



Fourth Quarter and Full Year 2019 Earnings Call

FEBRUARY 26, 2020

4Q and FY19 Earnings Call Agenda

INTRODUCTION

Mark Johnson | Vice President, Investor Relations

OPENING REMARKS

Steve Davis | Chief Executive Officer

COMMERCIAL UPDATE

Michael Yang | Chief Commercial Officer

R&D UPDATE

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

FINANCE UPDATE

Elena Ridloff | Chief Financial Officer

CLOSING REMARKS

Steve Davis | Chief Executive Officer

Q&A

Forward-Looking Statement

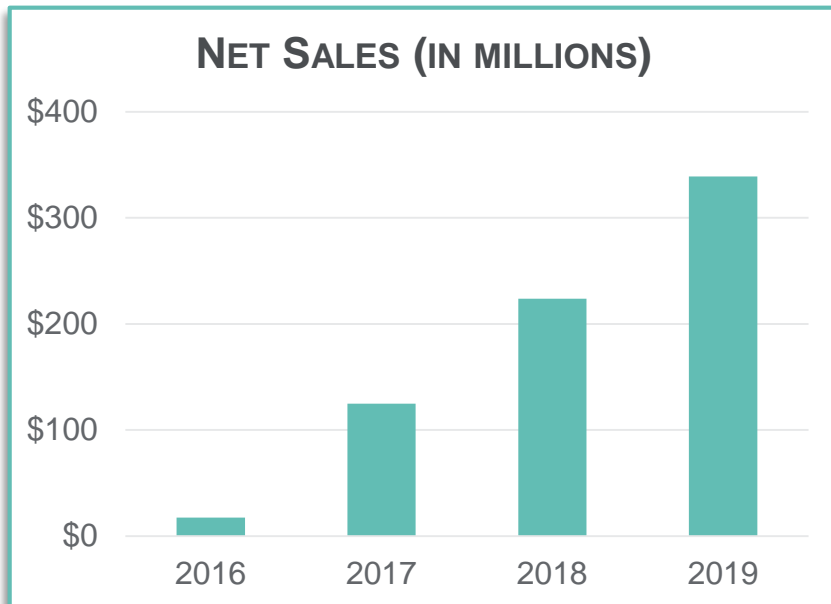
This presentation contains forward-looking statements. These statements relate to future events and involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements 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.

