



Third Quarter 2019 Earnings Call

OCTOBER 30, 2019

3Q19 Earnings Call Agenda

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Mark Johnson | Vice President, Investor Relations

CEO OPENING REMARKS

Steve Davis | Chief Executive Officer

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Serge Stankovic, M.D., M.S.P.H. | President

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Forward-Looking Statement

This presentation contains forward-looking statements. These statements relate to future events and involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed in or implied by such forward-looking statements. Each of these statements is based only on current information, assumptions and expectations that are inherently subject to change and involve a number of risks and uncertainties. Forward-looking statements include, but are not limited to, statements about (i) plans for, including timing and progress of commercialization of, NUPLAZID® or for the clinical development of our product candidates, including pimavanserin and trofinetide; (ii) benefits to be derived from and efficacy of our product candidates, including the use of pimavanserin in dementia-related psychosis, schizophrenia, depression or other neurological or psychiatric indications, potential advantages of NUPLAZID versus existing antipsychotics or antidepressants, and expansion opportunities for NUPLAZID; (iii) estimates regarding the prevalence of PD, PD Psychosis, dementia-related psychosis, schizophrenia or depression and the potential use of trofinetide in Rett syndrome; (iv) potential markets for any of our products, including NUPLAZID and trofinetide; and (v) our estimates regarding our future financial performance, cash position or capital requirements.

In some cases, you can identify forward-looking statements by terms such as “may,” “will,” “should,” “could,” “would,” “expects,” “plans,” “anticipates,” “believes,” “estimates,” “projects,” “predicts,” “potential” and similar expressions (including the negative thereof) intended to identify forward-looking statements. Given the risks and uncertainties, you should not place undue reliance on these forward-looking statements. For a discussion of the risks 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, 2018 as well as our subsequent filings with the SEC. The forward-looking statements contained herein are made as of the date hereof, and we undertake no obligation to update them for future events.

CEO Opening Remarks

Steve Davis
CEO



Recent Highlights of Executing on our Strategy



Grow

3Q19 net sales \$94.6M; 62% YoY growth¹

FY 2019 net sales guidance \$330 to \$340 million

- Represents a 50% YoY net sales increase at mid-point of the range



Leverage

Pimavanserin for Dementia-Related Psychosis:

- Phase 3 HARMONY study positive; top-line results to be presented at CTAD

Pimavanserin for Major Depressive Disorder:

- Phase 3 CLARITY-2 and Phase 3 CLARITY-3 enrolling well

Pimavanserin for Schizophrenia:

- Phase 2 ADVANCE results expected in 4Q19

Trofinetide for Rett Syndrome:

- Phase 3 LAVENDER study initiated in 4Q19

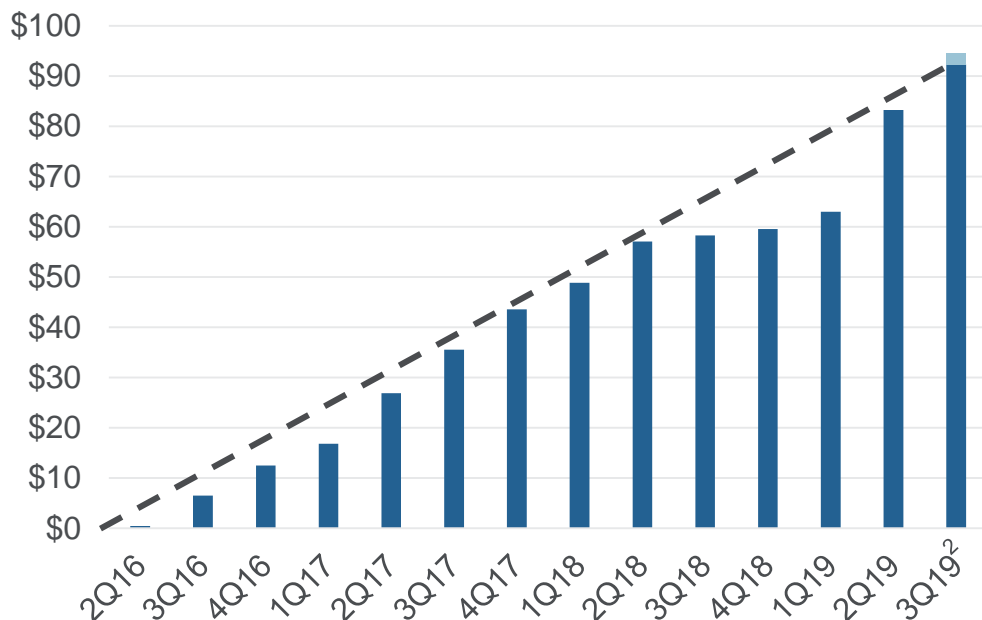


Commercial Update

Michael Yang
Chief Commercial Officer

Strong Commercial Execution in PDP

Net Sales¹ (in millions)



\$94.6M in Net Sales for 3Q19²

- ✓ Continued growth in new patients and from new and existing prescribers
- ✓ Continued volume growth in both specialty pharmacy and specialty distribution channels
- ✓ Sustained high-level of compliance and fulfillment for established patients

¹Net Sales shown above on a sell-in basis since launch; ACADIA changed revenue recognition methodology from sell-through to sell-in in Q2 2017.

²3Q19 includes a \$2.2M benefit resulting from a change in estimate for our Medicare accrual; highlighted in light blue in the graph above.

NUPLAZID is approved in the U.S. for hallucinations and delusions associated with Parkinson's disease psychosis.

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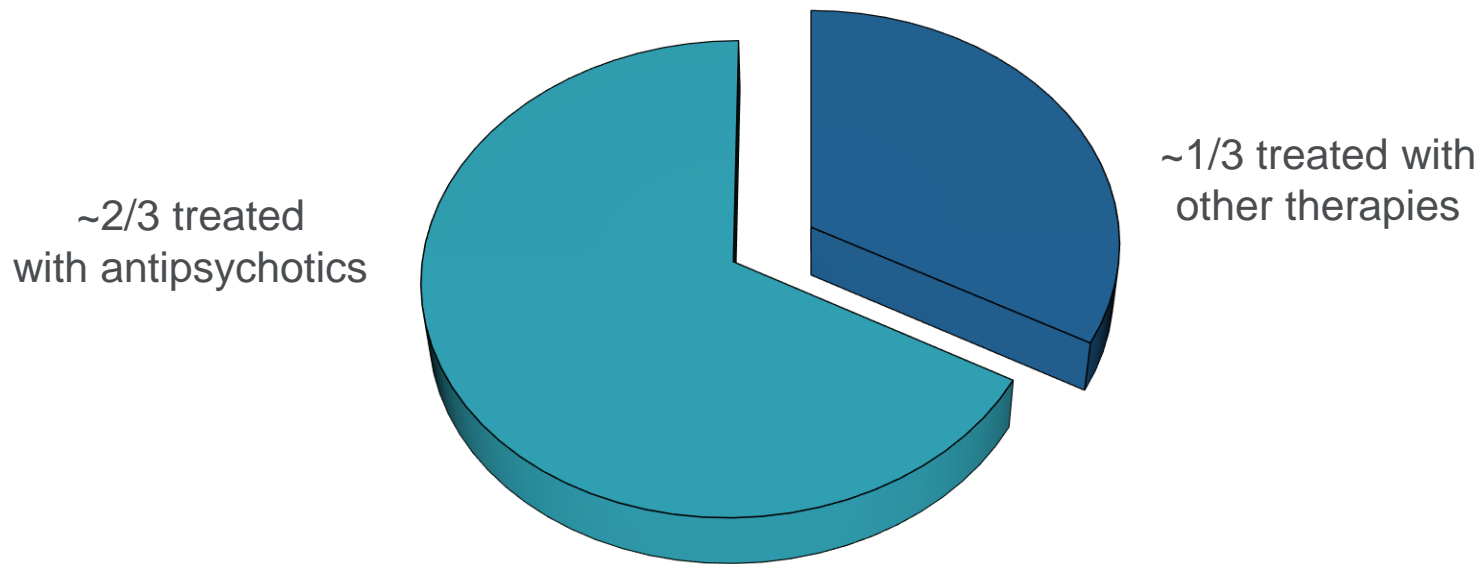
Continued Focus on PDP Disease Education and Awareness

