

# 43<sup>rd</sup> Annual JP Morgan Healthcare Conference

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Chief Executive Officer



# Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements other than statements of historical fact and can be identified by terms such as “may,” “will,” “should,” “could,” “would,” “expects,” “plans,” “anticipates,” “believes,” “estimates,” “projects,” “predicts,” “outlook,” “potential” and similar expressions (including the negative thereof) intended to identify forward-looking statements. Forward-looking statements contained in this presentation, include, but are not limited to, statements about: (i) our business strategy, objectives and opportunities; (ii) plans for, including timing, development and progress of commercialization or regulatory timelines for, NUPLAZID, DAYBUE and our product candidates; (iii) benefits to be derived from and efficacy of our products, including the potential advantages of NUPLAZID and DAYBUE and expansion opportunities for NUPLAZID and DAYBUE in other indications, and for DAYBUE in jurisdictions outside the U.S. and Canada; (iv) estimates regarding the prevalence of the diseases targeted by our products and product candidates; (v) potential markets for any of our commercial products; and (vi) our estimates regarding our future financial performance, cash position, profitability or capital requirements.

Forward-looking statements are subject to known and unknown risks, uncertainties, assumptions and other factors that may cause our actual results, performance or achievements to differ materially and adversely from those anticipated or implied by our forward-looking statements. Such risks, uncertainties and other factors include, but are not limited to: our dependency on the continued successful commercialization of NUPLAZID and DAYBUE and our ability maintain or increase sales of NUPLAZID or DAYBUE; the costs of our commercialization plans and development programs, and the financial impact or revenues from any commercialization we undertake; our ability to obtain necessary regulatory approvals for our product candidates and, if and when approved, market acceptance of our products; our dependence on third-party collaborators, clinical research organizations, manufacturers, suppliers and distributors; the impact of competitive products and therapies; our ability to generate or obtain the necessary capital to fund our operations; our ability to grow, equip and train our specialized sales forces; our ability to manage the growth and complexity of our organization; our ability to maintain, protect and enhance our intellectual property; and our ability to continue to stay in compliance with applicable laws and regulations. Given the risks and uncertainties, you should not place undue reliance on these forward-looking statements. For a discussion of these and other risks, uncertainties and other factors that may cause our actual results, performance or achievements to differ, please refer to our annual report on Form 10-K for the year ended December 31, 2023 as well as our subsequent filings with the Securities and Exchange Commission from time to time, including our quarterly report on Form 10-Q for the period ended September 30, 2024. The forward-looking statements contained herein are made as of the date hereof, and we undertake no obligation to update them after this date, except as required by law.

This presentation also contains estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. Projections, assumptions and estimates of the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk. The trademarks included herein are the property of the owners thereof and are used for reference purposes only.

# Announced Today

2025 expected to be first full year of **\$1 billion+ revenue**

**Filing of DAYBUE MAA with EMA** with expected approval in Q1 2026

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**Anticipated timelines** for last patient in and top line results for:

**ACP-101** Phase 3 study in Prader-Willi Syndrome

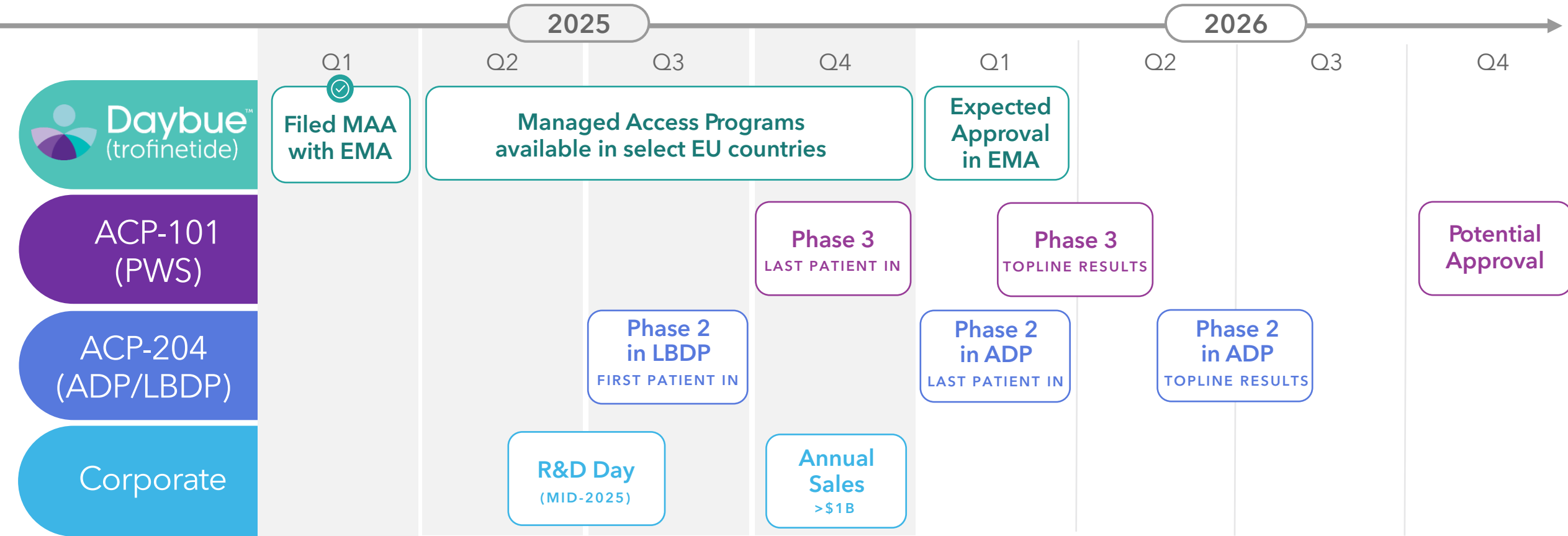
**ACP-204** Phase 2 study in Alzheimer's Disease Psychosis

