November 15, 2026

Expert Perspectives on PBH & Avexitide



Opening Remarks

Camille Bedrosian, MD

Chief Medical Officer of Amylyx

DISCLAIMER

Statements contained in this presentation regarding matters that are not historical facts are “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Such statements include, but are not limited to, Amylyx’s expectations regarding: the therapeutic potential and safety of avexitide as a treatment for PBH; expectations regarding the timing for NDA submission with the U.S. Food and Drug Administration; the potential benefits of regulatory designations held with the FDA; and expectations regarding timing and preparedness for potential commercialization of avexitide, if approved. Any forward-looking statements in this presentation are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in or implied by such forward-looking statements. Risks that contribute to the uncertain nature of the forward-looking statements include: the success, cost, and timing of Amylyx’s program development activities, including ongoing and planned clinical trials, Amylyx’s ability to execute on its development and regulatory strategy, regulatory developments, Amylyx’s cash runway and ability to fund operations, as well as the risks and uncertainties set forth in Amylyx’s United States Securities and Exchange Commission (SEC) filings, including Amylyx’s Annual Report on Form 10-K for the year ended December 31, 2025, and subsequent filings with the SEC. All forward-looking statements contained in this presentation speak only as of the date on which they were made. Amylyx undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made.

Agenda

1	Opening Remarks & PBH Community Video	Camille Bedrosian, MD, Chief Medical Officer of Amylyx
2	Post Bariatric Hypoglycemia: Current Disease State	Zeb Saeed, MD, LUCIDITY clinical trial Investigator and Associate Physician at Brigham and Women's Hospital
3	Review of the Pivotal Phase 3 LUCIDITY Results	Marilyn Tan, MD, FACE, FACP, Principal Investigator of the LUCIDITY clinical trial and Clinical Professor of Medicine at Stanford University School of Medicine
4	Q&A	Moderated by Jamie Timmons, MD, Senior Vice President, Medical Affairs at Amylyx
5	Closing Remarks	Camille Bedrosian, MD, Chief Medical Officer of Amylyx

Key Themes for Today's Discussion

1. PBH is a debilitating condition with no approved treatment options that can profoundly impact patients' quality of life

2. In LUCIDITY, avexitide demonstrated consistent, highly statistically significant, and clinically meaningful reductions in hypoglycemic events across all endpoints

3. Based on the strength and consistency of the data, we believe avexitide has the opportunity to potentially change practice in PBH

Post Bariatric Hypoglycemia: Current Disease State

Zeb Ijaz Saeed, MD

Associate Physician, Brigham and Women's Hospital

Instructor of Medicine, Harvard Medical School

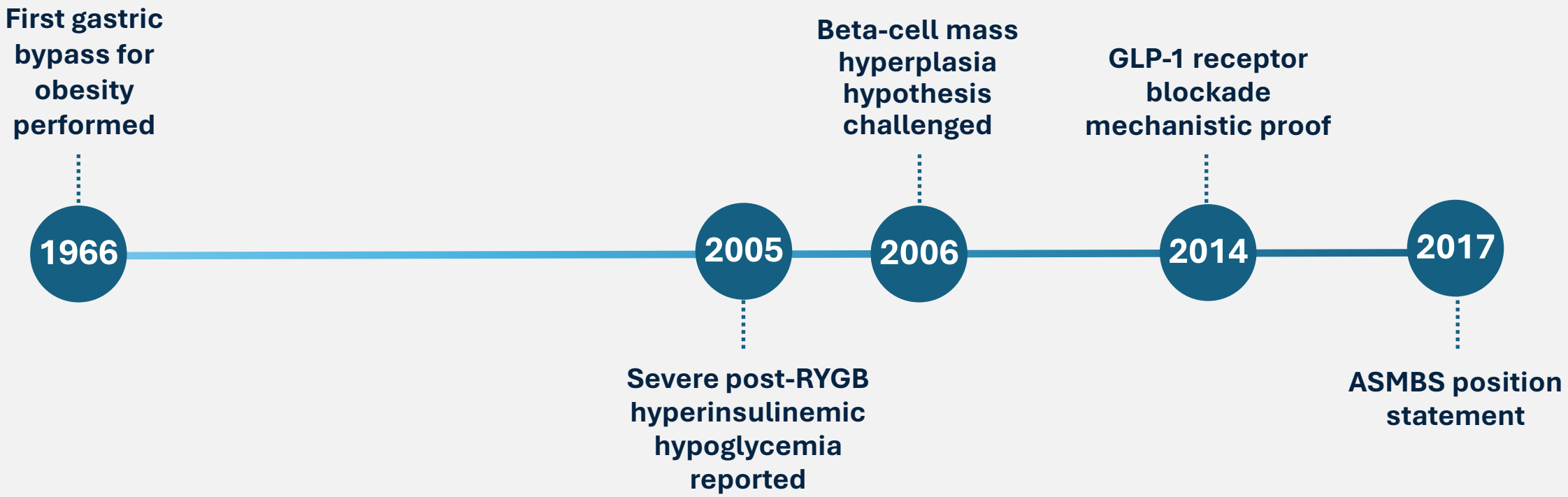
Site-Investigator in LUCIDITY trial



DISCLOSURES

ZIS's institution receives research support from Amylyx Pharmaceuticals, Kailera Therapeutics and Eli Lilly.
ZIS completed an advisory consultation with Sanofi Pharmaceuticals

Post-Bariatric Hypoglycemia (PBH): Evolution of Understanding



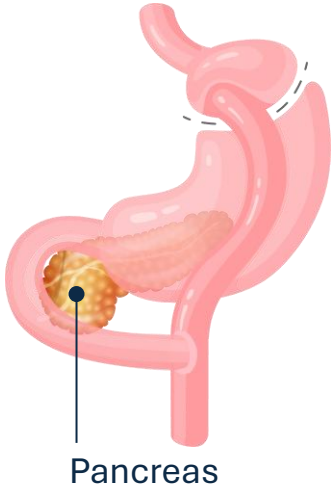
1. Mason EE, Ito C. *Surg Clin North Am*. 1967;47:1345–1351. 2. Service GJ et al. *N Engl J Med*. 2005;353:249–254. 3. Meier JJ et al. *Diabetes Care* 1 July 2006; 29 (7): 1554–1559. 4. Salehi M et al. *Gastroenterology*. 2014;146:669–680.e2. 5. Eisenberg D et al. *Surg Obes Relat Dis*. 2017;13:371–378.