- ✓ Increase awareness of NUPLAZID's inclusion in the updated MDS Commissioned Review¹
- ✓ Executing on an integrated consumer awareness strategy including digital, print, and in-office assets as well as recently deployed television commercials
- ✓ Michael J. Fox Foundation held its first event of the "Parkinson's IQ + You" series, designed to educate and empower patients with PD and their caregivers



Potential DRP Opportunity for Pimavanserin

~2.4 million patients in the U.S. with DRP of which
~1.2 million patients are treated¹



Launching DRP Disease Education and Awareness Initiatives

- ✓ Launched [MoreThanCognition.com](https://www.morethancognition.com) – DRP disease awareness and education microsite for HCPs
- ✓ Sponsoring disease awareness initiatives at major medical congresses, including a recent KOL symposium and booth in conjunction with the Alzheimer's Association International Conference (AAIC)

MORE
THAN COGNITION

Dementia-Related Psychosis:
Understanding the impact and consequences of delusions and hallucinations



Actor portrayal



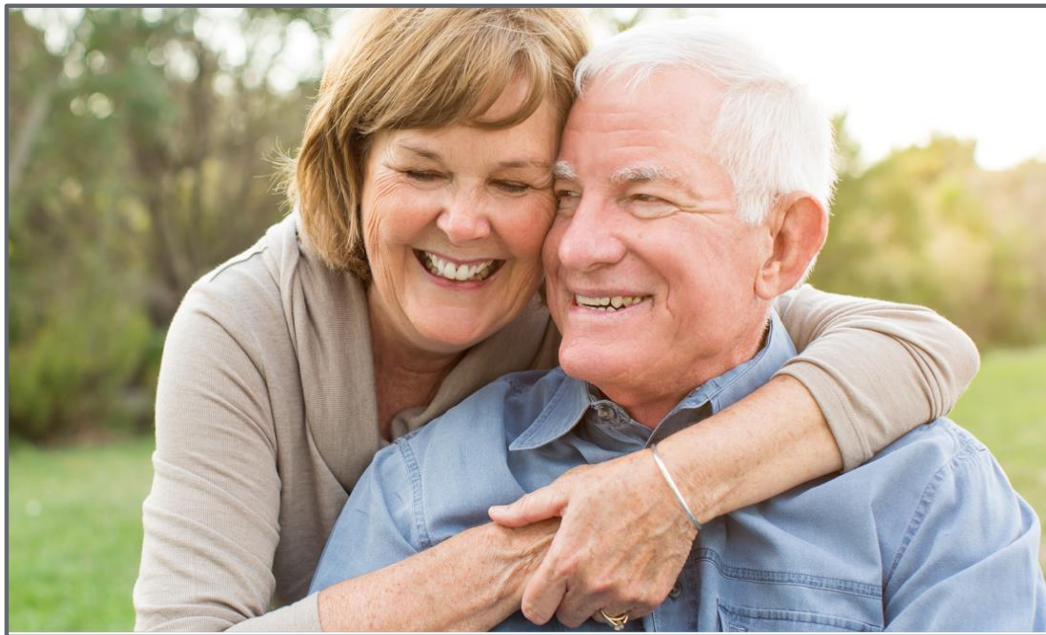
R&D Update

Serge Stankovic, M.D., M.S.P.H.
President

Significant Late-Stage Pipeline Opportunities

COMPOUND/ PROGRAM	INDICATION	IND-TRACK	PHASE 1	PHASE 2	PHASE 3	MARKETED
NUPLAZID® (pimavanserin) ¹	Hallucinations and Delusions associated with PD Psychosis					
Pimavanserin	Dementia-Related Psychosis					
Pimavanserin	Major Depressive Disorder <i>Adjunctive Therapy</i>					
Trofinetide ²	Rett Syndrome					
Pimavanserin	Schizophrenia Negative Symptoms <i>Adjunctive Therapy</i>					

Dementia-Related Psychosis (DRP)