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Plans to initiate a **Phase 2 of ACP-204** in Lewy Body Dementia with Psychosis

Plans to host **first R&D day in company history** in mid-2025

# 2025-2026 Anticipated Milestones



Two late-stage pipeline programs anticipated to have topline results in 2026

# Building Long Term Growth in CNS & Rare Disease

## Core Franchise

## Core Pipeline

## Expansion Areas

Neuro Psych

ONCE-DAILY  
**NUPLAZID**<sup>®</sup>  
(pimavanserin) 34mg capsules

**ADP**  
ACP-204  
(new 5-HT2A)

**LBDP**  
ACP-204  
(new 5-HT2A)

**Essential Tremor**  
ACP-711  
(selective GABA<sub>A</sub>-α3  
modulator)

**TRD/MDD/Other**  
ACP-211  
(NMDA receptor antagonist)

Neuro Rare

 **Daybue**<sup>™</sup>  
(trofinetide)

**PWS**  
ACP-101  
(Intranasal carbetocin)

**Rett/Fragile X**  
ACP-2591  
(cGP analogue)

**Expansion to other Rare Disease  
areas under evaluation**

Endocrine      Nephrology      Cardiovascular  
Metabolic      Immunology





# Neuropsychiatric Franchise

# Disease Awareness Campaign





# Overview of Parkinson's Related Hallucinations and Delusions

**~1M patients with Parkinson's Disease (PD) in U.S.**

Around 50% may develop hallucinations and/or delusions at some point during course of their disease<sup>1</sup>

## SYMPTOMS

- ▶ Seeing, hearing or experiencing things that others don't
- ▶ Believing things that are not true

## Low awareness

Market research indicated that at the start of 2024 less than 10% of caregivers and patients were aware that hallucinations and delusions are associated with PD<sup>2</sup>

**~130,000 PD patients**

are treated with an atypical antipsychotic annually<sup>3</sup>

<sup>1</sup> Elin B Forsaa, et al. A 12-year population-based study of psychosis in Parkinson disease *Arch. Neurol.* 2010; Aug;67(8):996-1001

<sup>2</sup> Source: Acadia confidential market research

<sup>3</sup> Acadia estimate as of June 2024 based on claims data



# NUPLAZID for the Treatment of Parkinson's Related Hallucinations and Delusions

**NUPLAZID is the first and only FDA-approved drug for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis.**



ONCE-DAILY  
**NUPLAZID**<sup>®</sup>  
(pimavanserin) 34mg capsules

## CLINICALLY PROVEN

Over 82,000 patients treated

Most studied Rx in PD-Psychosis, with no negative impact on motor function or cognition<sup>1</sup>

## GROWTH ACCELERATING

~10% YTD net sales growth Q1-Q3 in 2024

DTC campaign driving increased referrals in Q4 2024

## OPPORTUNITY REMAINS

~20% share of 130,000 patients on atypical antipsychotics

Composition of matter to Oct. 2030; formulation to Feb. 2038

**ANTICIPATED >\$325M IN INCREASING ANNUAL CASH FLOW FUELING CORPORATE GROWTH**

# NUPLAZID Commercial Strategy & Outlook



## Activate Consumers

Pull through interested consumers from branded DTC campaign



## Drive Market Share

Further leverage real world evidence to drive prescriber decisions



## Maximize field force efficiency

Leverage AI and data to call on the right prescribers at the right time

### NUPLAZID 2025 OUTLOOK

Accelerating sales growth

Increasing market share

Additional data publications

Additional real world evidence out to 5 years



# DAYBUE





# Overview of Rett Syndrome

## Typically caused by mutations in the MECP2 gene

disrupting the function of MECP2 protein crucial for brain development and function

## Debilitating Symptoms of Rett Syndrome<sup>1</sup>

- ▶ Fine and gross motor impairment
- ▶ Loss of independence and require 24/7 support
- ▶ Loss of verbal and nonverbal communication
- ▶ GI symptoms including severe constipation
- ▶ Hand stereotypies
- ▶ Seizures

