 (6.2) [15] 61.3 (30.5) [8]	12.7 (16.2) [34] 77.2 (64.0) [21]
Mean 24-h SBP (mm Hg) [n]	138.1 (9.4) [30]	124.6 (9.0) [47]	141.6 (11.0) [71]	135.5 (12.6) [148]
Mean 24-h DBP (mm Hg) [n]	90.8 (5.7) [30]	76.0 (7.3) [47]	88.1 (7.6) [71]	84.8 (9.4) [148]
HbA1c (%)	5.4 (0.5)	7.1 (1.6)	7.2 (1.6)	6.8 (1.6)

[a]Median ACTH was <5 pg/mL (hypertension only); 9 pg/mL (hyperglycemia only, hypertension and hyperglycemia, and overall). ACTH, adrenocorticotropic hormone; BMI, body mass index; DBP, diastolic blood pressure; OL, open-label phase; SBP, systolic blood pressure; SD, standard deviation; UFC, urinary free cortisol.

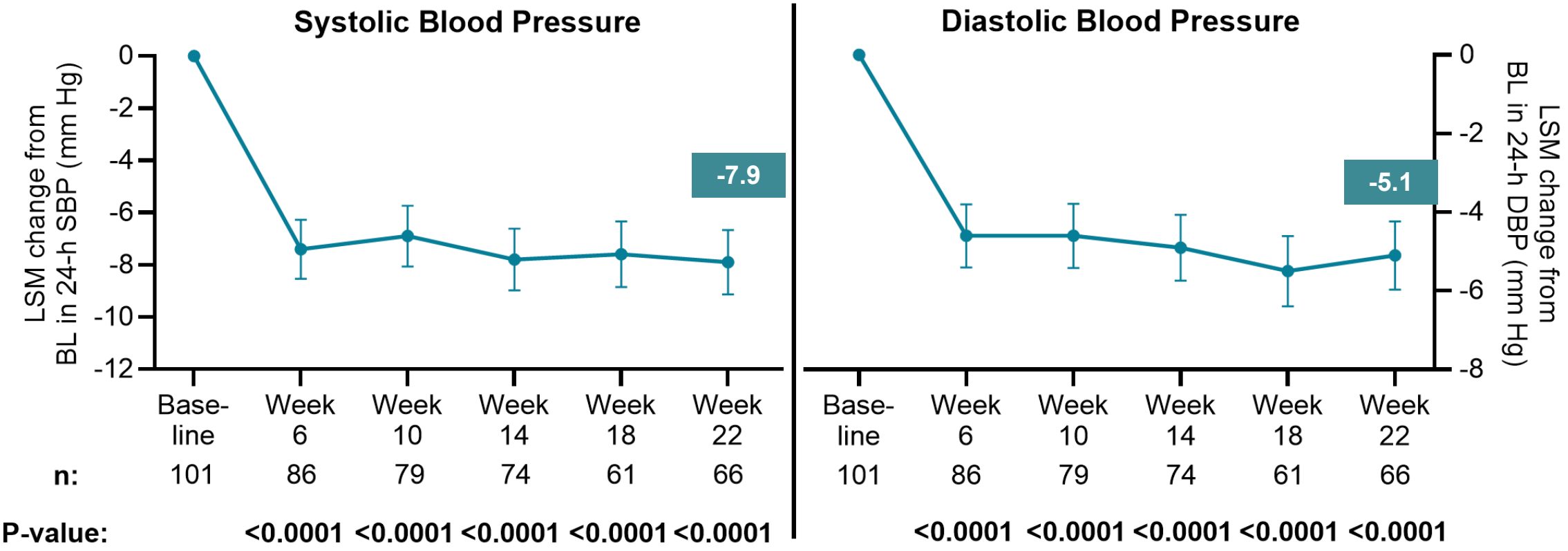
Open-label Results

HYPERTENSION

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Open Label

Patients with **hypertension**

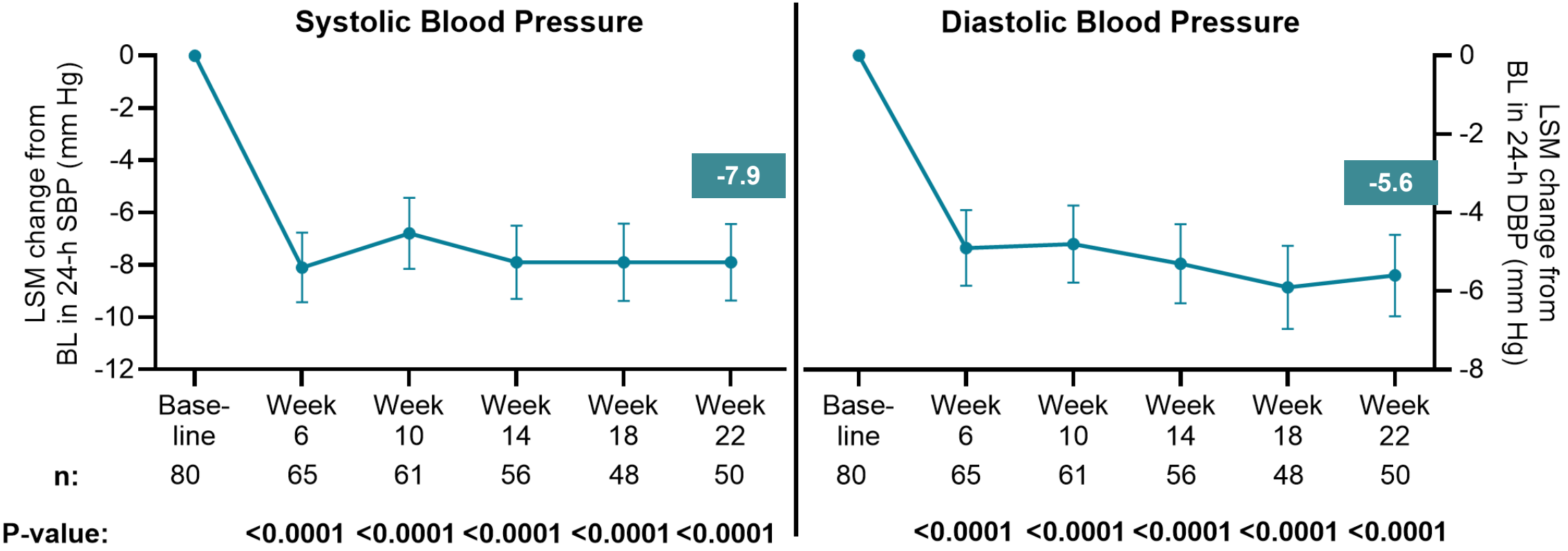


ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean; SBP, systolic blood pressure; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test p-values for the mean change from baseline shown.

Open Label

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Patients with hypertension who **took blood pressure medications**



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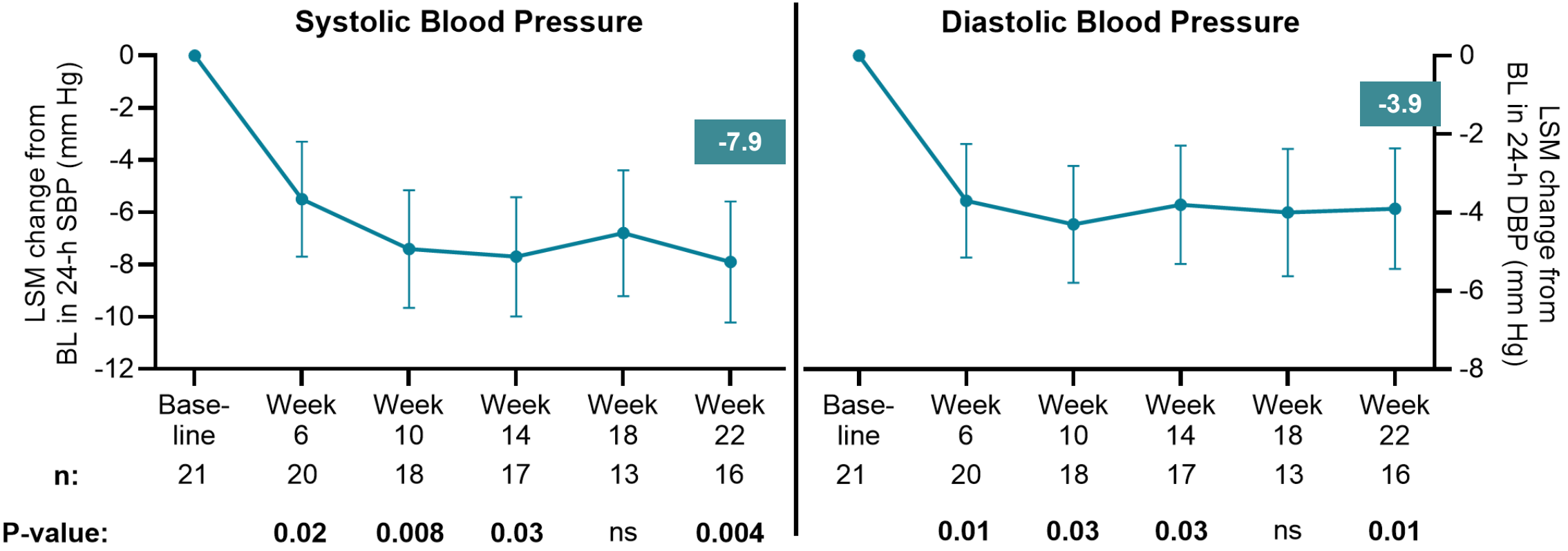
Open Label

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Patients with hypertension who **did not take blood pressure medications**