Opening Remarks

Steve Davis
CEO

2019 Full-Year Highlights



\$339.1M FY 2019 NET SALES

52% NUPLAZID® NET SALES GROWTH YOY

POSITIVE HARMONY STUDY

PIVOTAL RESULTS ANNOUNCED FOR
DEMENTIA-RELATED PSYCHOSIS

POSITIVE ADVANCE STUDY

PIVOTAL RESULTS ANNOUNCED FOR THE
NEGATIVE SYMPTOMS OF SCHIZOPHRENIA

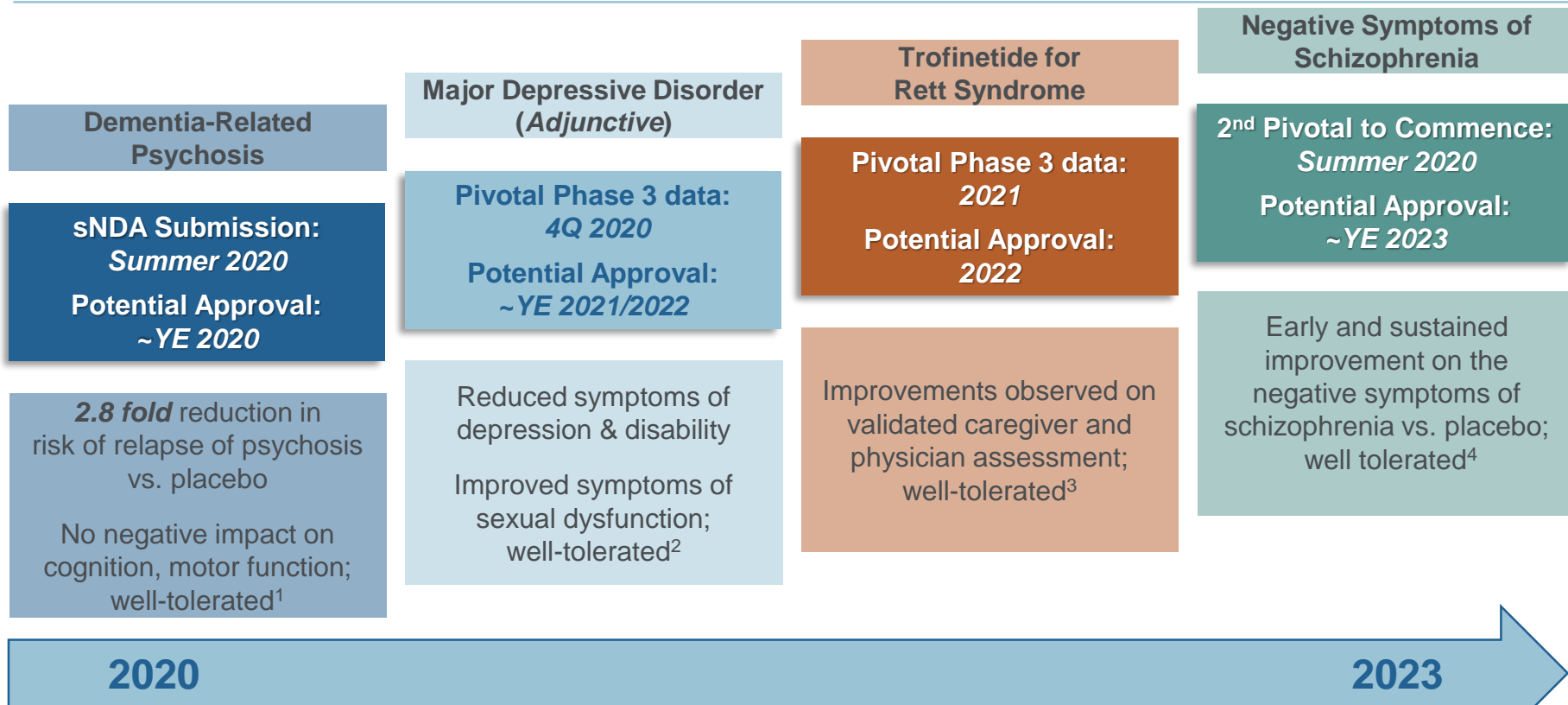
INITIATED CLARITY-2 AND CLARITY-3 STUDIES

PHASE 3 PROGRAM FOR
ADJUNCTIVE MAJOR DEPRESSIVE DISORDER

INITIATED LAVENDER STUDY

PHASE 3 PROGRAM FOR
TROFINETIDE IN RETT SYNDROME

Multi-Year Cadence of Potential Approvals



¹Data presented at CTAD 2019 on 12/4/2019. 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.

³Glaze D, et al. *Neurology*. Apr 2019, 92 (16) e1912-e1925. ⁴Top-line results from ADVANCE study, additional details in company press release issued on 11/25/2019.

NUPLAZID (pimavanserin) is only approved in the U.S by the FDA for the treatment of hallucinations and delusions associated with Parkinson's disease psychosis.

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2020 Strategic Pillars

Drive NUPLAZID® Growth in PDP



Deliver DRP Opportunity to the Market



Develop Innovative Treatments For Unmet Needs



2020: A Transformational Year

2020: A Transformational Year

Drive NUPLAZID® Growth in PDP

FY 2020 Net Sales Guidance:
\$440 to \$470M

Represents 34% YoY growth at
midpoint of range



Deliver DRP Opportunity to the Market

**2nd Indication for
Pimavanserin**

**Regulatory Timelines
On-Track:**
Pre-sNDA meeting in 1Q20;
sNDA submission summer 2020

Commercial Expansion:
Growing from
~200 to ~500 field roles
for launch

Develop Innovative Treatments For Unmet Needs

Major Depressive Disorder:
Phase 3 results 4Q20

**Negative Symptoms of
Schizophrenia:**
ADVANCE-2
initiating summer 2020

Business Development:
Leverage proven
commercial and R&D capabilities
with new opportunities