**~5,500 - 5,800 diagnosed patients in U.S.<sup>2</sup>**

with a prevalent population of ~6,000 - 9,000

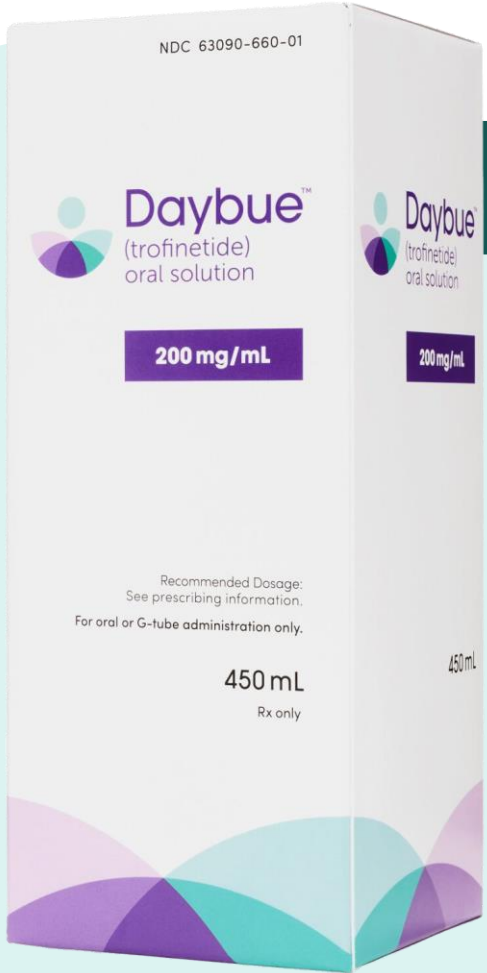
**~9,000 - 12,000 prevalent population in EU<sup>3</sup>**

<sup>1</sup> Acadia market research, Neul JL et al, Annal Neurol. 2010;68;944-50 and <https://www.rettsyndrome.org/about-rett-syndrome/what-is-rett-syndrome/>.

<sup>2</sup> Based on Acadia Pharmaceuticals analysis of claims data as of 2024 claims data.

<sup>3</sup> Based on Acadia internal estimates.

# DAYBUE for the Treatment of Rett Syndrome



**DAYBUE is the first and only FDA-approved drug for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older.**

## GROWING CLINICAL EXPERIENCE      FAVORABLE ACCESS ENVIRONMENT      EVOLVING PATIENT EXPERIENCE

Over 1,600 patients treated to date

66% of active patients have been on treatment  $\geq 10$  months

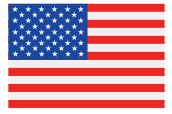
90% of DAYBUE families pay <\$10 per month

Management of common adverse events (diarrhea and vomiting) further informed by real world experience and engagement



**EMA FILING SUBMITTED AND EU MANAGED ACCESS PROGRAMS AVAILABLE IN 2025**

# DAYBUE Commercial Strategy & Outlook



## Deepening clinical experience and increasing awareness

**UNITED STATES** | Est 6,000-9,000 patients

- ▶ Substantially increasing our field force and use of predictive analytics
- ▶ Launching branded Direct-to-Consumer campaigns
- ▶ Omni-channel strategy to bring DAYBUE clinical data to life



## Pursuing targeted filings and launches

**EUROPEAN UNION** | Est 9,000-12,000 patients

Building out EU launch team for potential Q1:2026 approval

**JAPAN** | Est 1,000-2,000 patients

PMDA discussions ongoing; study start anticipated by Q3:2025

**CANADA** | Est 600-900 patients

Approval granted; first sales anticipated in Q3:2025

## DAYBUE 2025 OUTLOOK

Increasing new patient growth in the U.S.

Maintaining stable persistency

Initial revenues from Managed Access Programs in select EU countries

Growing body of real world experience, including updated data from LOTUS study



# Pipeline



# Deep and Diverse Pipeline Across CNS and Rare Disease



PROGRAM	INDICATION	MECHANISM OF ACTION	DISCOVERY	IND ENABLING	PHASE 1	PHASE 2	PHASE 3	LAUNCHED
<b>CNS</b>								
NUPLAZID	Parkinson's Disease Psychosis	5HT2A inverse agonist						
ACP-204	Alzheimer's Disease Psychosis	New 5HT2A inverse agonist						
ACP-204	Lewy Body Dementia w/ Psychosis	New 5HT2A inverse agonist						
ACP-711	Essential Tremor	Selective GABA <sub>A</sub> -α3 modulator						
<b>RARE DISEASE</b>								
DAYBUE	Rett Syndrome	Analogue of GPE						
ACP-101	Hyperphagia in Prader-Willi Syndrome	Intranasal Carbetocin						
ACP-2591	Rett Syndrome; Fragile X Syndrome	cGP analogue						
STOKE ASO 1	Rett Syndrome	Antisense oligonucleotide (ASO)						
STOKE ASO 2	SYNGAP1	Antisense oligonucleotide (ASO)						
STOKE ASO 3	Not disclosed	Antisense oligonucleotide (ASO)						
<b>CNS/RARE DISEASE</b>								
ACP-211	TRD/MDD/Other	NMDA receptor antagonist						
ACP-271	Neurology	GPR88 agonist						