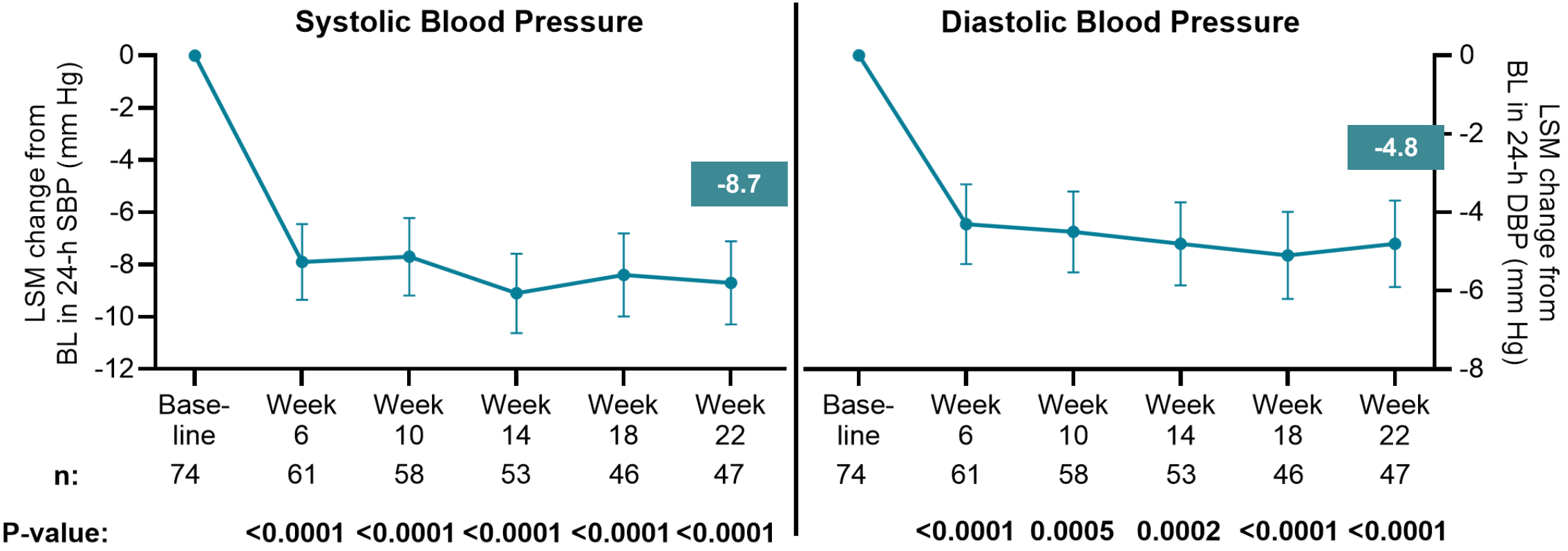


ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean; ns, not significant ($P \geq 0.05$); SBP, systolic blood pressure; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test p-values for the mean change from baseline shown.

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Open Label

Patients with **systolic hypertension**

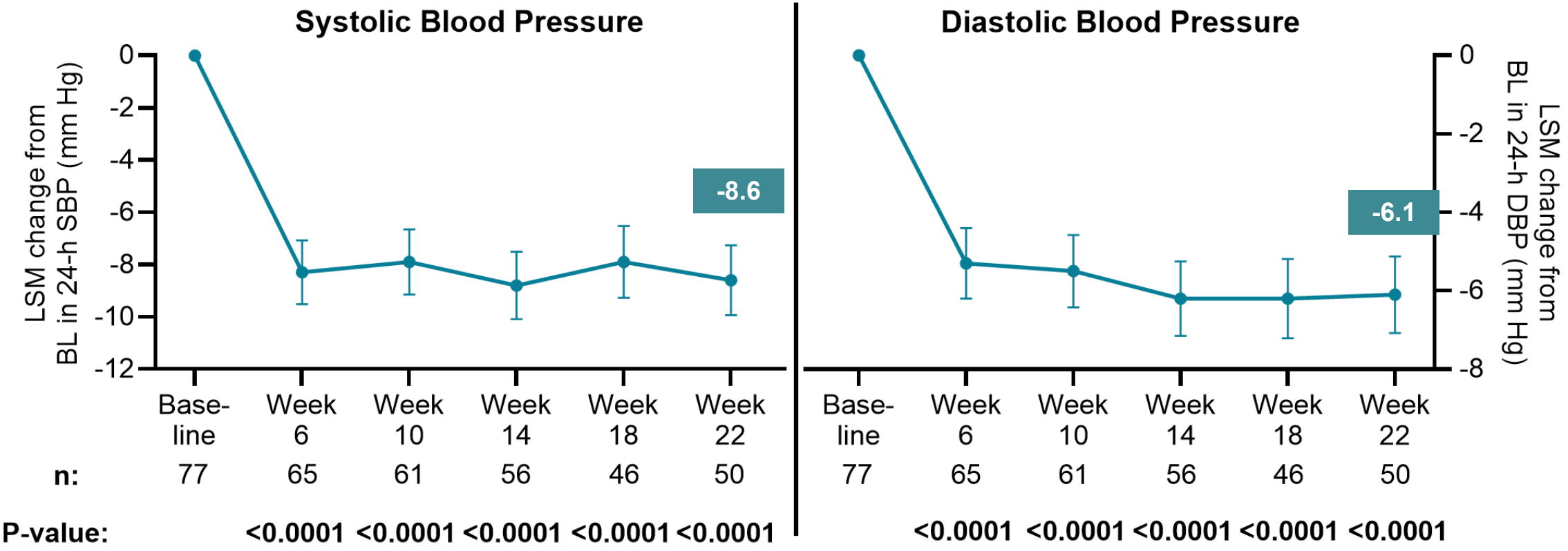


ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean. SBP, systolic blood pressure; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test p-values for the mean change from baseline shown.

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Open Label

Patients with **diastolic hypertension**

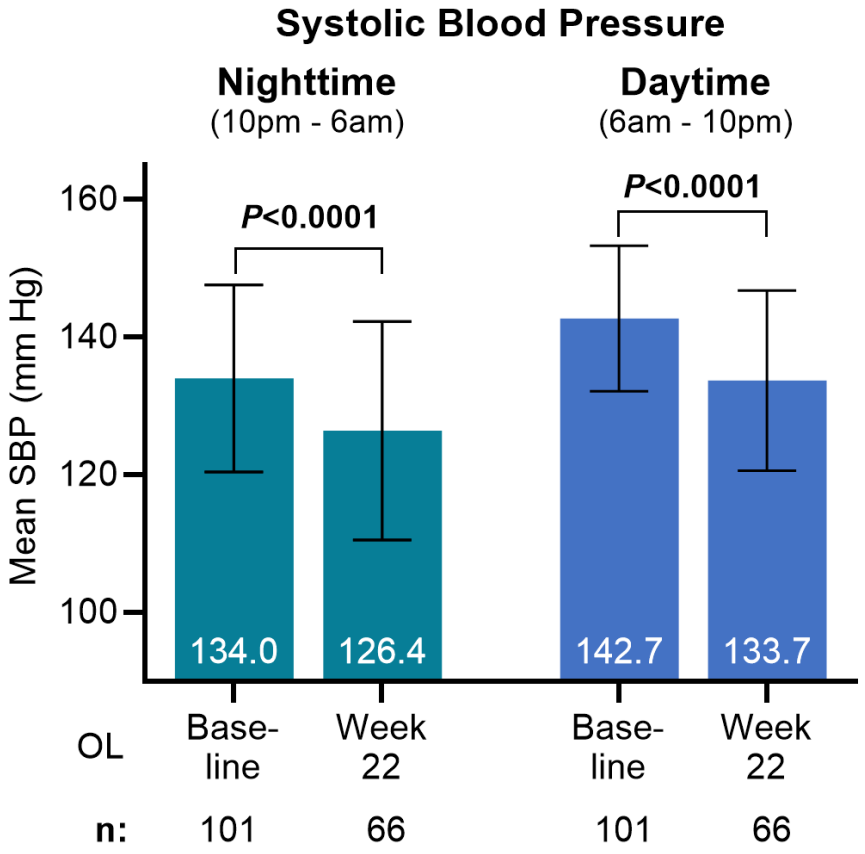


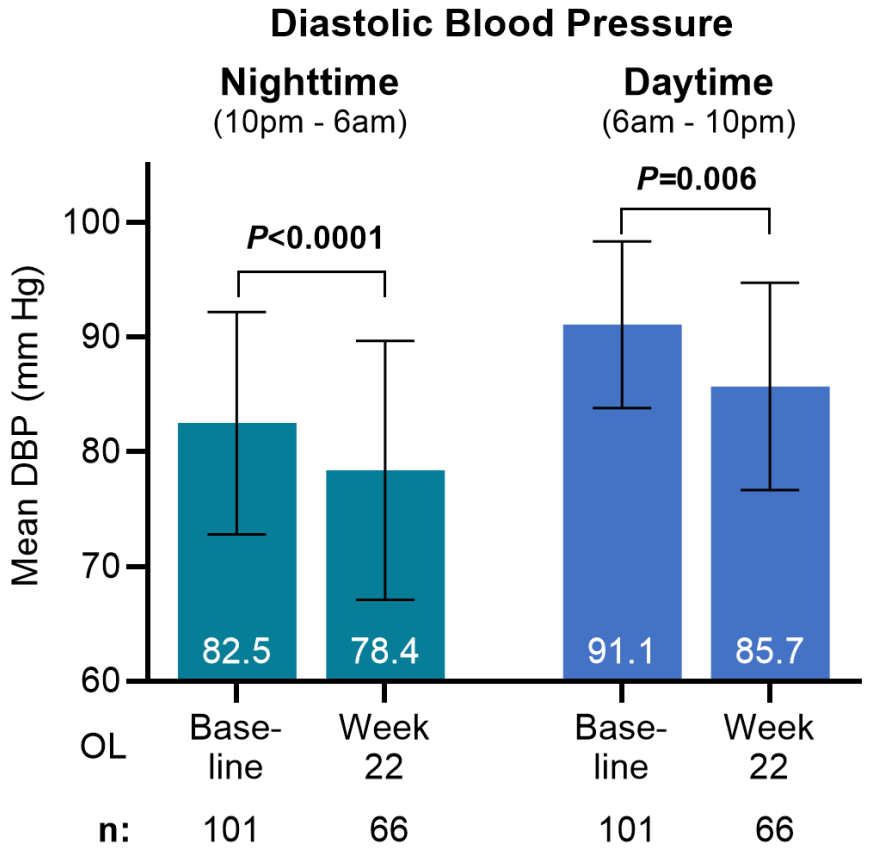
ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean; SBP, systolic blood pressure; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test p-values for the mean change from baseline shown.