Commercial Update

Michael Yang
Chief Commercial Officer

NUPLAZID® 2020 Growth Initiatives in PDP

Grow

- Significant opportunity to grow market share (*currently in high-teens*)
- New patient market share continues to exceed overall market share

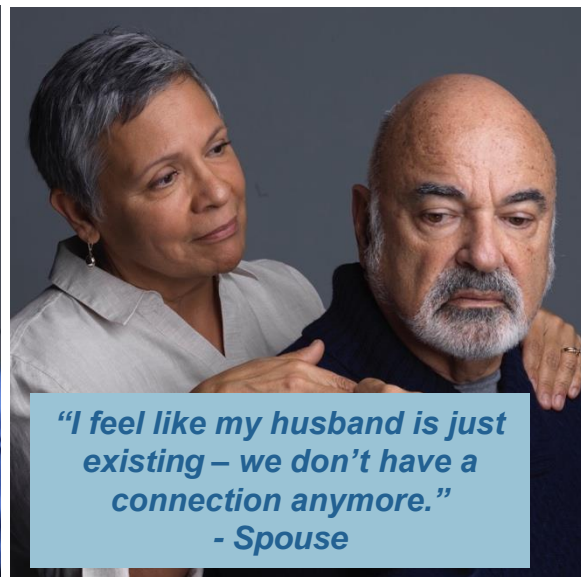
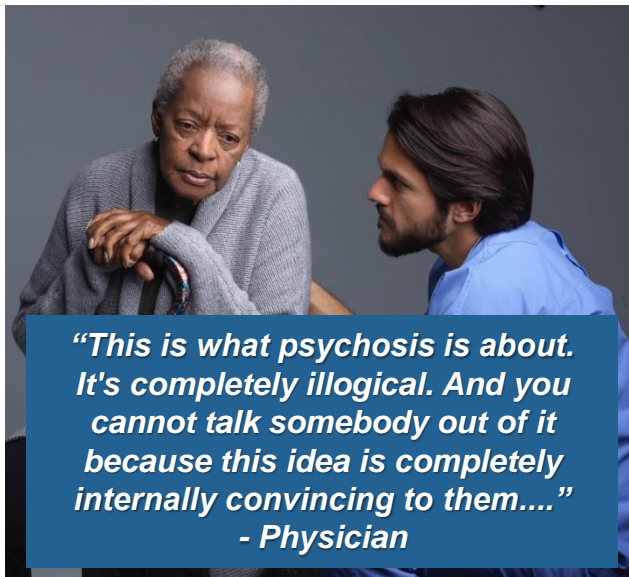
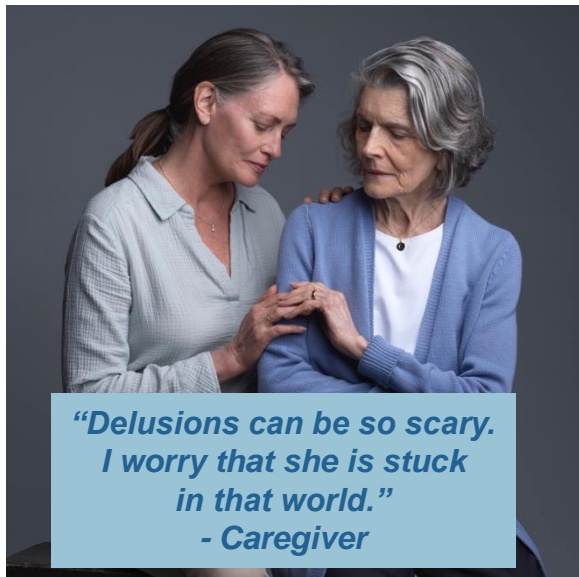
Elevate

- Highlight inclusion in MDS evidence-based guidelines review update
- New long-term, open-label NUPLAZID safety data
- Data highlighting reduction of caregiver burden for patients on NUPLAZID¹
- Integrated patient/caregiver consumer campaigns

Leverage

- 34 mg capsule benefits
- Continued high compliance and long-term adherence rate

High Burden of Disease: Dementia-Related Hallucinations and Delusions



Hallucinations and delusions may increase likelihood of nursing home placement



Increased burden for caregivers, who are often the target of patient's delusions

Learn more at: www.morethancognition.com

Market Expansion with Second Indication: Dementia-Related Psychosis

Key Similarities between PDP & DRP:

- High unmet need
- First and only FDA approved treatment¹
- Off-label treatments associated with troublesome side-effects and may worsen the primary disease
- Elderly patient population
- High caregiver burden
- High need for awareness and education

Key Differences between PDP & DRP:

- 1.2M treated population that is **10X** larger than the treated PDP population²
 - ~2/3 treated with antipsychotics (DRP)
- Greater physician recognition for the association between dementia and psychosis, compared to PD and psychosis
- Expanded audience of healthcare providers:
 - Psychiatrists & geriatric general practitioners
- Larger commercial footprint:
 - Growing from 200 to ~500 field roles



R&D Update

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

Innovative Late-Stage Pipeline

COMPOUND/ PROGRAM	INDICATION	PHASE 1	PHASE 2	PHASE 3	MARKETED
NUPLAZID® (pimavanserin) ¹	Parkinson's Disease Psychosis				
Pimavanserin	Dementia-Related Psychosis				
Pimavanserin	Major Depressive Disorder <i>Adjunctive Therapy</i>				
Trofinetide ²	Rett Syndrome				
Pimavanserin	Negative Symptoms of Schizophrenia				

Major Depressive Disorder – Adjunctive Therapy

High unmet need for differentiated adjunctive therapy



- **~17M patients in the U.S. have MDD¹**
 - Majority of patients with MDD do not respond to initial antidepressant therapy
- **~2.5M treated with adjunctive therapy²**
- **Adjunctive use of existing antipsychotics can lead to significant side effects:**
 - Sexual dysfunction
 - Sedation
 - Weight gain
 - Cognitive impairment
 - Extrapyrimalidal symptoms
 - Rare but serious tardive dyskinesia

Advancing Adjunctive Treatment for MDD

CLARITY Results

Meaningful Efficacy:

Primary endpoint achieved – Depression¹

- **HAMD-17 (*p-value*=0.039)**

Robust effect in the parallel design Stage 1

- **HAMD-17 (*p-value* = 0.0003;
Effect size = 0.63)**

Key secondary endpoint achieved - Disability¹

- **SDS (*p-value*=0.004)**

Secondary Outcome Findings:

- Early and sustained antidepressant treatment effect²
- Improvement in sexual dysfunction symptoms
- Improvement in daytime sleepiness
- No meaningful weight gain
- No cognitive side effects observed
- No extrapyramidal symptoms observed
- No tardive dyskinesia observed

CLARITY-2 Phase 3 Results 4Q20

CLARITY-3 Phase 3 Results 1Q21

Two ongoing Phase 3 studies with only one additional positive study necessary for sNDA

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

²Week 1 separation from placebo observed in Stage 1 (n=203) and Week 10 separation from placebo observed in Stage 1 patients who were not re-randomized in Stage 2 (n=174).

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Negative Symptoms of Schizophrenia