**+ Multiple undisclosed discovery programs in CNS and rare disease**

# Pipeline Program Spotlights

PROGRAM	INDICATION	MECHANISM OF ACTION	DISCOVERY	IND ENABLING	PHASE 1	PHASE 2	PHASE 3	LAUNCHED
<b>CNS</b>								
NUPLAZID	PDP	5HT <sub>2A</sub>						
ACP-204	Alzheimer's Disease Psychosis	New 5HT <sub>2A</sub> inverse agonist						
ACP-204	Lewy Body Dementia w/ Psychosis	New 5HT <sub>2A</sub> inverse agonist						
ACP-711	Essential Tremor	Selective GABA <sub>A</sub> -α3 modulator						
<b>RARE DISEASE</b>								
DAYBUE	Rett Syndrome	Analogue of GPE						
ACP-101	Hyperphagia in Prader-Willi Syndrome	Intranasal Carbetocin						
ACP-2591	Rett Syndrome; Fragile X Syndrome	cGP analogue						
STOKE ASO 1	Rett Syndrome	Antisense oligonucleotide (ASO)						
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ACP-211	TRD/MDD/Other	NMDA receptor antagonist						
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**+ Multiple undisclosed discovery programs in CNS and rare disease**





# ACP-101 in Prader-Willi Syndrome



# Prader-Willi Syndrome (PWS)

Rare genetic disorder caused by a deletion or mutation on chromosome 15

Typically diagnosed during infancy based on characteristic lack of muscle tone

Complex neurobehavioral disease most often managed by pediatric endocrinologists

**Hyperphagia is a defining characteristic of PWS distinguished by unrelenting hunger**

due to impaired neural response to food intake and inability to regulate food intake in line with energy needs

**~30 years average life expectancy<sup>1</sup>**

with obesity and obesity-related complications leading to mortality

**Affecting ~8,000 - 10,000 patients in the U.S .**

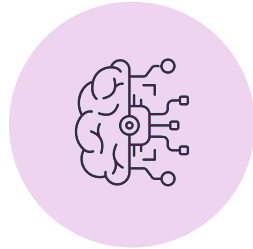
<sup>1</sup> Causes of Death in Prader-Willi Syndrome: Prader-Willi Syndrome Association (USA) 40-Year Mortality Survey. Genet Med. 2017. June; 19(6): 635-642.

# ACP-101

## Intranasal Carbetocin



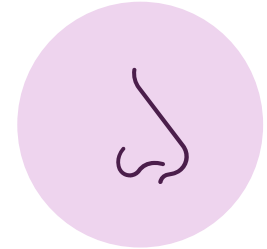
ACP-101 (carbetocin) is a long-acting **analogue of human oxytocin**



**Selectively binds to oxytocin** receptors (more so than oxytocin itself)



Intended to **overcome the functional deficit in oxytocin receptor agonism** in Prader Willi Syndrome