**No new FDA-approved treatments
for people with dementia since 2003**

HIGH UNMET NEED

No FDA-approved treatments for DRP

~2.4 million dementia patients with psychosis

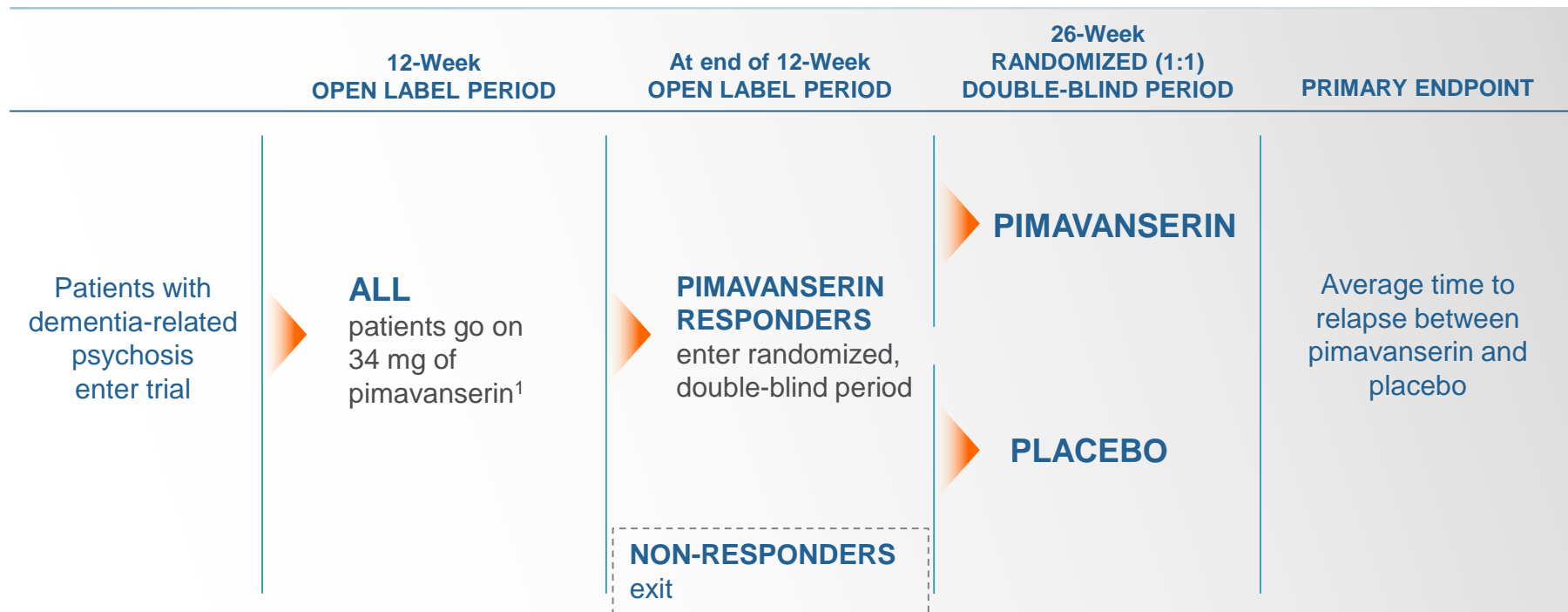
~1.2 million DRP patients are treated¹

Serious Consequences:

- Repeated hospital stays
- Earlier progression to nursing home
- More rapid progression of dementia
- Increased risk of morbidity and mortality

Antipsychotics used off-label
can accelerate cognitive decline and
carry significant side effects²

Phase 3 HARMONY Relapse Prevention Study



HARMONY met the pre-specified stopping criteria at the planned interim efficacy analysis requiring one-sided p-value less than 0.0033 on the study's primary endpoint

DRP Next Steps

- We remain on track to meet with the FDA in 1H20 to discuss our sNDA submission
- Based on our End-of-Phase 2 meeting with the FDA, we confirmed that the basis of an sNDA submission for DRP can rely on the HARMONY study provided that results are both statistically and clinically persuasive. In addition to HARMONY, we plan to include:

Positive Phase 2 Alzheimer's Disease Psychosis Study¹

Statistically significant reduction in psychosis in Alzheimer's disease patients vs. placebo

Positive Phase 3 Pivotal study in PDP²

Positive results from pre-specified subgroup of patients with MMSE<25³

Safety and Tolerability Data

Completed placebo-controlled studies and ongoing placebo-controlled PMC (post-marketing commitment) safety study

Pimavanserin has Breakthrough Therapy Designation for the treatment of DRP

¹Ballard C, et al. Lancet. 2018;17:213-222.