Pathophysiology of PBH

PBH is driven in part by an exaggerated GLP-1 response after meals

1

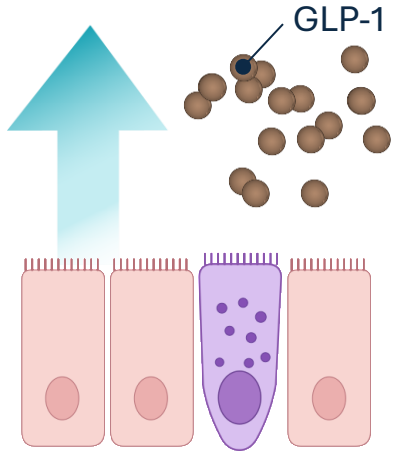
Altered Gastrointestinal Anatomy



Rapid nutrient transit

2

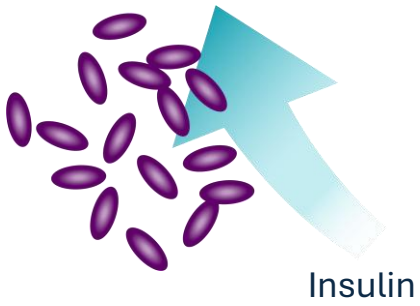
Nutrient Delivery in the Intestine



Post-meals GLP-1 overproduction

3

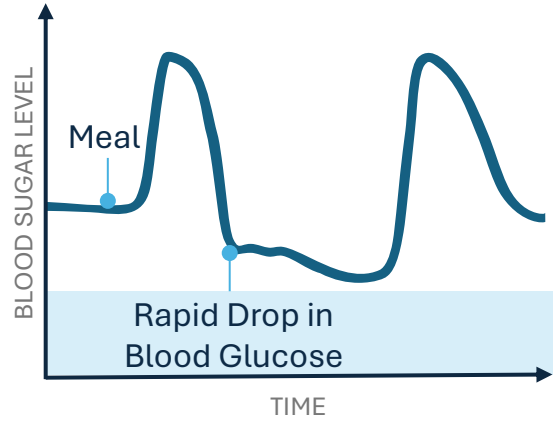
Hyper-secretion of Insulin in the Bloodstream



Exaggerated insulin response

4

Recurrent, Rapid Drops in Blood Glucose



Recurrent postprandial hypoglycemia

1. Sheehan A, Patti ME. *Diabetes Metab Syndr Obes*. 2020;13:4469-4482; **2**. Craig CM, et al. *Diabetes Obes Metab*. 2017; 1-10. **3.** Thorens B,. et al. *Diabetes*.1993;42(11):1678-1682. **4.** Craig CM, et al. *Diabetes Obes Metab*. 2018;20(2):352-361. **5.** Smith NK, et al. *Neurochem Int*. 2019;128:94-105. **6.** Meloni AR, et al. *Diabetes Obes Metab*. 2013;15(1):15-27. **7.** Craig CM, et al. *Diabetologia*. 2017;60(3):531-540. **8.** Craig C, et al. *J Endocr Soc*. 2022;6(Suppl 1):A349.

PBH: Prevalence and Diagnostic Challenges

Prevalence :~160,000 People in U.S. Estimated to be Living with PBH Requiring Medical Management [1]

Prevalence depends on how PBH diagnosed

~ 1/3rd patients with history of RYGB or Sleeve Gastrectomy have reported symptoms of hypoglycemia[2]

PBH Diagnosis: Clinical Suspicion then Biochemical Confirmation

Delayed presentation
Symptoms may begin long after routine bariatric follow-up ends.

Missed testing
Not capturing postprandial lows.

PBH May be Underdiagnosed or Have Delayed Diagnosis[3]

Nonspecific symptoms
Dizziness, fatigue and confusion may be attributed to other conditions.

Hypoglycemic unawareness
Recurrent hypoglycemia may blunt warning symptoms.

RYGB= Roux-en-Y Gastic Bypass. PBH=post-bariatric hypoglycemia; **1.** Craig CM, et al. *Surg Obes Relat Dis.* 2026;22(6):674-683. **2.** Lee CJ et al. *Obes Relat Dis.* 2018 Jun;14(6):797-802.
3. Hazlehurst J et al. *Endocr Connect.* 2024 May 1;13(5):e230285.

Why PBH Matters: Symptoms, Consequences, and Real-World Impact



Clinical Presentation[1-4]

Autonomic Symptoms:

- Sweating
- Tremors
- Palpitations
- Anxiety

Neuroglycopenic Symptoms:

- Confusion
- Impaired cognition
- Blurred vision
- Loss of consciousness
- Fatigue
- Disorientation
- Seizures



Recurrent Hypoglycemia [5-7]

- Recurrent neuroglycopenia :
  - > Cognitive impairment
  - > Impaired judgment
  - > Increased risk of falls
  - > Increased risk of accidents
- Neuroglycopenia may be the first recognized manifestation



Impact of PBH and Consequences[1-4, 5-8,9]

- Psychological distress
- Worse quality of life
- Reduced productivity
- Impaired daily functioning
- Safety concerns: driving, falls
- High disability burden
  - > **In one study, over 90% self-reported living with a disability)**[9]

1. Suhl E, et al. *Surg Obes Relat Dis.* 2017; 13(5): 888-896. **2.** Varma S, et al. *Surg Obes Relat Dis. 2017; 13(10): 1728-1734.* **3.** Patti ME and Goldfine AB, *Gastroenterology*. 2014; 146(3):605-608. **4.** Hazlehurst J, et al. *Endocr Connect.* 2024; 13(5);e230285. **5.** Salehi M, et al. *J Clin Endocrinol Metab*. 2018;103(8):2815-2826. **6.**Patti ME, et al. *Diabetologia*. 2005;48(11):2236-2240. **7.** Lehmann V, et al. *Diabetes Obes Metab*. 2021;23(9):2189-2193. **8.** Yu Y, et al. *Metab Syndr Relat Disord*. 2025;23(5):244-252. **9.** Craig CM and McLaughlin TL. *Surg Obes Relat Dis.* 2021 Nov;17(11):1865-1872.

Current PBH Management and Gaps in Treatment

Current Treatment Approaches

Diet

- Restrictive eating
- Difficult to sustain

Off-Label Medications

- Variable efficacy
- Tolerability limitations

Surgical Intervention

- Last resort
- Invasive procedure
- Significant burden

Persistent gaps in treatment exist
No FDA approved therapies for PBH
Symptoms often misattributed or underdiagnosed
Patients can continue to experience sudden hypoglycemic events despite active management

Significant unmet need remains for therapies that provide reliable control of PBH symptoms and address underlying disease mechanism

PBH can significantly impact daily life…

…yet PBH management is limited and inconsistent

"I get very confused, very sleepy to the point that I can't almost hold my head up and I sweat profusely. […] And I usually sleep at least 2 hours after I get my blood sugar back up, my body doesn't recover. **It can ruin a day.**"

"Severely disoriented to the point where I actually felt drunk. Disoriented confusion […] And uncoordinated, completely uncoordinated. And like I said, disoriented. **I could not put a simple thought together.**"

"They could happen in my car, and I would just pull over on the highway just because I'd kind of **forget where I was at.**"

"I actually had my own in-home daycare. I was taking care of several kids and when the episodes started getting bad enough and so unpredictable **I had to close my daycare** because I just felt like it was really unsafe for me to be caring for infants and other children when I'd have these episodes of being confused and just absolutely exhausted afterwards."