**Drug-device combination** product for **intranasal** administration

# COMPASS-PWS Phase 3 Study

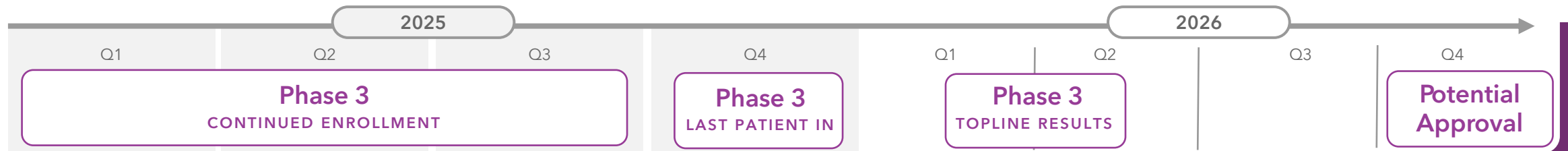
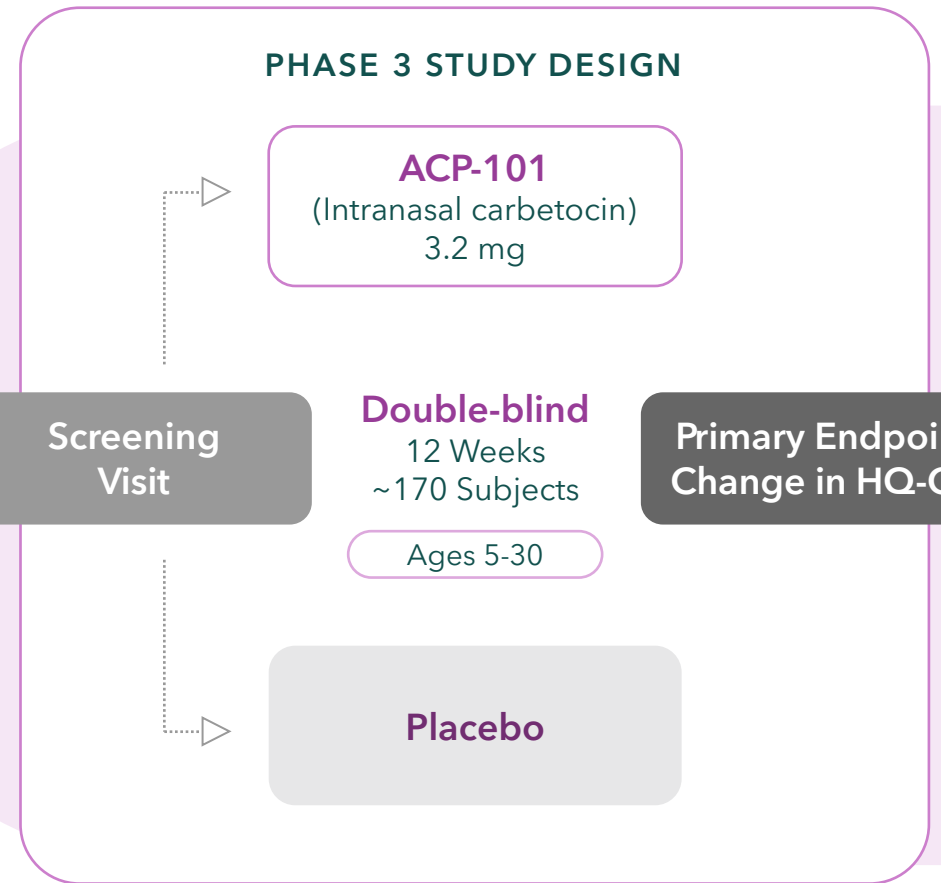
Global, placebo-controlled, double-blind Phase 3 enrolling



## Trial design builds on previous Phase 3 clinical trial experience

3.2 mg dose was observed to reduce hyperphagia-related behaviors

- ▶ Q4:2025: COMPASS PWS last patient in expected
- ▶ Q1:2026: COMPASS PWS topline results expected
- ▶ Q4:2026: Potential approval



<sup>1</sup> Hyperphagia Questionnaire for Clinical Trials (HQ-CT) is an observer-reported outcome measure that has been widely used in interventional studies to assess changes in hyperphagia behaviors individuals with PWS.





# ACP-204 Development Plans

# Alzheimer's Disease Psychosis (ADP)

**Approximately 30% of patients with Alzheimer's disease experience psychosis** commonly consisting of hallucinations and delusions<sup>1</sup>.

**There are no approved treatments** for hallucinations and delusions associated with Alzheimer's disease psychosis.

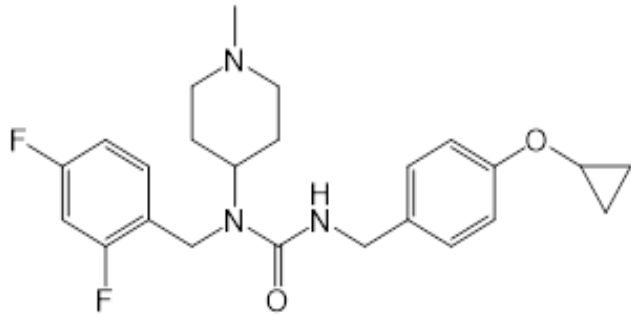
**Affecting 800,000 to 850,00 Alzheimer's patients in U.S.** currently treated with antipsychotics, antidepressants or mood stabilizers<sup>2</sup>.

<sup>1</sup> Cummings J. et al. Criteria for Psychosis in Major and Mild Neurocognitive Disorders: International Psychogeriatric Associations (IPA) Consensus Clinical and Research Definition. Am J of Geriatric Psychiatry. 2020; 28 (12); 1256-1269

<sup>2</sup> Based on Acadia internal estimates.