²NUPLAZID Prescribing Information; Cummings J, et al. Lancet. 2014;383:533-540.

³MMSE = Mini-Mental Status Examination, a test of cognitive function.

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Major Depressive Disorder (MDD) – Adjunctive Therapy



HIGH UNMET NEED

Majority of patients with MDD do not respond to initial antidepressant therapy

Potential U.S. Addressable Population:
~2.5M treated with adjunctive therapy¹

Current adjunctive use of antipsychotics in MDD can lead to significant side effects:

- Sedation
- Weight gain
- Sexual dysfunction
- Cognitive impairment
- Extrapyrimal symptoms
- Rare but serious tardive dyskinesia

Recent Publications/Presentations Supporting MDD Program

Phase 2 CLARITY Study (n=207)

Results recently published in *The Journal of Clinical Psychiatry*

- Primary endpoint achieved: depression (HAMD-17¹) $p=0.039$
- Key secondary endpoint achieved: disability (SDS¹) $p=0.004$
- Meaningful improvement in daytime wakefulness observed
- No meaningful weight gain and no motor function impairment

Meaningful improvement in symptoms of sexual dysfunction recently presented at 2019 Psych Congress

- Secondary endpoint achieved: Massachusetts General Hospital Sexual Functioning Index (MGH-SFI) *nominal* $p=0.0003^2$
- In Stage 1, adjunctive pimavanserin showed significant improvement on mean MGH-SFI scores from baseline vs. placebo *nominal* $p=0.0002$; *effect size*=0.614

Open-label Exploratory Phase 2 Study in Parkinson's Disease Patients with Comorbid Depression (n=47)

Study results recently presented at 2019 MDS Congress

- 8-week, open-label, single-arm Phase 2 study evaluating pimavanserin as monotherapy or adjunct to SSRI/SNRI therapy for PD patients with depressive symptoms
- Primary endpoint achieved: depression (HAMD-17) $p<0.0001$
- 60.0% of patients responded (Improvement of $\geq 50\%$ on HAMD-17)
- 44.4% of patients reached remission (HAMD-17 ≤ 7)

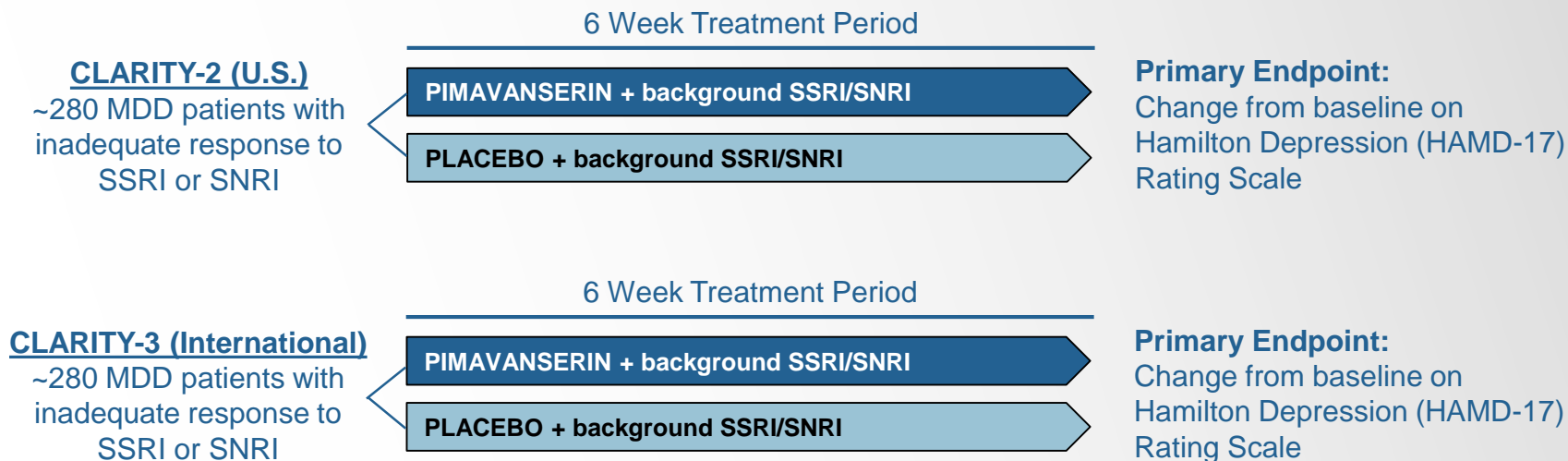
¹HAMD-17: 17-item Hamilton Depression Rating Scale; SDS = Sheehan Disability Scale.