"I do a lot of administration work, and there are times that I have to take a break because, like I said, when I'm having an episode, I seem to be confused. Like, I'm in a daze kind of, and rather than make a mistake, **I just [do] not work until it passes.**"

Quotes from qualitative interviews performed in subgroup of LUCIDITY participants at Week 16 when asked to describe the hypoglycemic events they experienced before entering LUCIDITY and the impact of PBH on their daily life.

Avexitide for Post-Bariatric Hypoglycemia: Results from the Pivotal Phase 3 LUCIDITY Trial

Marilyn Tan, MD, FACE, FACP

Clinical Professor of Medicine (Endocrinology)
Stanford University School of Medicine

On Behalf of the LUCIDITY Investigators



Avexitide is an investigational product and has not been approved by any regulatory agency

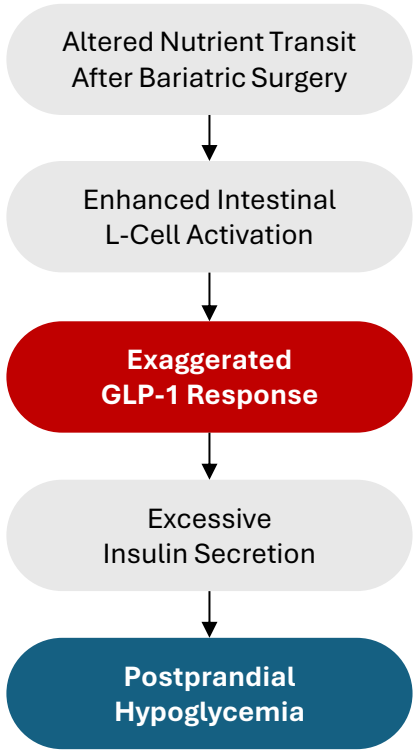
RELEVANT DISCLOSURES:
Dr. Tan has received research payments from Amylyx Pharmaceuticals as primary investigator and received consulting payments.

PBH is a Chronic Metabolic Condition Characterized by Exaggerated Postprandial GLP-1 Responses, Recurrent Hypoglycemia, and Substantial Clinical Burden

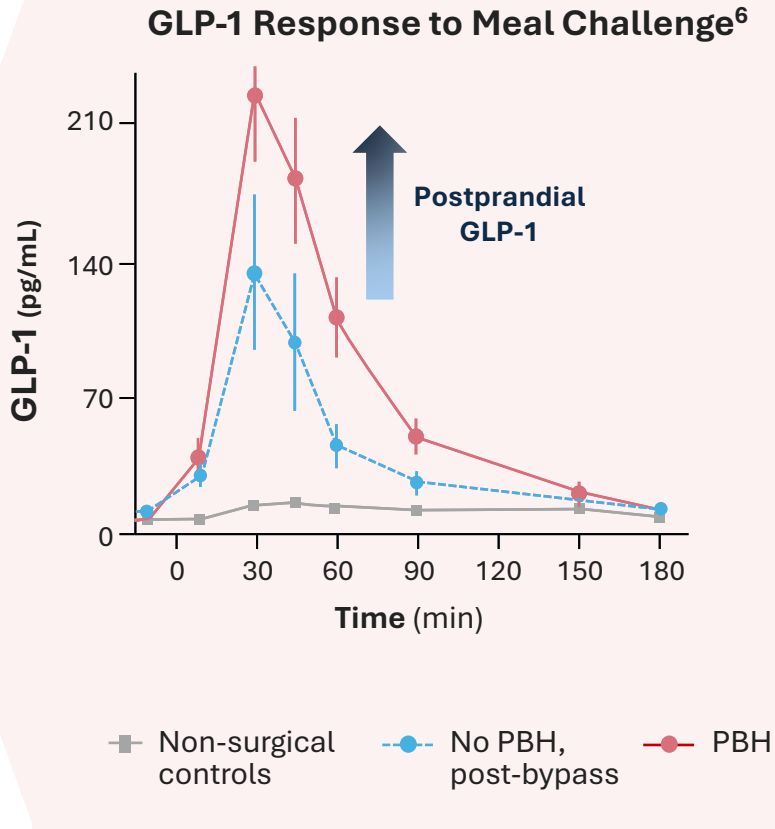
About Post-Bariatric Hypoglycemia[1-5]

- Characterized by **recurrent postprandial hypoglycemia**, typically 1-3 hours after meals and developing months to years after surgery
- Requires medical management in **~12% of patients after Roux-en-Y gastric bypass (RYGB)** and **~5% after sleeve gastrectomy**
- Associated with **substantial clinical burden**, including effects on patient safety, daily functioning, and long-term health
  - > In a recent survey, **>90% of respondents with PBH considered themselves functionally disabled** by their condition

The Role of GLP-1 in PBH[3,4]



Episodes may also be triggered by stress, physical activity, and other factors



GLP-1, glucagon-like peptide-1; PBH, post-bariatric hypoglycemia; RYGB, Roux-en-Y gastric bypass