Open Label

Improvements in Day- and Nighttime Blood Pressure by ABPM With Relacorilant

Patients with **hypertension**



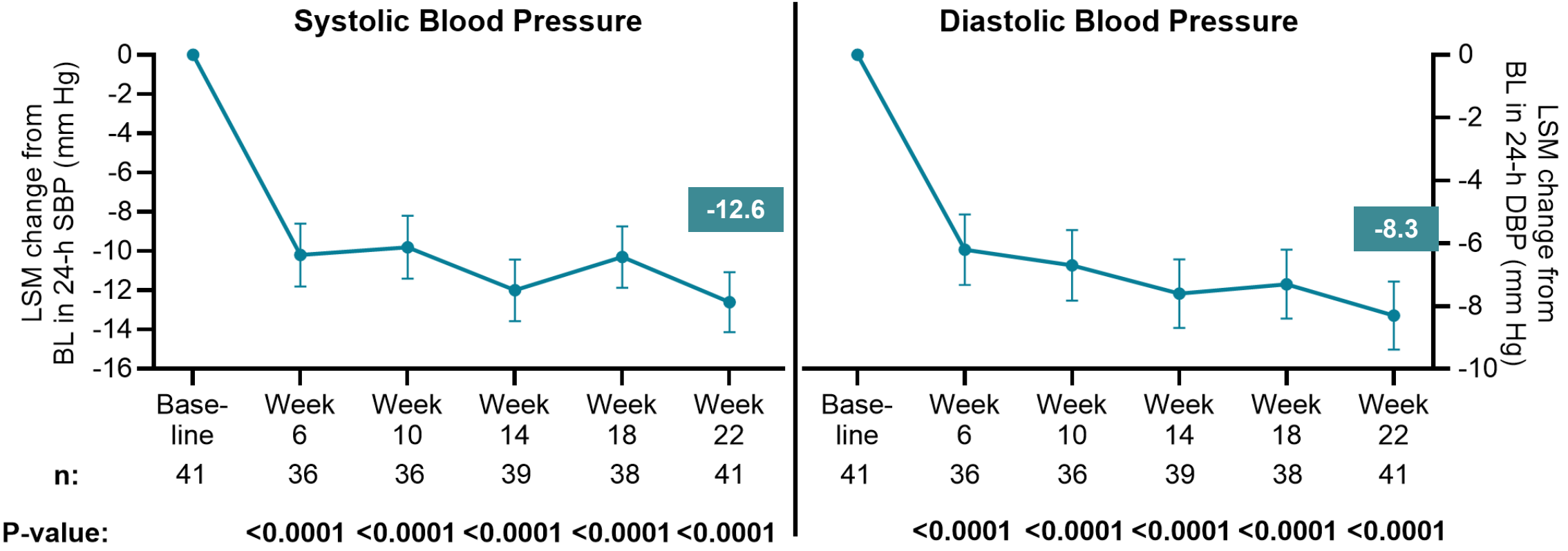


ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean; SBP, systolic blood pressure; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test p-values for the mean change from baseline shown.

Open Label

Rapid and Sustained Improvements in Blood Pressure by ABPM With Relacorilant

Patients with hypertension who **met hypertension response criteria & entered the RW phase**



ABPM, ambulatory blood pressure monitoring; BL, baseline; DBP, diastolic blood pressure; LSM, least squares mean; SBP, systolic blood pressure; SE, standard error.
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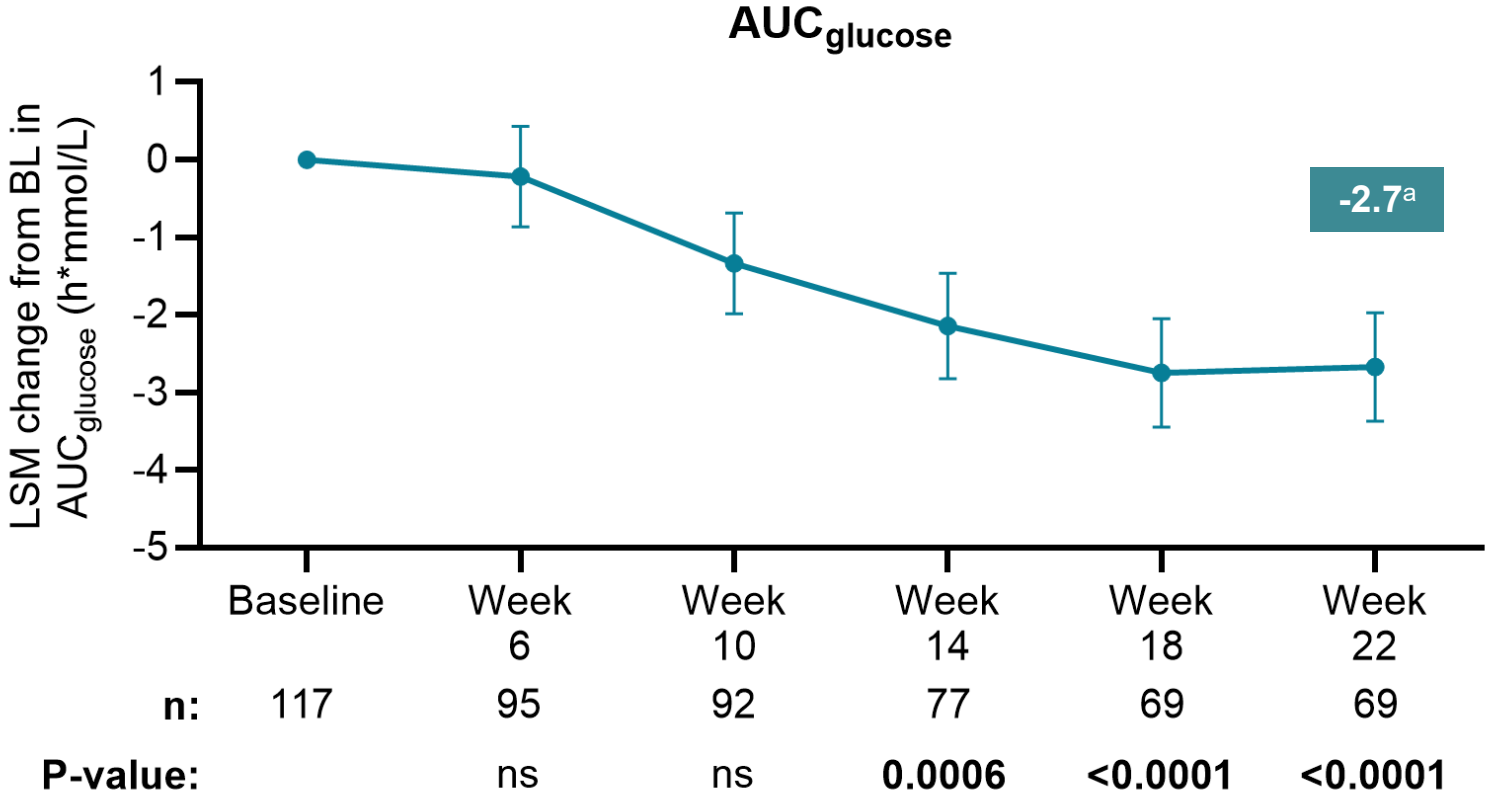
Open-label Results

HYPERGLYCEMIA

Rapid and Sustained Improvements in $AUC_{glucose}$ With Relacorilant

Open Label

Patients with **hyperglycemia (IGT or DM[b])**

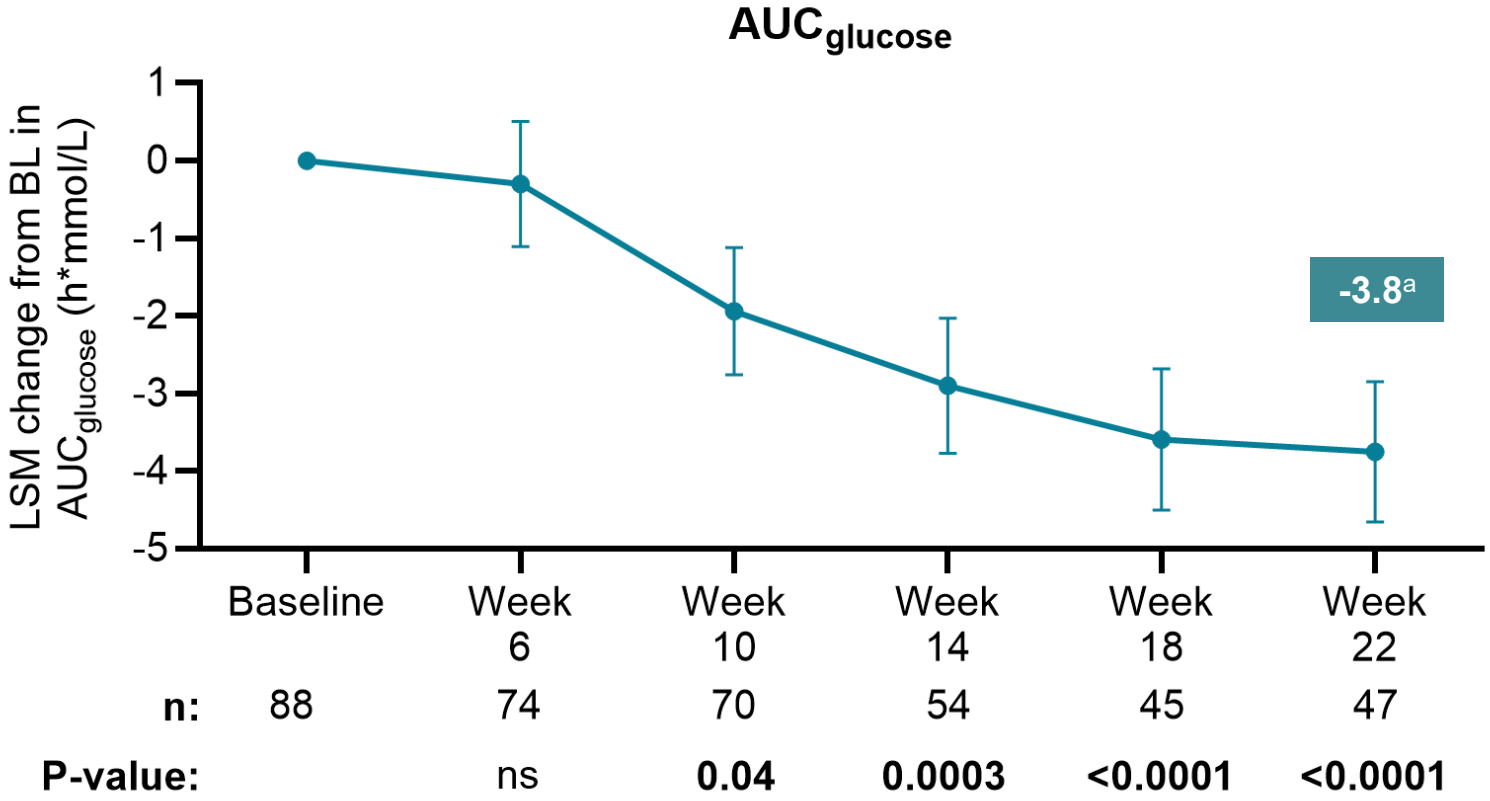


[a]Mean change from baseline to visit OL 22: -3.3 h*mmol/L. [b]Diabetes defined as fasting plasma glucose ≥126 mg/dL, 2-h oGTT plasma glucose ≥200 mg/dL, or HbA1c ≥6.5%.
$AUC_{glucose}$, glucose area under the curve; BL, baseline; DM, diabetes mellitus; hemoglobin A1c; HbA1c, hemoglobin A1c; IGT, impaired glucose tolerance; LSM, least squares mean; ns, not significant ($P \geq 0.05$); oGTT, oral glucose tolerance test; SE, standard error. Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