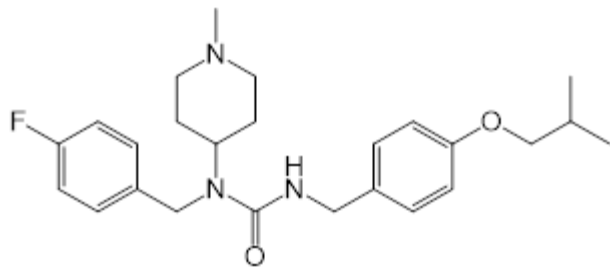
# ACP-204 Builds Upon but is Structurally Distinct from Pimavanserin

ACP-204



ACP-204 FEATURES A COMBINATION OF STRUCTURAL CHANGES VS PIMAVANSERIN, AND REDUCED OFF-TARGET EFFECTS ALONG WITH EQUAL OR INCREASED POTENCY

Pimavanserin



## ACP-204 TARGET PRODUCT PROFILE

Mitigate or eliminate QT prolongation

Enable doses higher than pimavanserin 34 mg equivalent

Improve time to onset of action

## RESULTS TO DATE

Planned publications [throughout 2025](#)



- >200 patients received ACP-204 in Phase 1 trials
- lower hERG vs. pimavanserin; No signs of QT prolongation
- Wide dose range established supporting potential for ~2x pimavanserin 34 mg equivalent
- Steady state PK achieved in 5 days vs pimavanserin 12 days

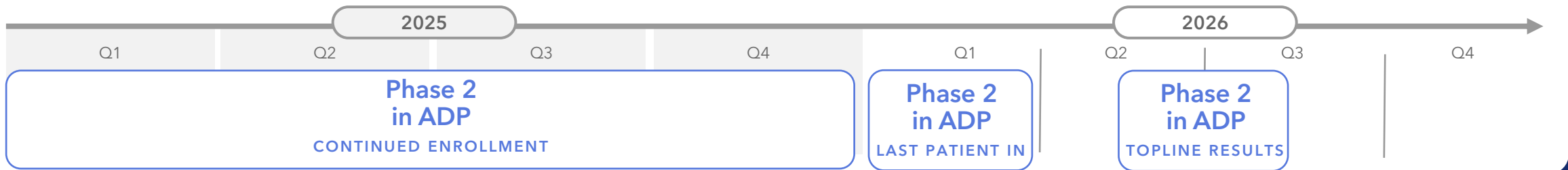
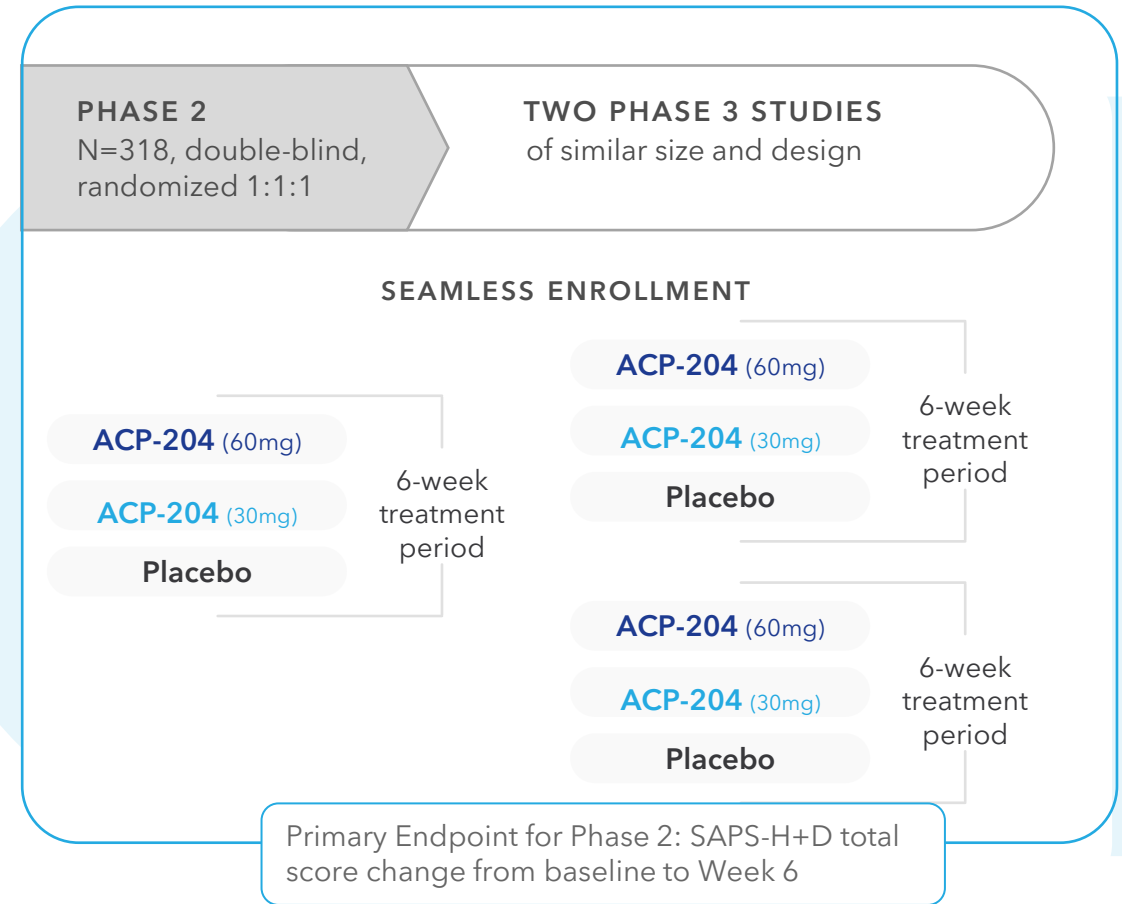
# RADIANT Phase 2 / Phase 3