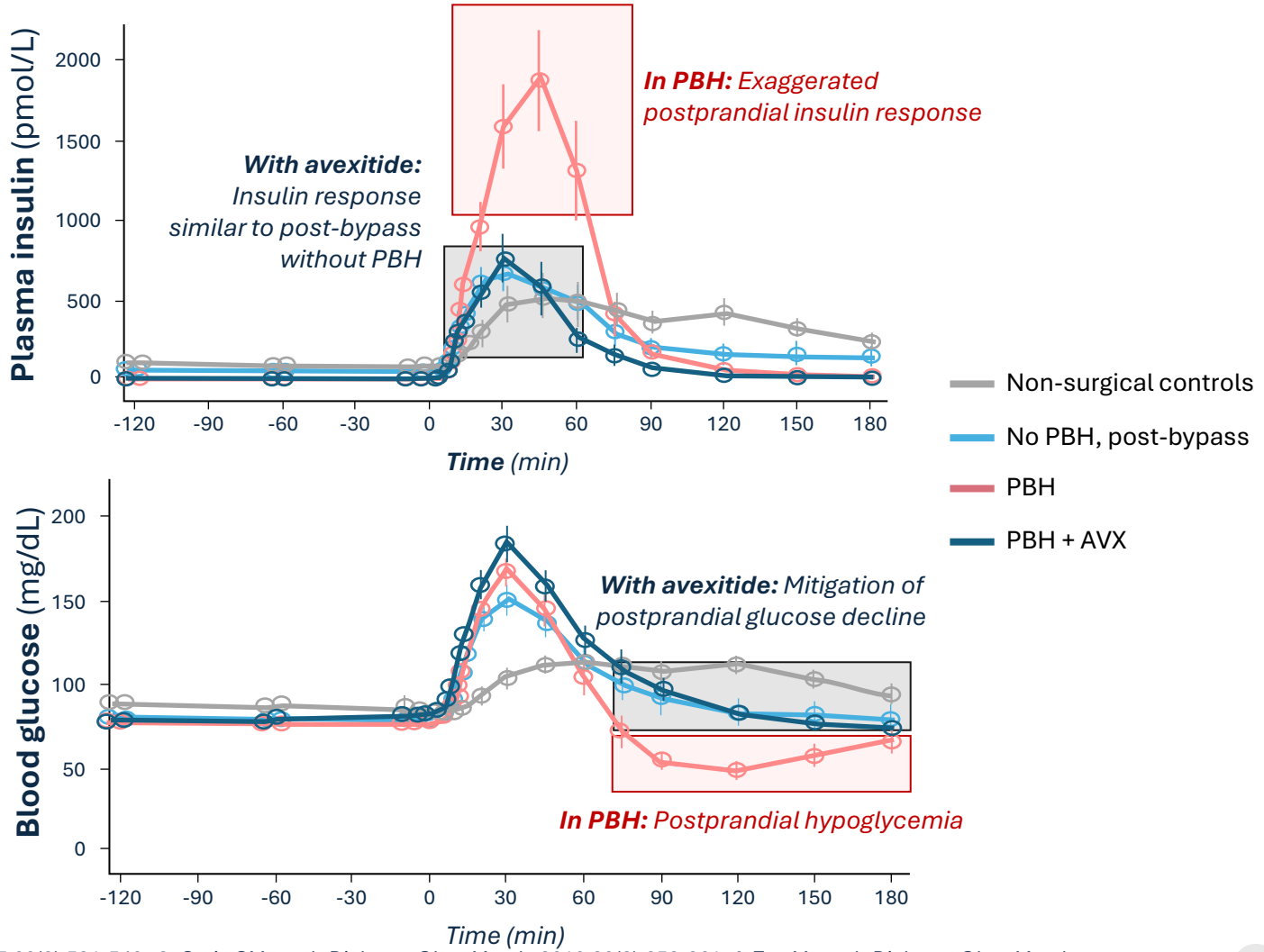
1. Craig CM, et al. *Surg Obes Rel Dis*. 2026;22(6):674-683. **2.** Patti ME, Goldfine AB. *Lancet Diabetes Endocrinol*. 2016;4(2):94-96. **3.** Salehi M, et al. *J Clin Endocrinol Metab*. 2018;103(8):2815-2826. **4.** Patti ME, Goldfine A. *Gastroenterology*. 2014;146(3):605-608. **5.** Craig CM, McLaughlin TL. *Surg Obes Relat Dis*. 2021;17(11):1865-1872. **6.** Salehi M, et al. *Gastroenterology*. 2014;146(3):669-680.e2. Graph adapted from Salehi M, et al. *Gastroenterology*. 2014;146(3):669-680.e2.

Avexitide Consistently Reduced Hypoglycemia Across a Mechanistic Study and Five Positive Clinical Trials in PBH

Avexitide
Investigational GLP-1 Receptor Antagonist

- **In a mechanistic study, avexitide attenuated the exaggerated postprandial insulin response** and subsequent glucose decline, with responses resembling those of post-bariatric individuals without PBH[1]
- **In 5 prior clinical trials, avexitide reduced hypoglycemia across multiple measures** with acceptable safety and tolerability, supporting Phase 3 evaluation[2-6]

Representative Mechanistic Meal Challenge Study[1]

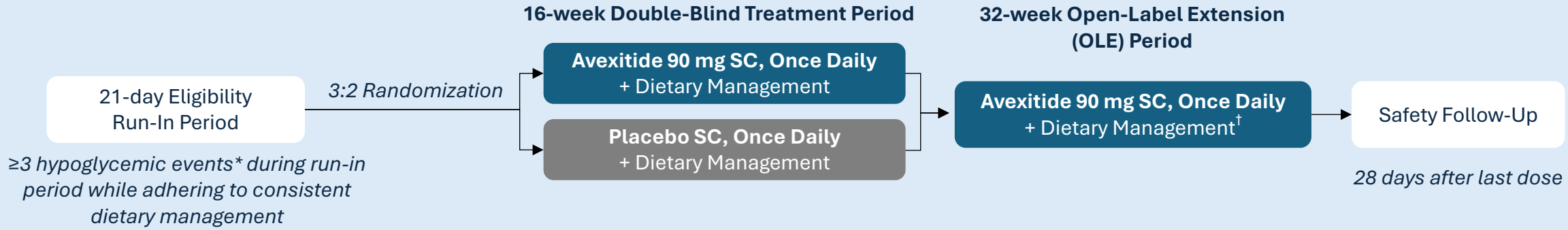


AVX, avexitide; PBH, post-bariatric hypoglycemia

1. Salehi M, et al. *Gastroenterology*. 2014;146(3):669-680.e2. **2.** Craig CM, et al. *Diabetologia*. 2017;60(3):531-540. **3.** Craig CM, et al. *Diabetes Obes Metab*. 2018;20(2):352-361. **4.** Tan M, et al. *Diabetes Obes Metab*. 2020;22(8): 1406-1416. **5.** Craig CM, et al. *J Clin Endocrinol Metab*. 2021;106(8):e3235-e3248. **6.** Craig C, et al. *Metabolism*. 2026;184:156773. Graphs adapted from Salehi M, et al. *Gastroenterology*. 2014;146(3):669-680.e2.

Phase 3 LUCIDITY Trial Design

Multicenter, randomized, double-blind, placebo-controlled trial



KEY TRIAL ENTRY CRITERIA

- History of RYGB ≥12 months prior to screening
- Clinical diagnosis of PBH
- No current or recent use (within five drug-specific half-lives) of drugs that alter glucose metabolism or promote weight loss (eg, semaglutide, acarbose)

DIETARY MANAGEMENT

- Written dietary guidance and training provided at screening
- Guidance reviewed and reinforced by site personnel at each study visit
- Dietary recommendations included ≤30 g carbohydrate/meal and ≤15 g/snack, avoidance of simple/high-GI carbohydrates, and adequate protein intake

RYGB, Roux-en-Y gastric bypass; SC, subcutaneous; EAC, Event Adjudication Committee; SMBG, self-monitoring of blood glucose; OLE, open-label extension
*At least one event had to be adjudicated as level 3 by the independent Event Adjudication Committee (EAC), while the remaining two or more events could be documented as level 2 by SMBG or adjudicated as Level 3
†For the first 8 weeks of the OLE, participants were instructed to follow the same dietary guidance as the run-in and double-blind periods

LUCIDITY Assessed Hypoglycemia Using Multiple Complementary Measures

Efficacy Endpoints

All assessed during the 16-week double blind period

Primary Efficacy Endpoint[1]

- Composite rate of SMBG-confirmed level 2 and blinded EAC-adjudicated level 3 hypoglycemic events

Secondary Efficacy Endpoints[1]

- Level 2 hypoglycemic events (SMBG)*
- Level 3 hypoglycemic events (EAC)*
- Level 2 hypoglycemic events (CGM)*
- % change from baseline in composite level 2/3 event rate