Rapid and Sustained Improvements in $AUC_{glucose}$ With Relacorilant

Open Label

Patients with **diabetes (DM)**[b]

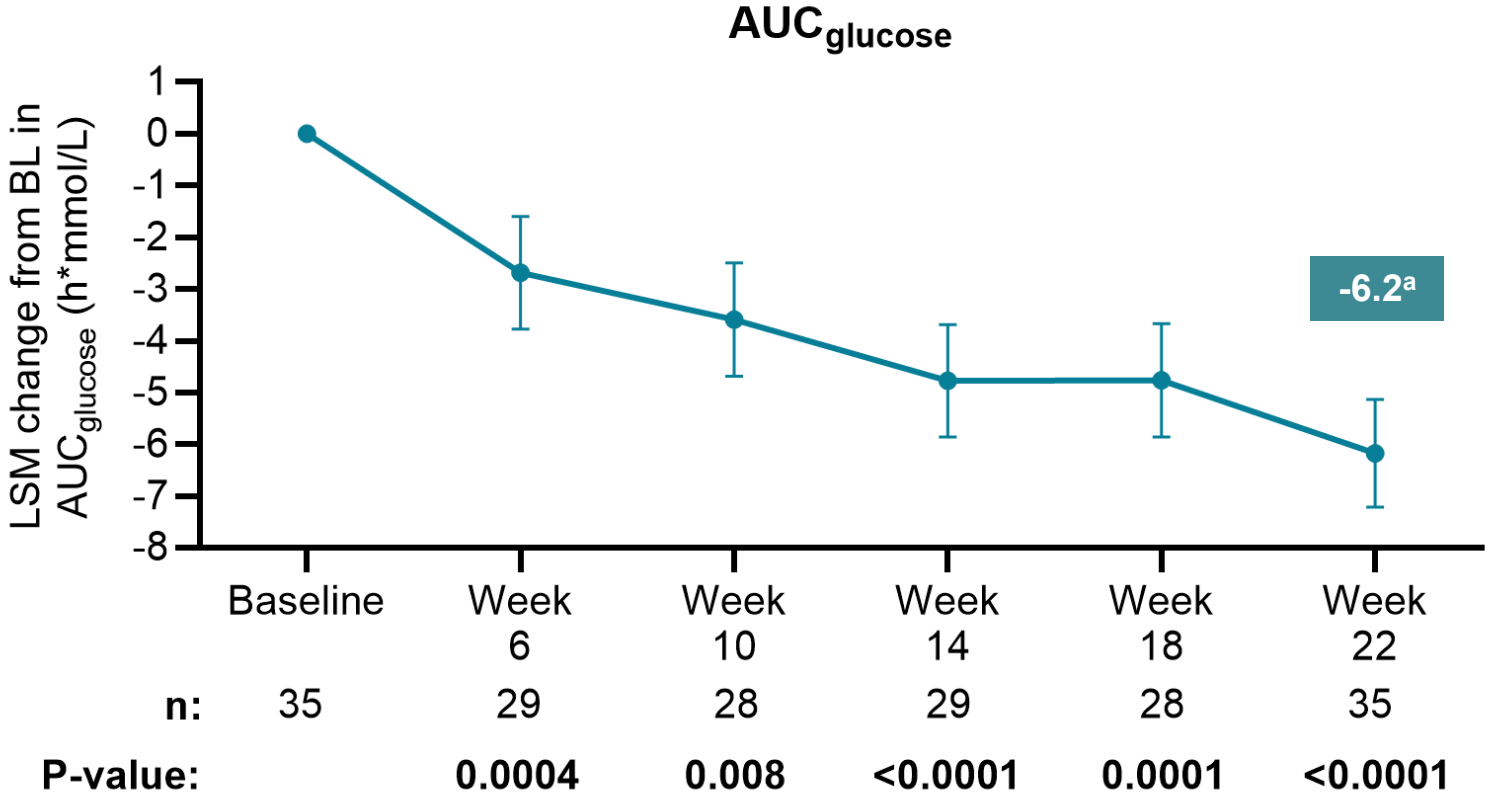


[a]Mean change from baseline to visit OL 22: -4.7 h*mmol/L. [b]Diabetes defined as fasting plasma glucose ≥126 mg/dL, 2-h oGTT plasma glucose ≥200 mg/dL, or HbA1c ≥6.5%.
$AUC_{glucose}$, glucose area under the curve; BL, baseline; LSM, least squares mean; ns, not significant ($P \geq 0.05$); oGTT, oral glucose tolerance test; SE, standard error. Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

Rapid and Sustained Improvements in $AUC_{glucose}$ With Relacorilant

Open Label

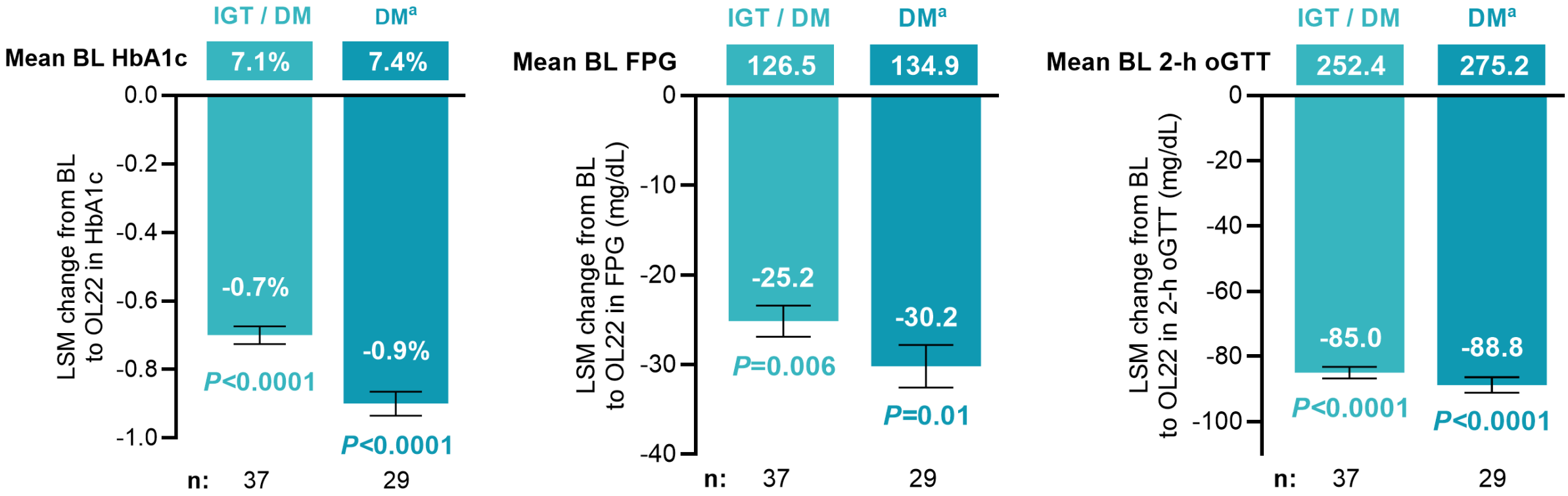
Patients with **hyperglycemia (IGT or DM[b]) who met hyperglycemia response criteria & entered the RW phase**



[a]Mean change from baseline to visit OL 22: -6.2 h*mmol/L. [b]Diabetes defined as fasting plasma glucose ≥126 mg/dL, 2-h oGTT plasma glucose ≥200 mg/dL, or HbA1c ≥6.5%.
$AUC_{glucose}$, glucose area under the curve; BL, baseline; DM, diabetes mellitus; HbA1c, hemoglobin A1c; IGT, impaired glucose tolerance; LSM, least squares mean; oGTT, oral glucose tolerance test; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

Greater Improvements in Glucose Parameters With Relacorilant in Hyperglycemia Responders

Open Label



[b]Diabetes defined as fasting plasma glucose ≥126 mg/dL, 2-h oGTT plasma glucose ≥200 mg/dL, or HbA1c ≥6.5%.
BL, baseline; DM, diabetes mellitus; HbA1c, hemoglobin A1c; FPG, fasting plasma glucose; IGT, impaired glucose tolerance; LSM, least squares mean; oGTT, oral glucose tolerance test; SE, standard error.
Error bars: SE of the mean. LSM and SE calculated using a linear mixed model for repeated measures (MMRM). Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