²Fava M, Dirks B, Freeman MP, et al. A phase 2, randomized, double-blind, placebo-controlled study of adjunctive pimavanserin in patients with major depressive disorder and an inadequate response to therapy (CLARITY). *J Clin Psychiatry*. 2019;80(6):19m12928.

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Phase 3 CLARITY-2 and CLARITY-3 Studies

Two 6 Week, Randomized, Double-blind, Placebo-controlled Multi-center Studies:



sNDA Strategy:

Phase 2 CLARITY study plus at least one of the two Phase 3 studies

Schizophrenia Negative Symptoms



HIGH UNMET NEED

No FDA-approved treatment for negative symptoms of schizophrenia

~40 - 50% of schizophrenia patients experience prominent negative symptoms¹

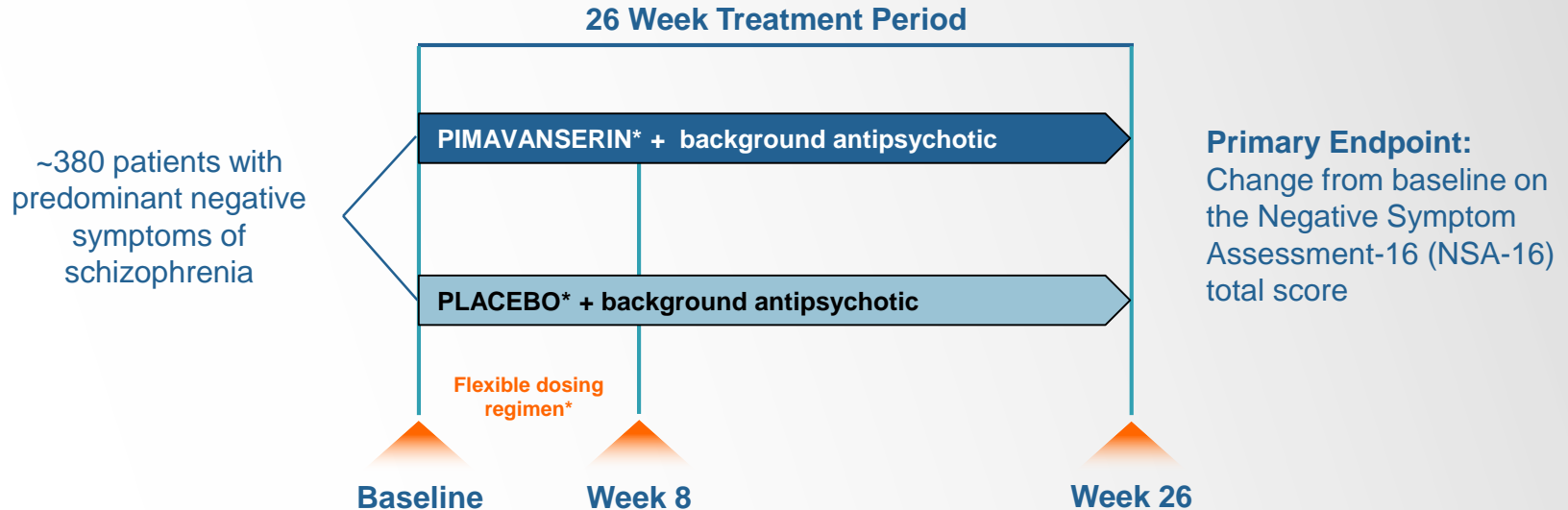
Potential U.S. Addressable Population:
>1M patients diagnosed¹