HYPOGLYCEMIC EVENT DEFINITIONS[1]

Level 2: Glucose <54 mg/dL†

Threshold at which neuroglycopenic symptoms begin to occur

Level 3: Hypoglycemic event characterized by altered mental and/or physical function requiring external assistance for recovery

Adjudication by a blinded, independent Event Adjudication Committee

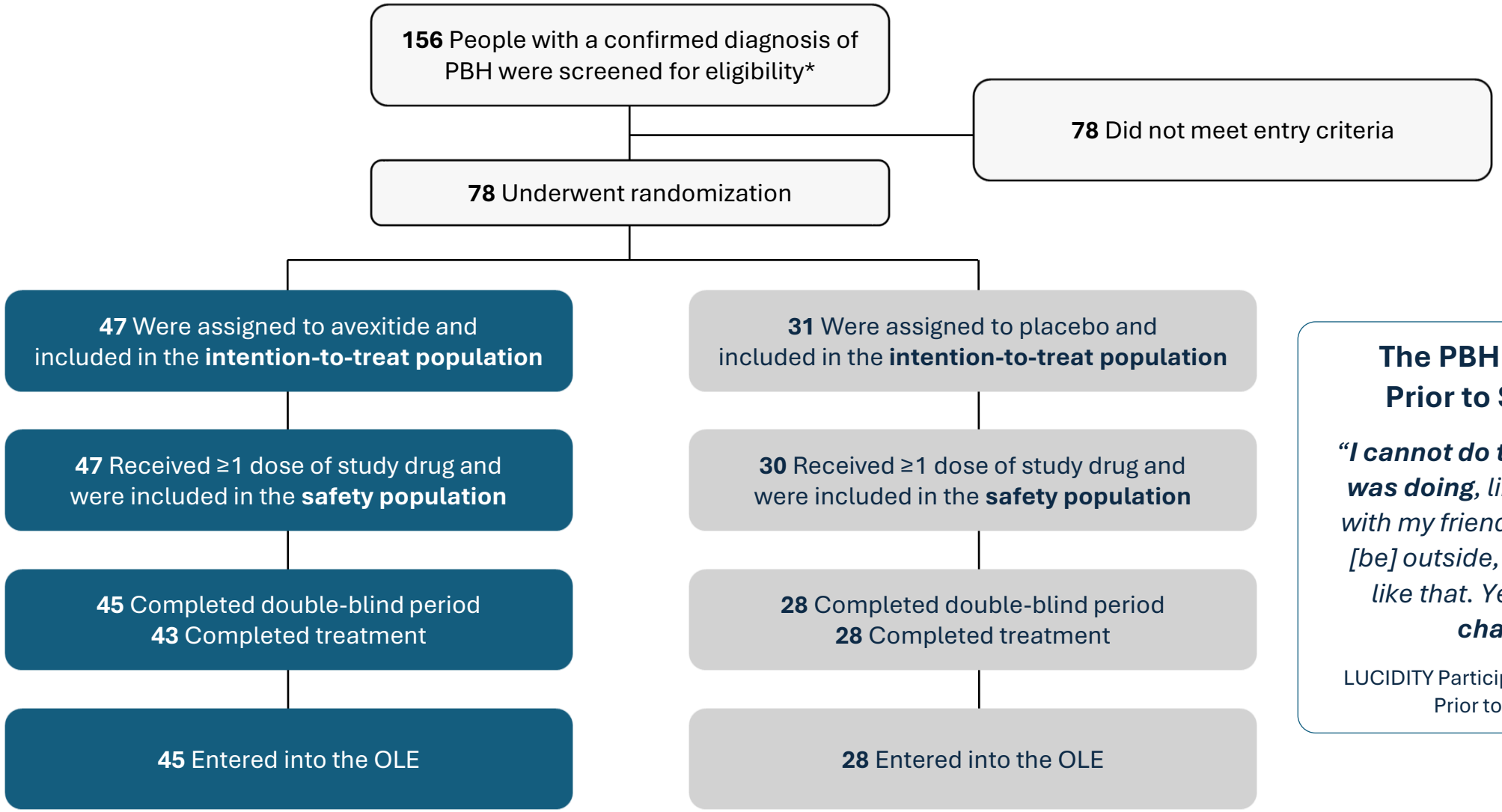
SMBG, self-monitoring of blood glucose; EAC, Event Adjudication Committee; CGM, continuous glucose monitoring

*Event-rate endpoints were multiplicity-controlled and tested hierarchically in the order listed

†SMBG: glucose <54 mg/dL; CGM: glucose <54 mg/dL for at least 15 minutes

1. ADA Professional Practice Committee. *Diabetes Care*. 2026;49(Suppl 1):S132-S149.

>90% of Participants Completed the Double-Blind Treatment Period; All Completers Continued into the OLE



The PBH Experience Prior to Study Entry

*"**I cannot do the same things I was doing**, like go out, like be with my friends, drink a coffee, [be] outside, go hiking, things like that. Yeah, **all my life changed.**"*

LUCIDITY Participant Asked About Life Prior to Study Entry

OLE, Open-label extension period
*Participants who failed initial screening could be re-screened once, for a maximum of two screening attempts
Quote from qualitative interviews performed in subgroup of LUCIDITY participants at Week 16 when asked to describe the hypoglycemic events they experienced before entering LUCIDITY and the impact of PBH on their daily life.

Baseline Characteristics Were Generally Balanced Between Groups

	Avexitide (n=47)	Placebo (n=31)	Overall (N=78)
DEMOGRAPHICS			
• Age (years)	50.5 ± 9.7	51.3 ± 10.0	50.8 ± 9.8
• Female – no. (%)	45 (96)	30 (97)	75 (96)
• BMI (kg/m^2)	29.0 ± 4.3	27.1 ± 3.6	28.3 ± 4.1
• Weight loss since surgery (kg)*	45.2 ± 21.5	44.7 ± 19.2	45.0 ± 20.5
PBH HISTORY			
• Months since RYGB	136.1 ± 92.1	111.1 ± 87.5	126.2 ± 90.6
• Months since first experience of postprandial hypoglycemia†	101.4 ± 84.7	73.8 ± 71.2	90.5 ± 80.3
• Months since PBH diagnosis	68.3 ± 76.5	49.3 ± 57.9	60.7 ± 69.9
• History of treatment for PBH – no. (%)	39 (85)	29 (94)	68 (88)
DISEASE BURDEN			
• Daily hypoglycemia symptoms‡ – no. (%)	27 (64)	21 (72)	48 (68)
• History of loss of consciousness§ – no. (%)	20 (44)	15 (52)	35 (47)
• Prior PBH-related hospitalization§ – no. (%)	12 (26)	9 (31)	21 (28)
MEAN WEEKLY HYPOGLYCEMIC EVENT RATES (RUN-IN PERIOD)			
• Composite level 2 by SMBG / level 3 by EAC	2.15 ± 1.73	2.75 ± 1.56	2.39 ± 1.68
• Level 2 by SMBG	1.27 ± 1.74	1.27 ± 0.90	1.27 ± 1.46
• Level 3 by EAC	1.58 ± 1.06	2.46 ± 1.65	1.93 ± 1.38
• Level 2 by CGM	3.83 ± 3.89	2.96 ± 2.07	3.48 ± 3.30