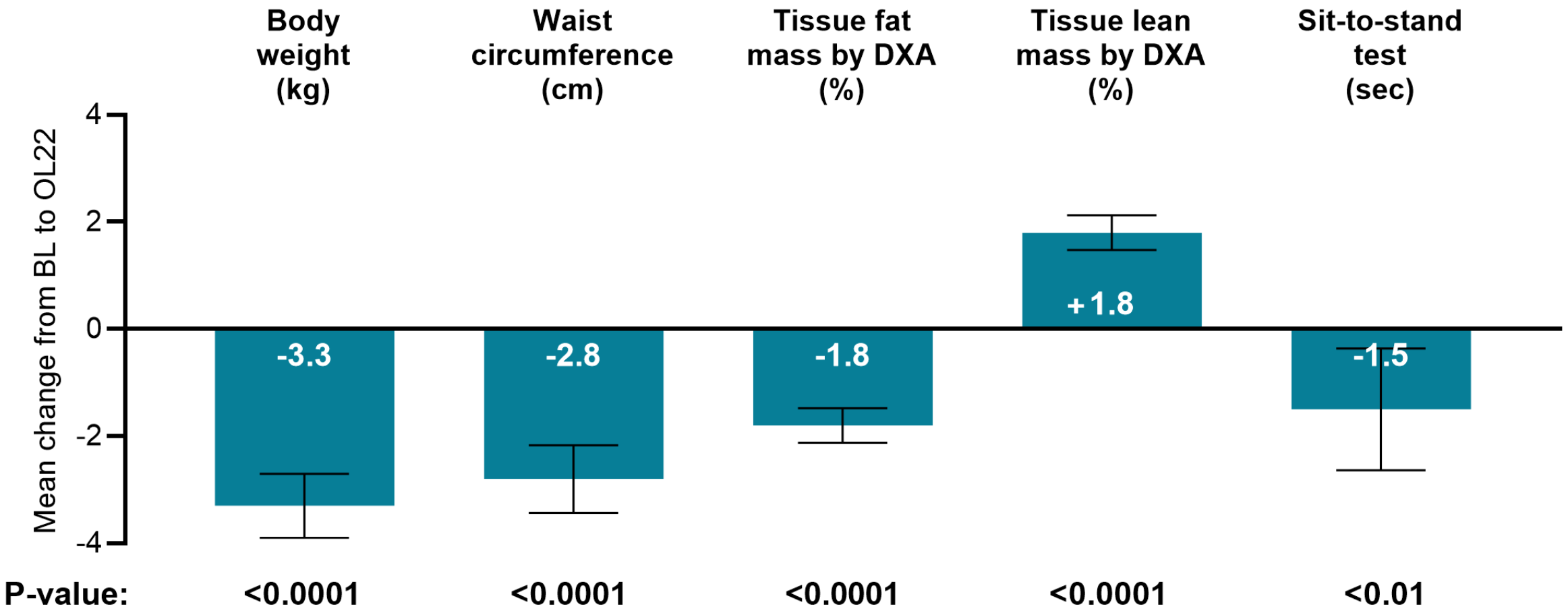
Open-label Results

OTHER SYMPTOMS AND COMORBIDITIES

grace STUDY

Significant Improvements in Body Composition With Relacorilant

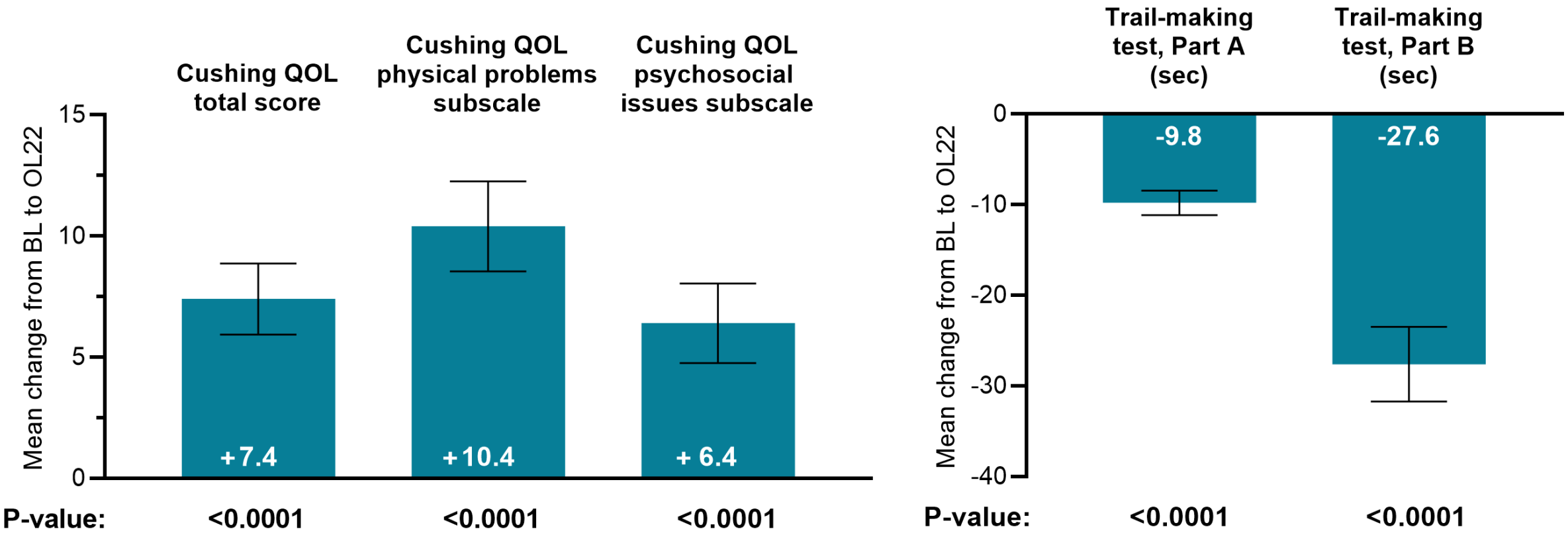
Open Label



BL, baseline; DXA, Dual Energy X-Ray Absorptiometry, OL, open label. Error bars: Standard deviation. Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

Significant Improvements in Quality of Life and Cognitive Assessments With Relacorilant

Open Label



BL, baseline, OL, open label; QOL, quality of life. Error bars: Standard deviation. Wilcoxon rank sum test *P*-values for the mean change from baseline shown.

Open-label Results

SAFETY

Open Label

Adverse Event Summary

n (%)	Relacorilant (N=152)
Patients reporting at least one TEAE (any grade)	147 (96.7)
Patients reporting at least one grade ≥3 TEAE	37 (24.3)
TEAEs resulting in:	
Dose interruption	50 (32.9)
Dose reduction	50 (32.9)
Permanent withdrawal	24 (15.8)
Serious TEAEs	28 (18.4)
Treatment-related serious TEAEs	7 (4.6)
TEAEs leading to death[a] (none relacorilant related)	2 (1.3)

- Due to relacorilant's **specificity for the GR** and its **unique mechanism of action**, the **observed efficacy was seen:**
  - Without cases of relacorilant-induced irregular **vaginal bleeding** with endometrial hypertrophy
  - Without **increases in cortisol** concentrations and relacorilant-induced **hypokalemia**
  - Without reported cases of **adrenal insufficiency**
  - Without independently confirmed **QT prolongation**

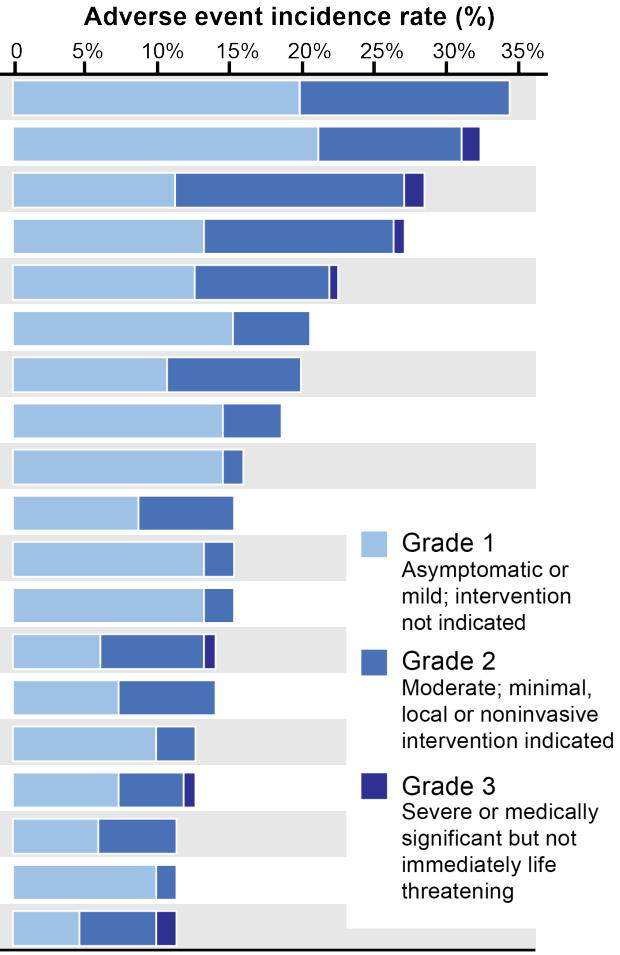
[a]Deaths due to COVID-19 and chronic cardiac failure.
GR, glucocorticoid receptor; OL, open label; TEAE, treatment-emergent adverse event.

Open Label

Adverse Events Occurring in ≥10% of Patients

Among patients in the open-label phase

n (%)	Relacorilant
Nausea	52 (34.2)
Edema peripheral	50 (32.9)
Pain in extremity	43 (28.3)
Back pain	41 (27.0)
Fatigue	34 (22.4)
Headache	31 (20.4)
Arthralgia	30 (19.7)
Diarrhea	28 (18.4)
Skin hyperpigmentation	24 (15.8)
Abdominal pain upper	23 (15.1)
Constipation	23 (15.1)
Dizziness	23 (15.1)
Myalgia	21 (13.8)
Paresthesia	21 (13.8)
Asthenia	19 (12.5)
Vomiting	19 (12.5)
Abdominal pain	17 (11.2)
Decreased appetite	17 (11.2)
Muscular weakness	17 (11.2)



- The majority of AEs were **mild to moderate** in severity
- No new safety signals were identified
- The frequency of serious AEs was low, with no dose-dependent pattern

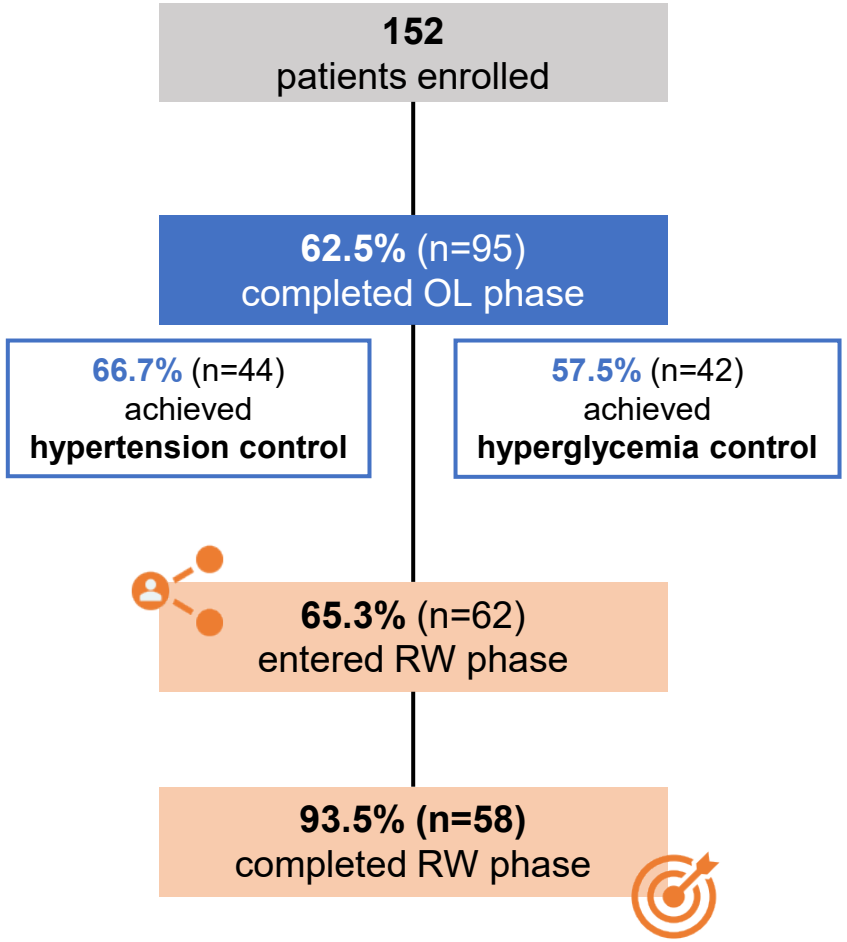
TEAEs and CTCAE grade shown. AE, adverse event; CTCAE, Common Terminology Criteria for Adverse Events; OL, open label; TEAE, treatment-emergent adverse event.