Prominent Negative Symptoms:

- Flat affect
- Loss of interest
- Emotional withdrawal
- Cognitive impairment

Phase 2 ADVANCE Study

ADVANCE: 26 Week, Randomized, Double-blind, Placebo-controlled, Multi-center Outpatient Study



*Starting daily dose of 20 mg at Baseline may be adjusted to 34 mg or 10 mg between weeks 2 and 8

Rett Syndrome



HIGH UNMET NEED

No FDA-approved treatment

Debilitating neurologic rare disease caused by mutations on the X chromosome on the *MECP2* gene

6,000 to 9,000 patients in the U.S.¹

Primarily occurs in females causing problems in brain function with rapid decline between 6 and 18 months of age.

Symptoms include:

- Cognitive, sensory, emotional, motor impairment
- Loss of independence
- Loss of purposeful hand use
- Loss of spoken communication

Trofinetide Clinical Program: Rett Syndrome

Trofinetide

Trofinetide is a novel synthetic analog of the amino-terminal tripeptide of IGF-1

Designed to treat the core symptoms of Rett syndrome by potentially reducing neuroinflammation and supporting synaptic function

Phase 2 study:

- Statistically significant improvements in **RSBQ¹ (*p-value* = 0.042)** and **CGI-I¹ (*p-value* = 0.029)** in girls 5 – 15 years of age
- Positive Phase 2 study results recently published in ***Neurology*^{®2}**

Clinical Program

LAVENDER Phase 3 study:

- ~180 females (ages 5 – 20) with Rett syndrome
- Double-blind, placebo-controlled
- Co-primary endpoints: RSBQ and CGI-I
- 12-week study duration

LILAC: 9-month extension study to evaluate long-term tolerability and safety of trofinetide

- U.S. Fast Track Status
- Orphan Drug Designation in the U.S. and Europe for Rett syndrome

2019 Clinical Milestones

COMPOUND/ PROGRAM	INDICATION	MILESTONE	EXPECTED TIMING
Pimavanserin	Major Depressive Disorder Adjunctive Therapy	✓ Commenced Phase 3 program	2Q19
Pimavanserin	Dementia-Related Psychosis	✓ Positive interim analysis for efficacy	3Q19
Trofinetide	Rett Syndrome	✓ Phase 3 study initiation	4Q19
Pimavanserin	Schizophrenia Negative Symptoms	Top-line Phase 2 ADVANCE study results	4Q19



Finance Update

Elena Ridloff
Chief Financial Officer

3Q19 Financial Highlights

Millions, Except EPS	3Q19 (GAAP)	3Q18 (GAAP)	YoY Change
Total Revenue	\$94.6 ¹	\$58.3	+62%
Cost of Product Sales, License Fees and Royalties	\$4.7	\$5.4	-13%
R&D	\$62.6	\$53.1	+18%
SG&A	\$72.7	\$61.1	+19%
Net Loss	(\$42.0)	(\$62.1)	-32%
Weighted Average Basic Shares Outstanding	145.9	125.0	+17%
EPS	(\$0.29)	(\$0.50)	+42%
Cash Balance 9/30/2019²	\$683.8		

FY2019 Financial Guidance

FY 2019	Updated Guidance	Previous Guidance	YoY Growth ¹
NUPLAZID® Net Sales	\$330 to \$340M	\$320 to \$330M	+50%
Gross-to-Net	15-16%	17-18%	-
GAAP R&D Expense	\$240 to \$250M	\$250 to \$265M	+31%
GAAP SG&A Expense	\$315 to \$325M	\$300 to \$315M	+20%
Non-Cash Stock-Based Compensation Expense	\$80 to \$90M	\$80 to \$90M	+4%

CEO Closing Remarks

Steve Davis
CEO





3 Strategic Pillars to Achieving our Vision



Grow

NUPLAZID® as the only approved treatment for patients with Parkinson's disease psychosis



Leverage

Our capabilities by developing our pipeline in additional indications with significant unmet need



Expand

Our pipeline through focused business development in CNS disorders with high unmet need



Q&A

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