Plus/minus values are means ± SD

The PBH Experience Prior to Study Entry

"I think just the burden of my husband having to babysit me, take me to all my appointments, drive me everywhere. ***It feels like [being] an invalid sometimes to have somebody have to care for me all the time."***

LUCIDITY Participant Asked About Life Prior to Study Entry

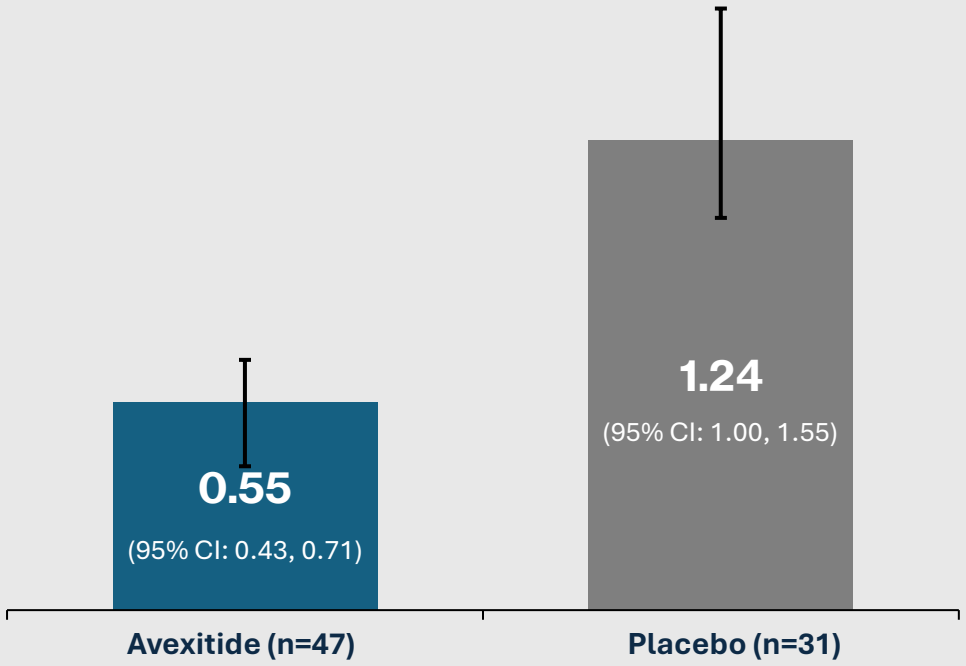
BMI, body mass index; SD, standard deviation
*Data available for 43 participants in the avexitide group and 28 participants in the placebo group
†Data available for 46 participants in the avexitide group and 30 participants in the placebo group
Quote from qualitative interviews performed in subgroup of LUCIDITY participants at Week 16 when asked to describe the hypoglycemic events they experienced before entering LUCIDITY and the impact of PBH on their daily life

‡Data available for 42 participants in the avexitide group and 29 participants in the placebo group
§ Data available for 46 participants in the avexitide group and 29 participants in the placebo group

Primary Endpoint Met: Avexitide Reduced the Composite Level 2/3 Hypoglycemic Event Rate by 55% vs Placebo

Mean Weekly Composite Level 2/3 Events (Model-Adjusted)

Double-blind treatment period (ITT, N=78)



Prespecified primary analysis: *Negative binomial regression adjusted for baseline composite event rate, with multiple imputation for missing follow-up time due to early study withdrawal. Bars show LS mean event rates (95% CI). Results use the treatment policy estimand.*

ITT, intent-to-treat; LS, least squares

PRIMARY ENDPOINT

55% Reduction in composite Level 2/3 hypoglycemic event rate vs placebo

Rate ratio: 0.45 (95% CI: 0.32-0.63)

***p* = 0.0000034**

Results consistent across prespecified sensitivity analyses and evaluated prespecified subgroups

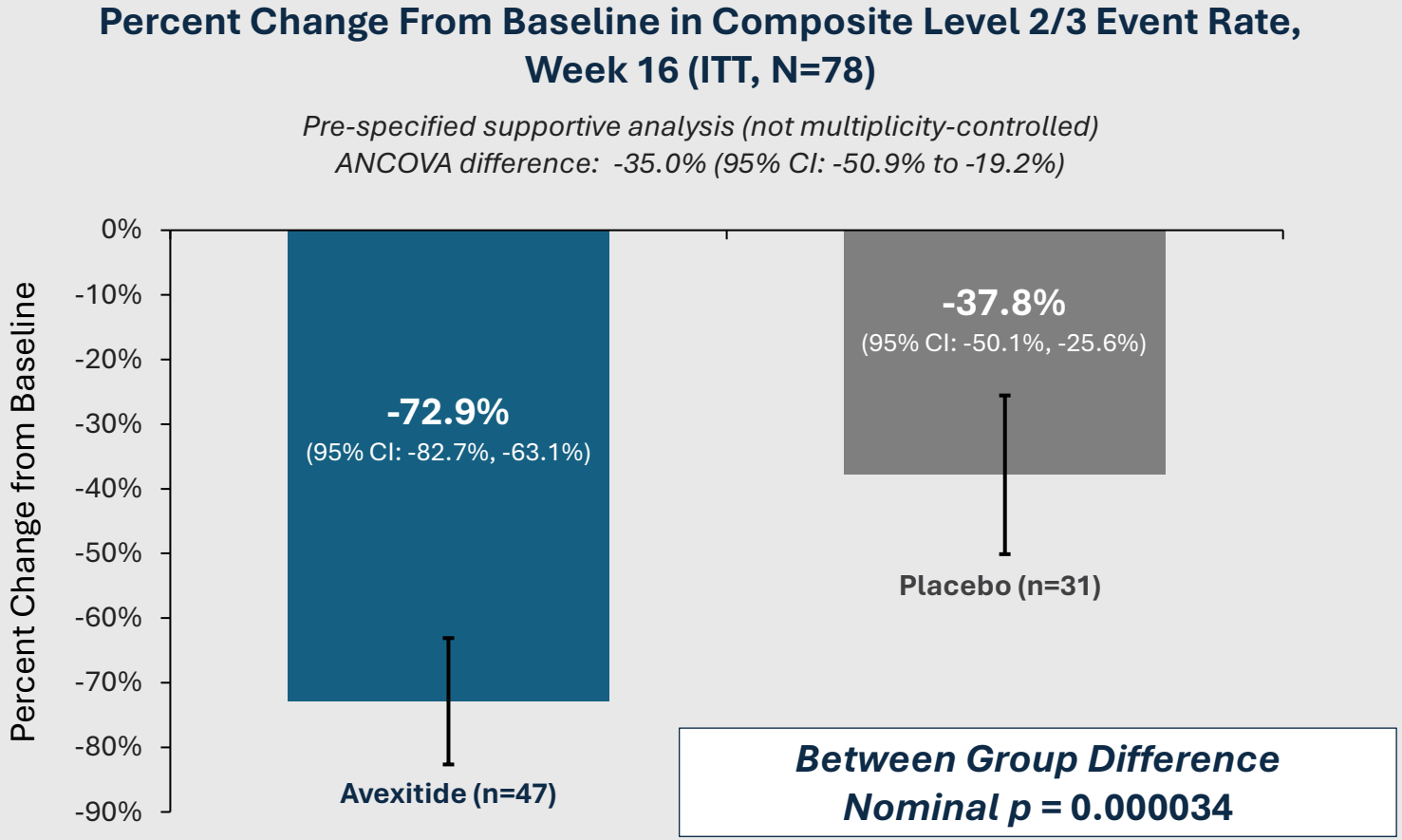
Sensitivity Analyses:

- Alternative missing-data handling
- Alternative estimation methods
- On-treatment estimand
- Alternative modeling of baseline event rate
- Alternative handling of adjudication-discordant level 3 events

Subgroup Analyses:

- Age
- Sex
- Race and ethnicity
- Body mass index

Avexitide Demonstrated Greater Reductions From Baseline in Composite Level 2/3 Hypoglycemic Event Rate Than Placebo at Week 16



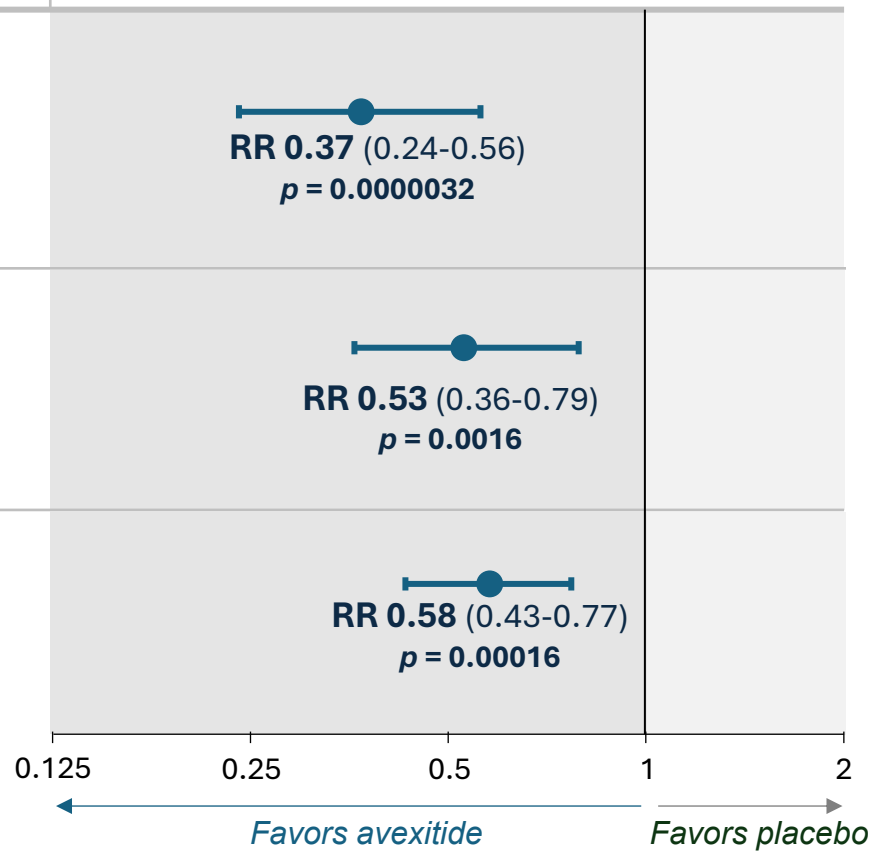
Study Factors That May Help to Explain Changes in the Placebo Group

- Regression to the mean
- Structured dietary management with ongoing reinforcement
- Increased monitoring, including CGM, and access to a healthcare team

ANCOVA (analysis of covariance) of percent change from baseline, adjusted for baseline (run-in) event rate; estimate represents the LS mean difference LS, least squares

All Prespecified, Multiplicity-Controlled Secondary Endpoints Met With Robust Statistical Significance

	PERCENT REDUCTION Mean weekly event rate over double-blind period (model-adjusted, ITT, N=78)	**RATE RATIO (95% CI)** Log scale
Level 2 (SMBG)	**63% Reduction vs Placebo** **Avexitide: 0.24** | Placebo: 0.66	**RR 0.37** (0.24-0.56) ***p* = 0.0000032**
Level 3 (EAC)	**47% Reduction vs Placebo** **Avexitide: 0.46** | Placebo: 0.87	**RR 0.53** (0.36-0.79) ***p* = 0.0016**
Level 2 (CGM)	**42% Reduction vs Placebo** **Avexitide: 1.72** | Placebo: 2.99	**RR 0.58** (0.43-0.77) ***p* = 0.00016**



Clinically meaningful and consistent reductions across all individual endpoint components and concordant SMBG- and CGM-based assessments supported the primary endpoint result

Rate ratios used a negative binomial model adjusted for baseline event rate and accounting for missing data due to loss to follow-up; event rates shown are LS means (model-adjusted)
RR, rate ratio; LS, least squares

TEAEs Were Primarily Gastrointestinal and Injection-Site Related and Generally Mild to Moderate