Randomized-withdrawal Results

Randomized Withdrawal

Patient Demographics & Baseline Characteristics

Mean (SD)	Relacorilant (n=30)	Placebo (n=32)
Age, yrs	46.6 (11.0)	48.8 (14.4)
Female, n (%)	22 (73.3)	26 (81.3)
Weight, kg	93.3 (27.4)	88.6 (21.1)
BMI, kg/m²	33.3 (7.6)	32.6 (6.5)
Waist circumference, cm	113.8 (17.7)	108.9 (17.1)
ACTH-dependent, n (%)	26 (86.7)	23 (71.9)
Plasma ACTH, pg/mL 24-h UFC, µg/d	91.7 (85.7) 257.1 (449.1)	71.7 (74.7) 301.3 (287.9)
ACTH-independent, n (%)	4 (13.3)	9 (28.1)
Plasma ACTH, pg/mL 24-h UFC, µg/d	5.9 (2.3) 66.9 (36.8)	10.0 (9.0) 142.2 (194.1)



ACTH, adrenocorticotropic hormone; BMI, body mass index; OL, open-label phase; RW, randomized withdrawal; SD, standard deviation; UFC, urinary free cortisol.

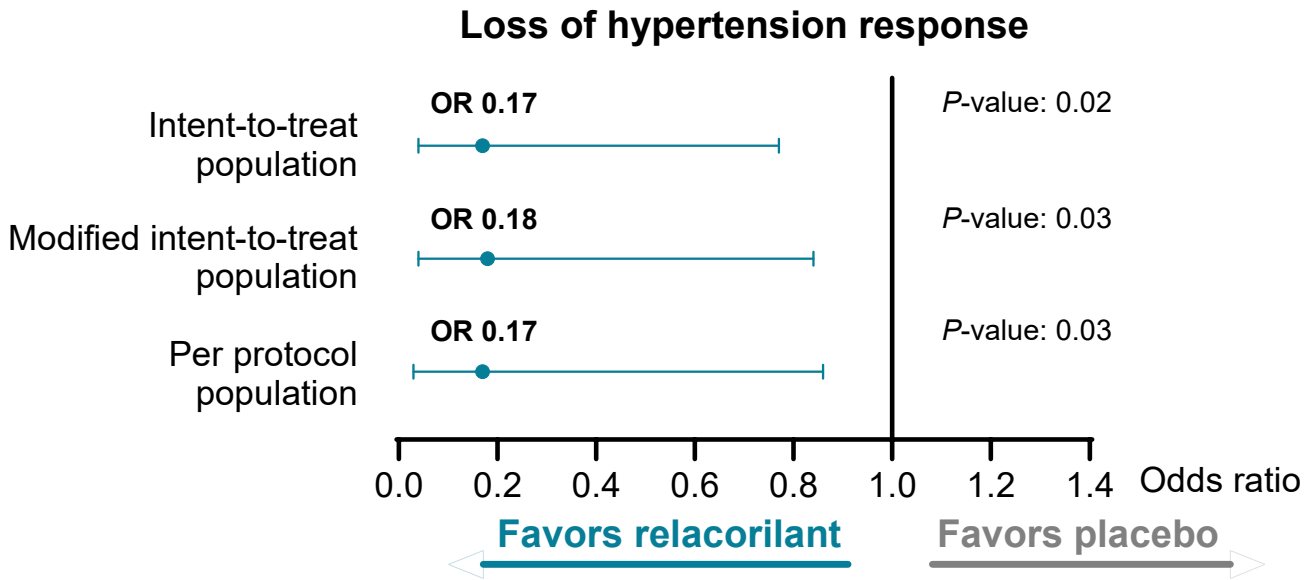
Randomized-withdrawal Results

HYPERTENSION

Primary Endpoint Met: Hypertension

Hypertension responders who randomized

- In the randomized-withdrawal phase, **significantly more patients receiving placebo lost hypertension control** compared to those who continued to receive relacorilant
  - **Odds ratio 0.17** for relacorilant vs placebo (*P*=0.02)
  - Patients receiving relacorilant were **5.9x more likely to maintain hypertension response**

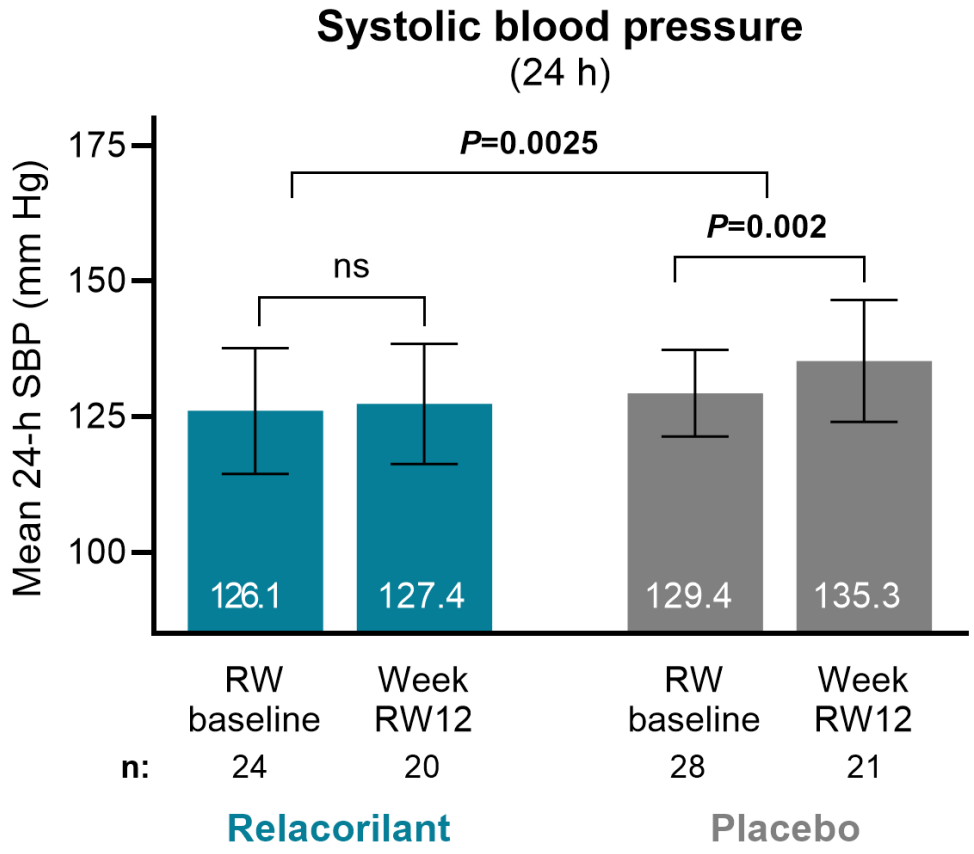


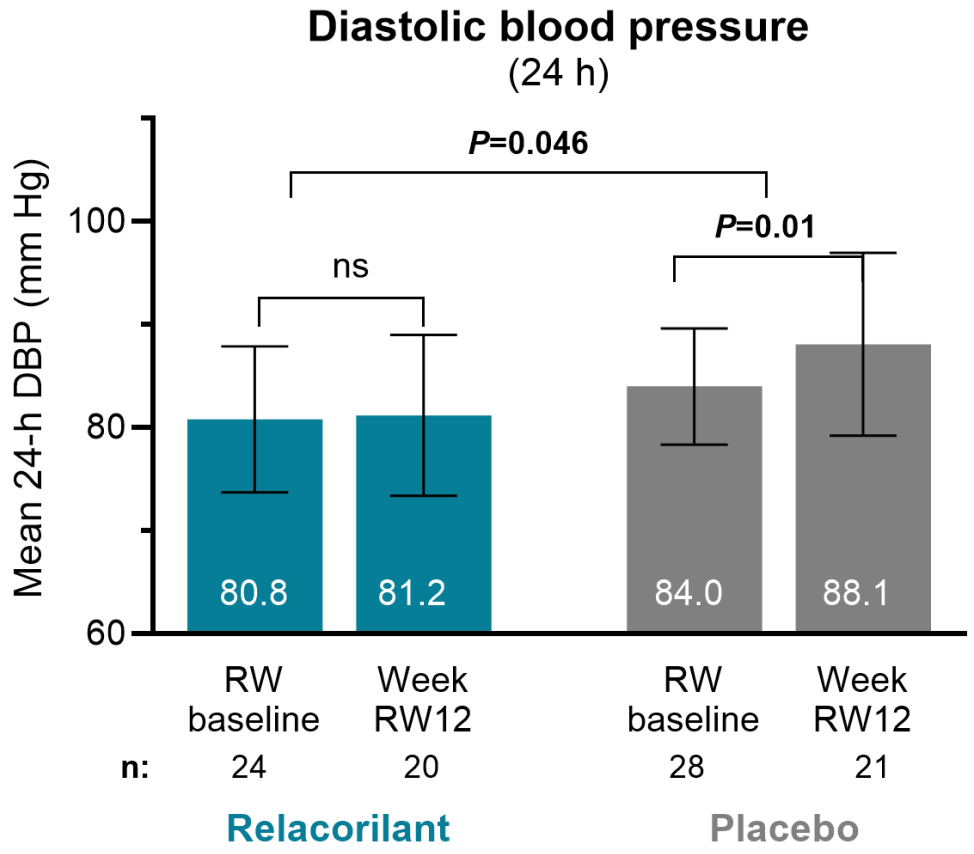
OR, odds ratio. Bars represent 95% confidence intervals.