Global, placebo-controlled, double-blind Phase 2 enrolling



## Pimavanserin experience supports P2/P3 program

- ▶ Q1:2026: Phase 2 in ADP last patient in expected
- ▶ Mid-2026: Phase 2 in ADP topline results expected





# Lewy Body Dementia (LBD)



**LBD is a progressive brain disorder that affects thinking, movement, mood, and behavior**

**Associated with abnormal deposits of alpha-synuclein in the brain<sup>1</sup>**

**No therapies approved for LBD with psychosis**  
and some traditional antipsychotics that are commonly used in other diseases can be harmful

**>1 million people in the U.S may be living with LBD;  
50% -75% of people with LBD experience psychosis (LBDP)<sup>2</sup>**

▶ Approximately 200,000 patients living with LBD are being treated with antipsychotics<sup>3</sup>

<sup>1</sup> Simuni T, Chahine LM, Poston K, et al. A biological definition of neuronal  $\alpha$ -synuclein disease: towards an integrated staging system for research. *Lancet Neurol.* 2024 Feb;23(2):178-190. doi: 10.1016/S1474-4422(23)00405-2. PMID: 38267190.

<sup>2</sup> Cummings et al 2018. <sup>3</sup> Based on IQVIA data and Acadia internal estimates.

# Plan to Initiate Phase 2 Study in **LBDP** in Q3 2025

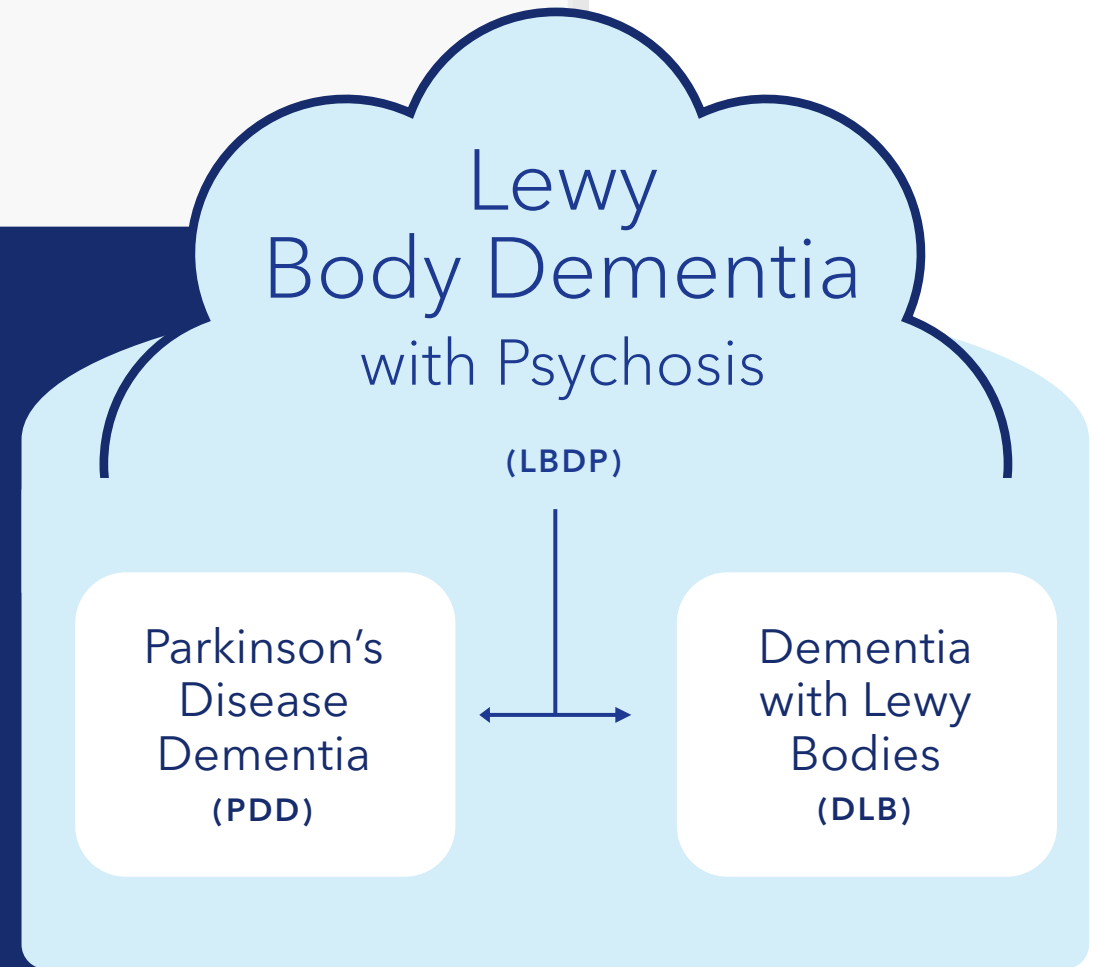
Phase 2 program will enroll both PDD and DLB patients with psychosis