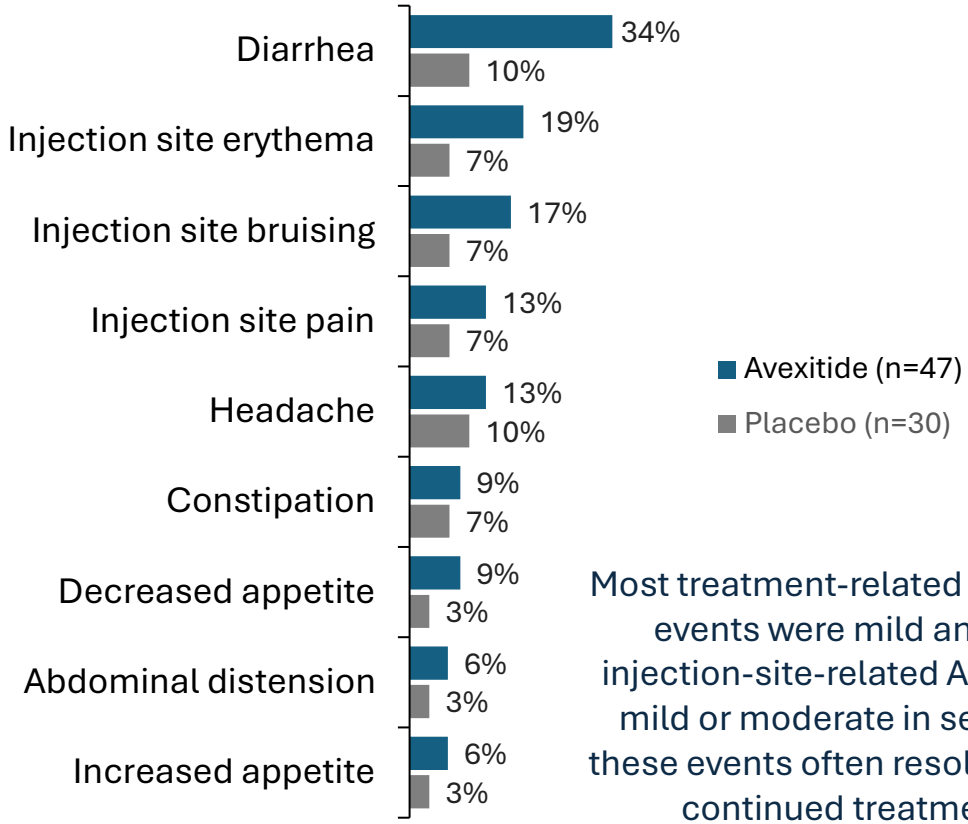
Summary of Treatment-Emergent Adverse Events (TEAEs)

(Participants with ≥1 event, n (%), double-blind period, safety population, N=77)

	AVEXITIDE (n=47)	PLACEBO (n=30)
TEAE	39 (83)	19 (63)
Drug-related TEAE	27 (57)	14 (47)
Treatment-emergent SAE	1 (2)	1 (3)
Related to study drug	0	0
TEAE leading to discontinuation	2 (4)	1 (3)
Related to study drug	1 (2)	1 (3)
Deaths	0	0

Most Common TEAEs

≥5% overall and more frequent with avexitide



Most treatment-related diarrhea events were mild and all injection-site-related AEs were mild or moderate in severity; these events often resolved with continued treatment

AE, adverse event; SAE, serious adverse event

No Clinically Meaningful Changes in Body Weight or Fasting Plasma Glucose Through Week 16

Body Weight Summary

kg, mean ± SD

	Avexitide	Placebo
Baseline	79.4 ± 15.2	71.2 ± 9.4
Week 16	80.0 ± 16.1	72.6 ± 10.6
Change from Baseline at Week 16	**+0.8 ± 3.7**	**+1.5 ± 3.2**

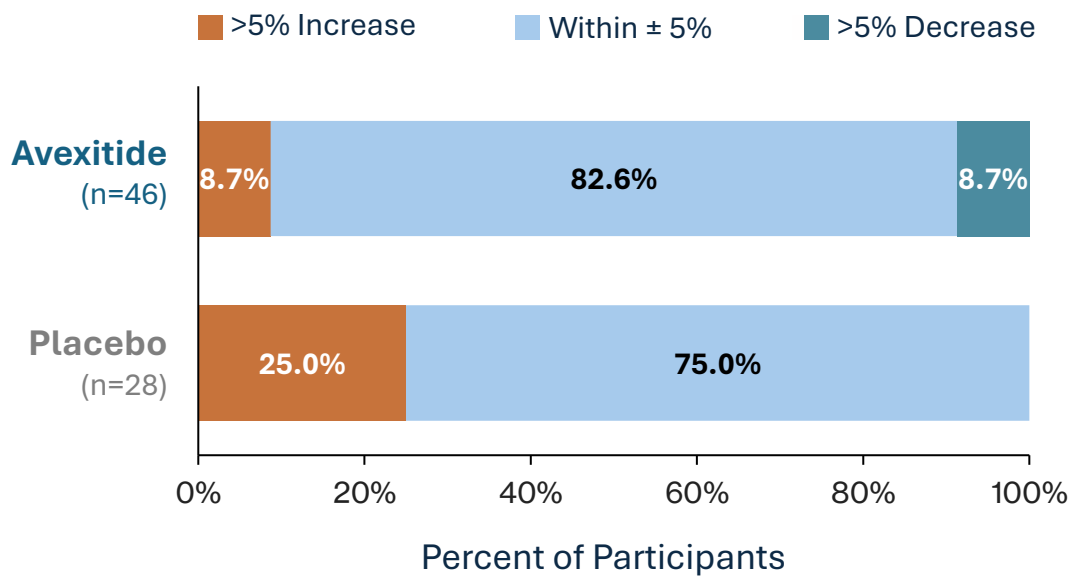
Fasting Plasma Glucose Summary

mg/dL, mean ± SD

	Avexitide	Placebo
Baseline	87.9 ± 11.4	85.3 ± 7.2
Week 16	91.1 ± 12.0	87.7 ± 8.8
Change from Baseline at Week 16	**+3.1 ± 12.7**	**+2.8 ± 9.2**

Distribution of Body Weight Change

From Baseline to Week 16



At Week 16, **91.3% (42/46) of avexitide-treated participants had body weight within 5% of baseline or lower** vs. 75% (21/28) of placebo-treated participants

SD, standard deviation
Sample sizes, n (baseline / Week 16 / change):
Weight: avexitide 47 / 46 / 46, placebo 30 / 28 / 28
Fasting plasma glucose: avexitide 47 / 45 / 45, placebo 29 / 26 / 25
n = participants with non-missing data. Change from baseline includes only participants with non-missing values at both baseline and Week 16

Key Conclusions

STUDY FINDINGS

1. **Avexitide, a GLP-1 receptor antagonist, reduced composite level 2/3 hypoglycemic event rates by 55% versus placebo (*p* = 0.0000034),** meeting the primary efficacy endpoint
2. **Consistent reductions across SMBG-confirmed level 2, adjudicated level 3, and CGM-detected level 2 events** supported the primary result
3. **TEAEs were primarily gastrointestinal and injection-site related, generally mild to moderate, and often resolved with continued treatment;** body weight and fasting plasma glucose remained stable

CLINICAL AND SCIENTIFIC IMPLICATIONS

1. **LUCIDITY represents a landmark study in PBH,** demonstrating clinically meaningful reductions in hypoglycemia with avexitide
2. The **findings are consistent with five prior avexitide studies,** supporting a reproducible efficacy and safety profile across the clinical program
3. **LUCIDITY establishes a rigorous framework for PBH clinical trials** using SMBG, CGM, and adjudicated Level 3 hypoglycemia endpoints
4. The results **further support GLP-1 receptor signaling as a central driver of PBH pathophysiology**
5. Together, these findings **advance both the understanding of PBH and the development of targeted therapies**

Q&A



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Closing Remarks

Camille Bedrosian, MD

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Advancing Toward a Potential First Approved Therapy for PBH



Significant Unmet Need



Compelling Clinical Profile



NDA Submission on Track for Year-end 2026



Executing on Launch Preparations