Randomized Withdrawal

Similar Blood Pressure Trends Observed in all Patients Randomized, Favoring Relacorilant Over Placebo

In all patients with available ABPM data



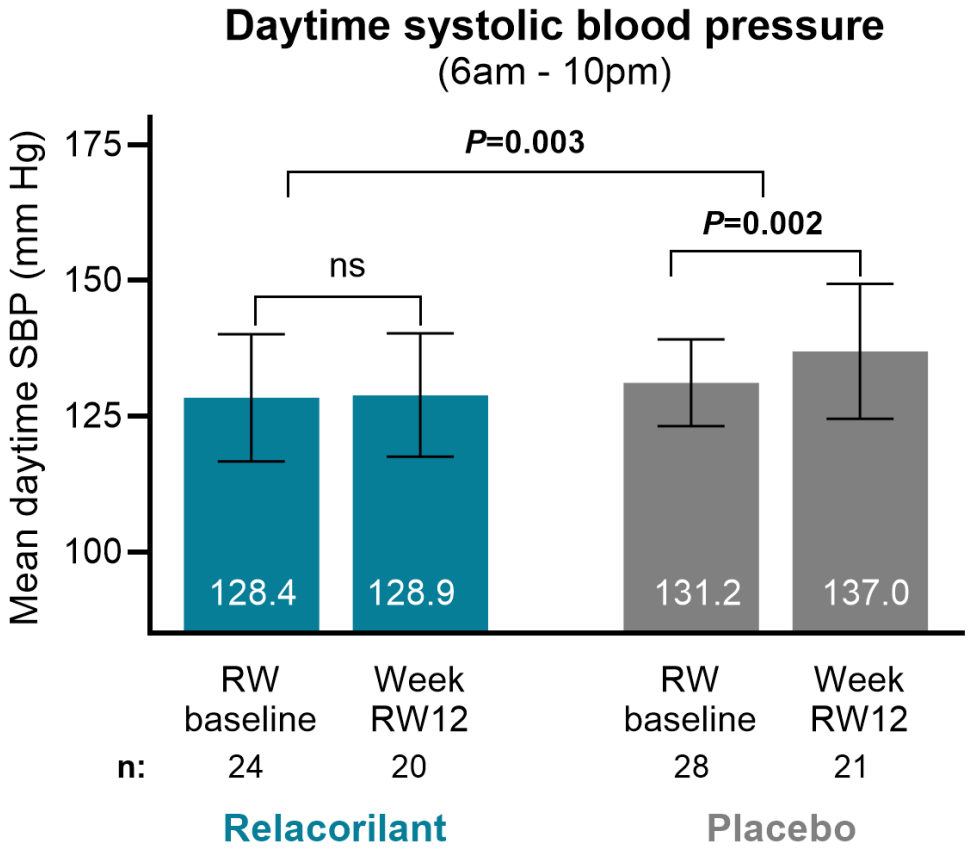


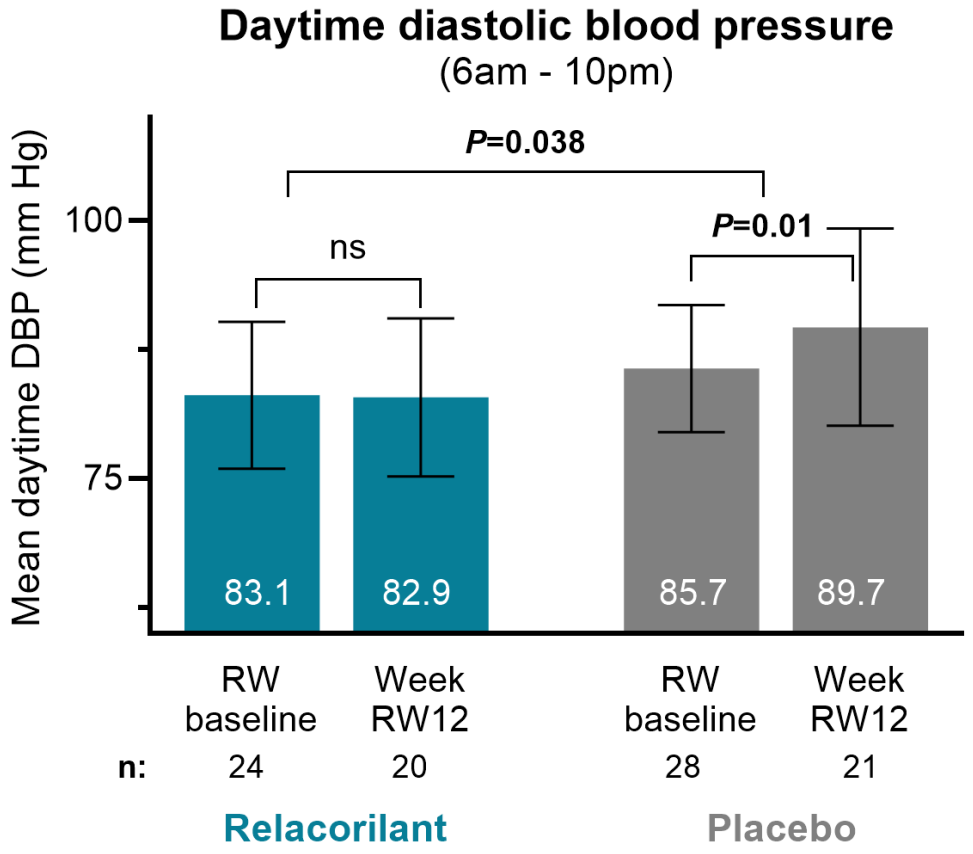
ABPM, ambulatory blood pressure monitoring; DBP, diastolic blood pressure; ns, not significant ($P \geq 0.05$); RW, randomized withdrawal; SBP, systolic blood pressure. Blood pressure measured by ABPM.
Error bars: Standard deviation. Wilcoxon rank sum test *P*-values for the observed mean within each treatment arm shown.

Similar Blood Pressure Trends Observed in all Patients Randomized, Favoring Relacorilant Over Placebo

Randomized Withdrawal

In all patients with available ABPM data



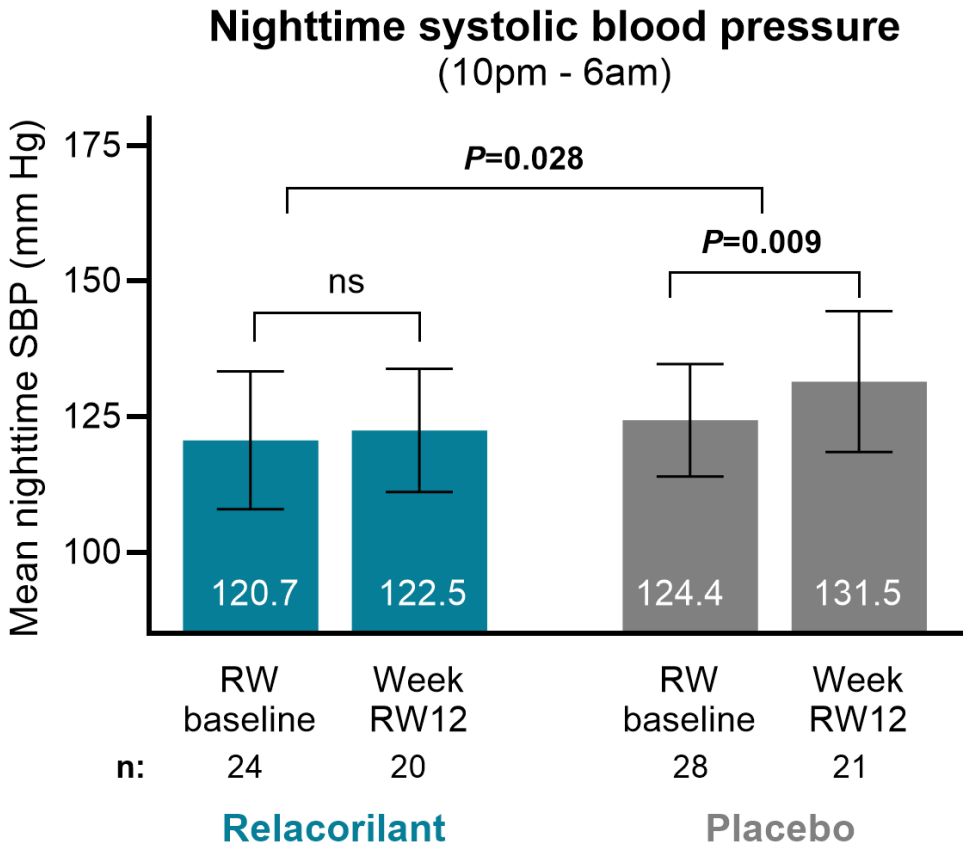


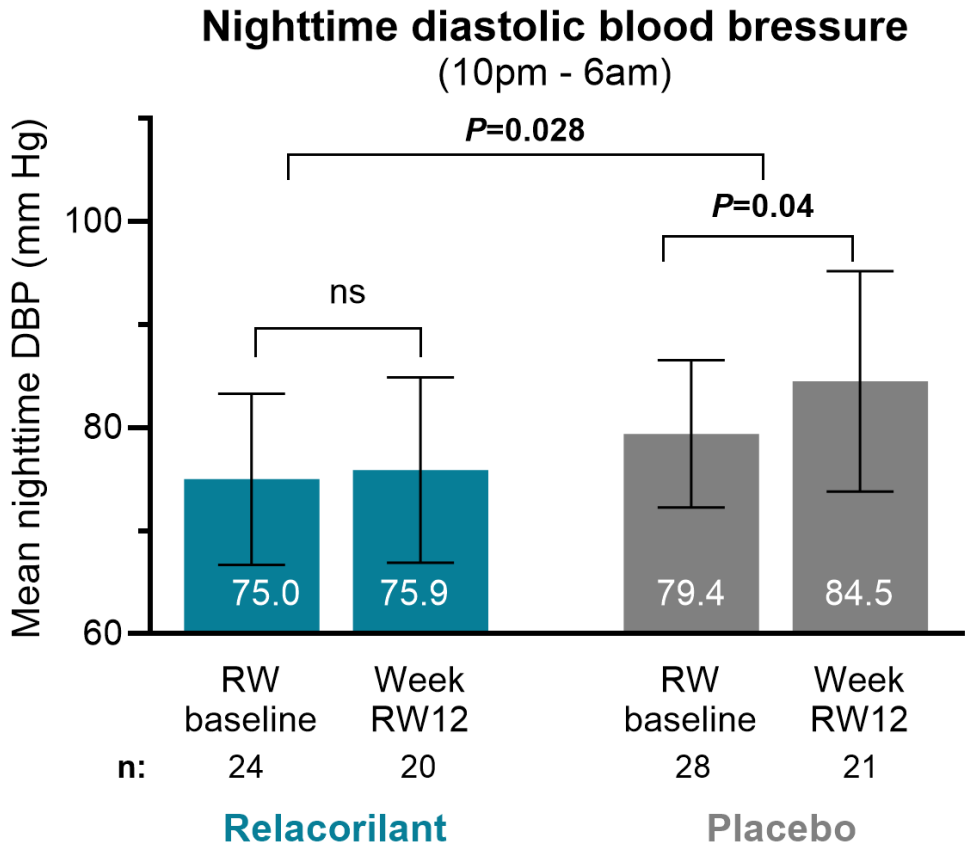
ABPM, ambulatory blood pressure monitoring; DBP, diastolic blood pressure; ns, not significant ($P \geq 0.05$); RW, randomized withdrawal; SBP, systolic blood pressure. Blood pressure measured by ABPM; daytime blood pressure defined as blood pressure measurements between 6am and 10pm. Error bars: Standard deviation. Wilcoxon rank sum test *P*-values for the observed mean within each treatment arm shown.