Relapse data from pimavanserin withdrawal study suggest potential utility of targeting 5HT2A

% of patients experiencing relapse:

- 5.3% (1 of 19) patients receiving pimavanserin
- 55.0% (11 of 20) receiving placebo

Alpha-synuclein and other biomarkers will be evaluated to characterize patient population





# ACP-711 for Essential Tremor

# Essential Tremor



**Shaking or trembling movements of the hands (and beyond)<sup>1</sup>**

▶ Can be debilitating, with significant impact on quality of life<sup>2</sup>

**Associated with physical and cognitive impairments, social avoidance, and other challenges that significantly impact patients' lives<sup>2-3</sup>**

**~ 7 million people affected in the United States**  
~ 1 million currently receiving Rx<sup>4</sup>

**Innovation needed**

only approved pharmaceutical treatment is more than 50 years old

**Aligns with Acadia's customer-facing footprint**  
and focus on movement disorder specialists/centers of excellence



# ACP-711 is a Natural Fit with Acadia's Commitment to Innovation in CNS

2025 activities planned to support Acadia's desired Phase 2 trial

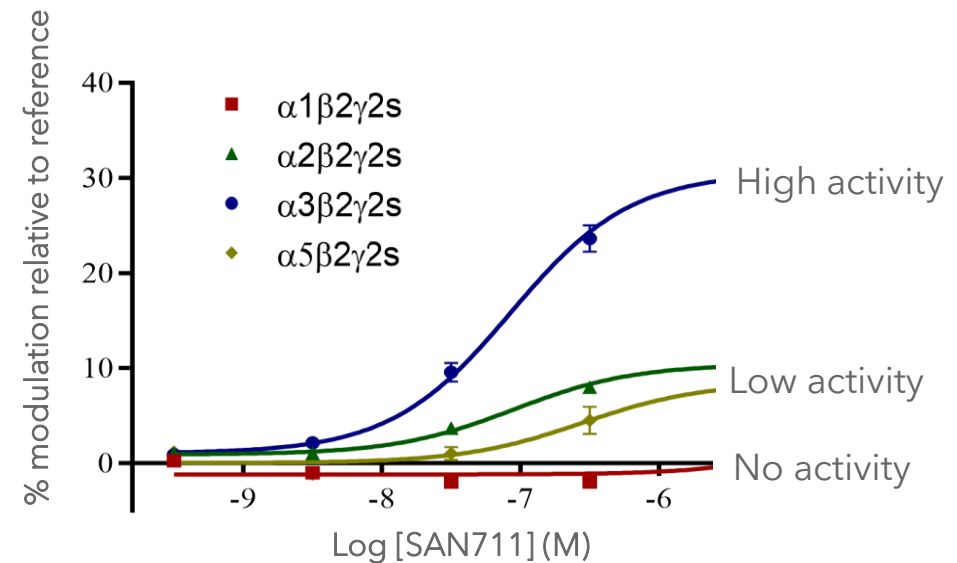
Inclusion of elderly cohort in ongoing Phase 1 to inform future development, given importance of treating the whole patient population

Phase 2 trial planned for 2026

Highly selective activity for alpha-3 subunit

GABA<sub>A</sub> α3 subunit is expressed in key regions involved in essential tremor (cerebellar dentate, thalamus, and cortex)<sup>1-4</sup>

ACP-711 is a selective GABA<sub>A</sub> α3 positive allosteric modulator (PAM) with potential to treat ET





# In Closing



Advancing breakthroughs in neuroscience and rare diseases to **elevate life.**

# Acadia Believes More is Possible

## COMMERCIAL

### TWO GROWING BRANDS

**NUPLAZID**<sup>™</sup>  
(pimavanserin) tablets

**Daybue**<sup>™</sup>  
(trofinetide)

2025 expected to be first full year of \$1 billion+ U.S. total revenues

Strong growth for both brands; initial revenues from Managed Access Programs in select EU countries

## R&D

### LATE-STAGE PIPELINE

**ACP-101** (P3-PWS)  
LPI expected Q4:2025

**ACP-204** (P2-ADP)  
LPI expected Q1:2026

Growing pipeline in CNS and rare disease with late-stage readouts expected in 2026

Hosting first-ever R&D Day in mid-2025

## FINANCIAL

Strong balance sheet with positive, growing cash flow to reinvest for growth

POWERED BY PRECISION MEDICINE | DATA INNOVATION | GLOBALIZATION | PATIENT EMPOWERMENT



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