Randomized Withdrawal

Similar Blood Pressure Trends Observed in all Patients Randomized, Favoring Relacorilant Over Placebo

In all patients with available ABPM data





ABPM, ambulatory blood pressure monitoring; DBP, diastolic blood pressure; ns, not significant ($P \geq 0.05$); RW, randomized withdrawal; SBP, systolic blood pressure. Blood pressure measured by ABPM; nighttime blood pressure defined as blood pressure measurements between 10pm and 6am. Error bars: Standard deviation. Wilcoxon rank sum test *P*-values for the observed mean within each treatment arm shown.

Randomized-withdrawal Results

HYPERGLYCEMIA AND OTHER SYMPTOMS AND COMORBIDITIES

Improvements in Glycemic Measures Maintained With Relacorilant

Randomized Withdrawal

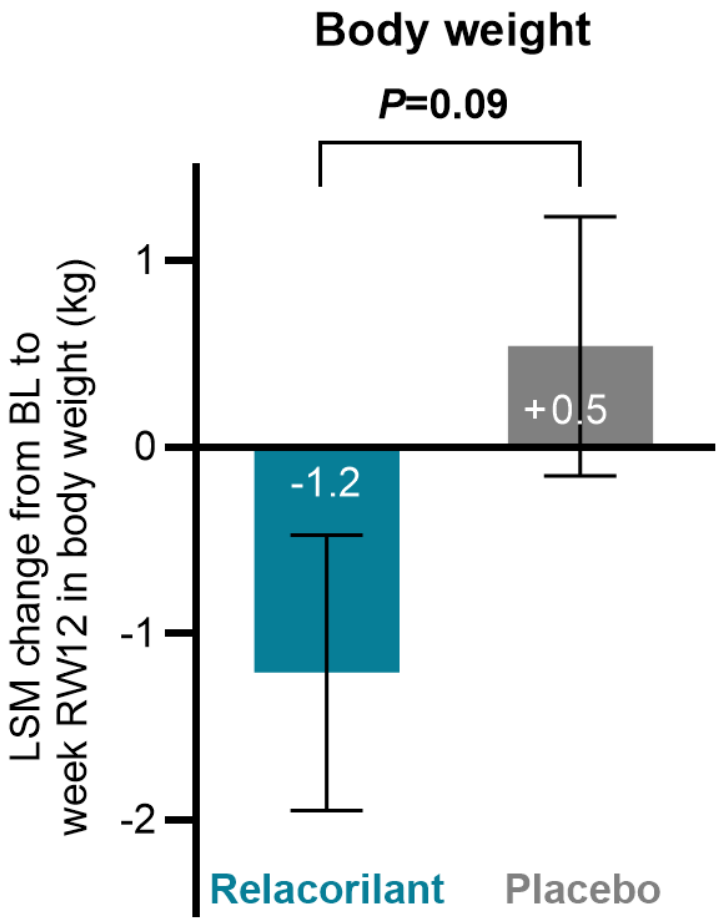
	Relacorilant (n=30)	Placebo (n=32)
Change from RW baseline to week RW12 in:		
$AUC_{glucose}$ (in patients with hyperglycemia at study entry), h*mmol/L		
n	15	19
Mean (SD)	+1.1 (4.7)	+4.9 (6.1)
Wilcoxon signed rank sum P-value[a]	ns	**0.0003**
HbA1c (in patients with hyperglycemia at study entry), %		
n	16	19
Mean (SD)	+0.1 (0.8)	+0.3 (0.6)
Wilcoxon signed rank sum P-value[a]	ns	**0.03**
HbA1c (in patients with diabetes at study entry), %		
n	13	13
Mean (SD)	+0.1 (0.8)	+0.4 (0.6)
Wilcoxon signed rank sum P-value[a]	ns	**0.04**

- At the end of the RW phase, patients who switched to **placebo experienced significant increases in $AUC_{glucose}$ and HbA1c**
- In contrast, glycemic measures were maintained in patients who continued to receive **relacorilant**

$AUC_{glucose}$, glucose area under the curve; HbA1c, hemoglobin A1c; ns, not significant ($P \geq 0.05$); RW, randomized withdrawal. Wilcoxon rank sum test *P*-values for the observed mean within each treatment arm shown.
[a]Wilcoxon signed-rank test P-values within each treatment arm.

Improvements in Body Composition Maintained With Relacorilant

Randomized Withdrawal



	Relacorilant (n=30)	Placebo (n=32)
Change from RW baseline to week RW12 in all patients in the RW phase:		
Waist circumference		
n	26	30
Mean (SD), cm	-1.2 (3.7)	+3.8 (10.4)
Wilcoxon signed rank sum P-value[a]	ns	**0.008**
Tissue fat mass		
n	17	22
Mean (SD), %	-0.2 (1.7)	+1.6 (1.8)
Wilcoxon signed rank sum P-value[a]	ns	**0.0002**
Tissue lean mass		
n	17	22
Mean (SD), %	+0.2 (1.7)	-1.6 (1.8)
Wilcoxon signed rank sum P-value[a]	ns	**0.0002**

- Similar trends observed across measures of body composition
- Those who **switched to placebo** experienced a **deterioration** in body composition
- In contrast, **trends toward further improvement** were observed in the **relacorilant** arm

BL, baseline; LSM, least squares mean; ns, not significant ($P \geq 0.05$); RW, randomized withdrawal; SE, standard error.
Error bars: SE of the mean. [a]Wilcoxon signed-rank test P-values within each treatment arm.

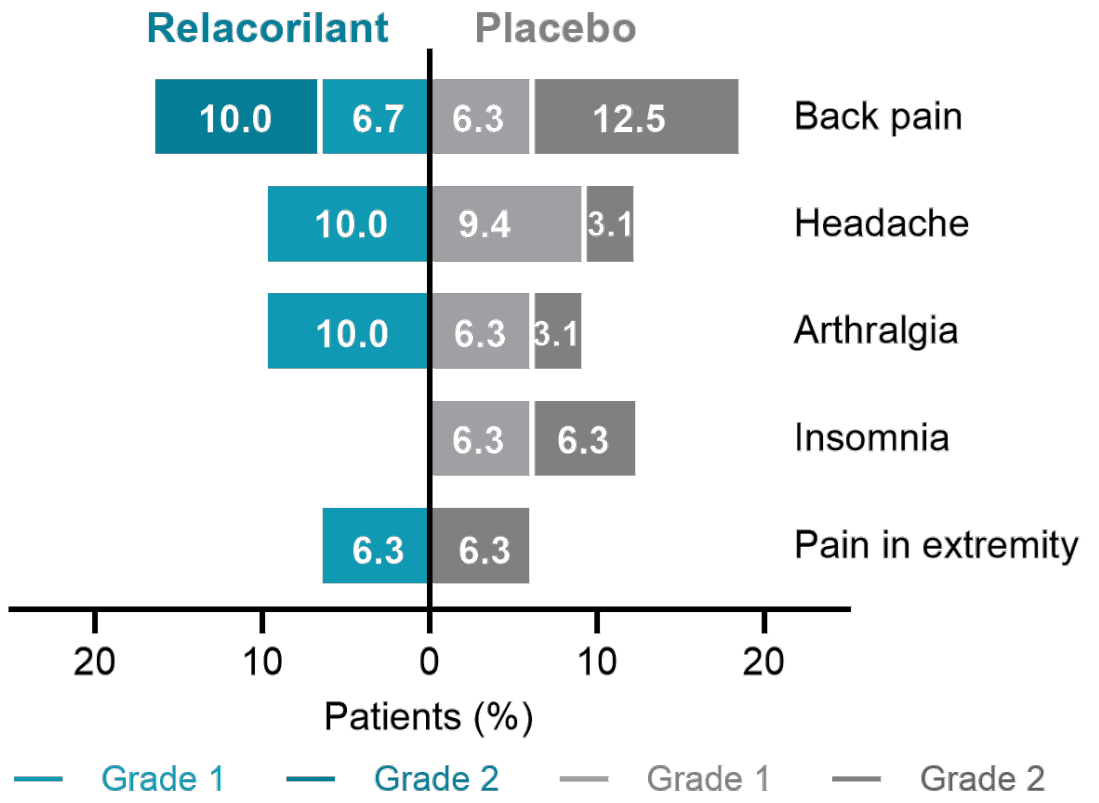
Randomized-withdrawal Results

SAFETY

Adverse Events Occurring in ≥5% of Patients

Among patients in the randomized-withdrawal phase

n (%)	Relacorilant (n=30)	Placebo (n=32)
Back pain	5 (16.7)	6 (18.8)
Headache	3 (10.0)	4 (12.5)
Arthralgia	3 (10.0)	3 (9.4)
Insomnia	0	4 (12.5)
Pain in extremity	2 (6.7)	2 (6.3)



Conclusions

- GRACE **met its primary endpoint**
- **Significant improvements in hypertension, hyperglycemia, and other manifestations of cortisol excess** were observed throughout the treatment with relacorilant
- Due to relacorilant's **specificity for the glucocorticoid receptor** and its **unique mechanism of action**, the observed efficacy was seen:
  - Without cases of relacorilant-induced irregular **vaginal bleeding** with endometrial hypertrophy
  - Without **increases in cortisol** concentrations and relacorilant-induced **hypokalemia**
  - Without reported cases of **adrenal insufficiency**
  - Without independently-confirmed **QT prolongation**

grace STUDY

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