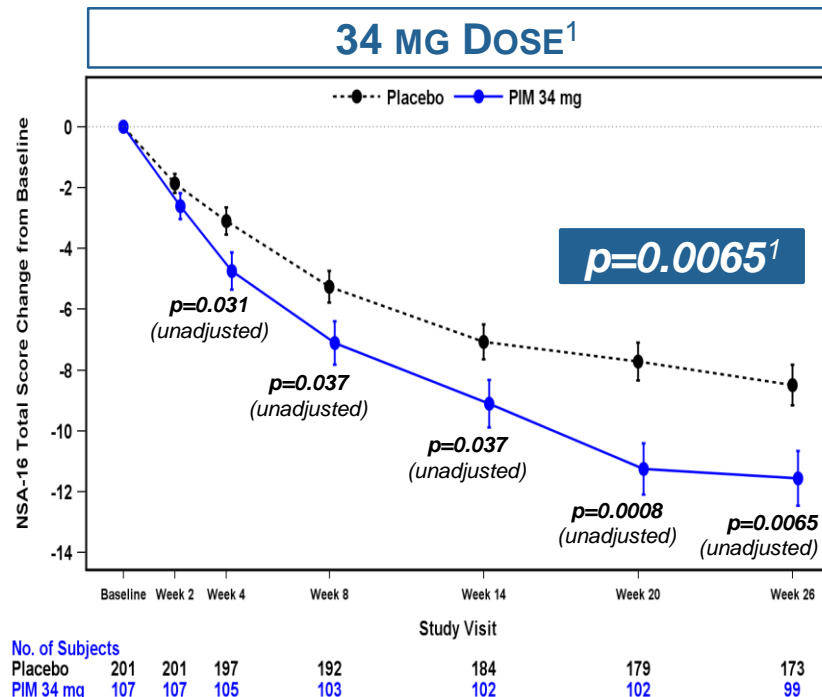
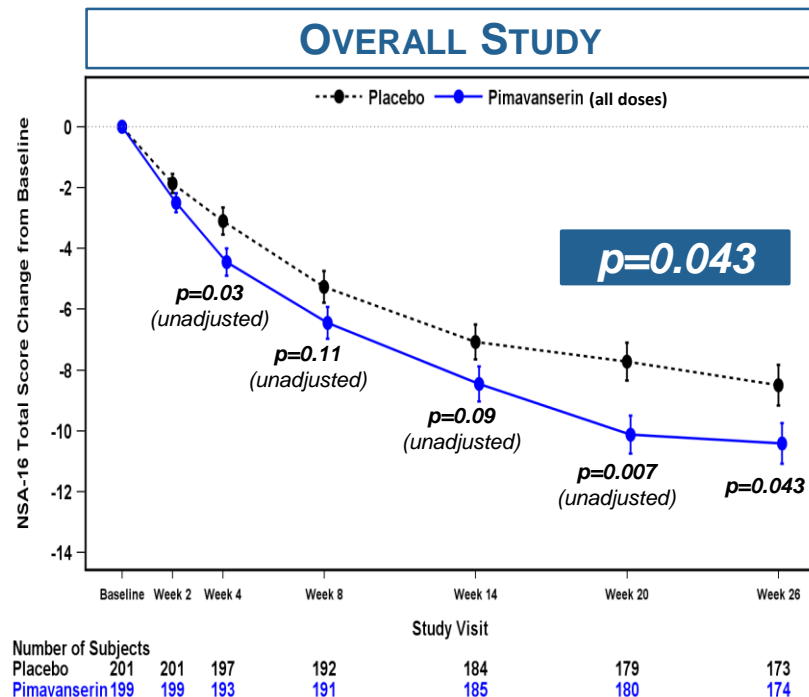
No FDA-approved treatment for the negative symptoms of schizophrenia



- ~40 - 50% of schizophrenia patients experience predominant negative symptoms¹
- **Negative symptoms include** social withdrawal, lack of emotion, restricted speech, and blunted affect and can lead to:
 - Low social functioning
 - Long-term disability
 - Significant caregiver burden

Positive ADVANCE Study Results

Primary Endpoint: NSA-16 Total Score



ADVANCE-2, Second Pivotal Study, Expected to Commence Summer 2020
ADVANCE-2 to evaluate fixed dose of 34 mg vs. placebo

Trofinetide for the Treatment of Rett Syndrome

No FDA-approved treatment for Rett syndrome



- Debilitating rare neurologic disease
- 6,000 to 9,000 patients in the U.S.¹
- Symptoms manifest primarily in young females:
 - Cognitive, sensory, emotional, and motor impairment
 - Loss of independence
 - Loss of purposeful hand use
 - Loss of spoken communication

Trofinetide Clinical Program

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

Clinical Program

LAVENDER Phase 3 study ongoing:

- ~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 LT tolerability and safety of trofinetide

Phase 2 study:

- Statistically significant results in **RSBQ** and **CGI-I**
- Positive Phase 2 study results published in *Neurology*^{®1}

LAVENDER Results Expected in 2021

Upcoming Clinical and Regulatory Milestones

COMPOUND	INDICATION	MILESTONE	EXPECTED TIMING
Pimavanserin	Dementia-Related Psychosis	Pre-sNDA Meeting	1Q20
		sNDA Submission	Summer 2020
Pimavanserin	Major Depressive Disorder <i>Adjunctive Therapy</i>	CLARITY-2 Results Expected	4Q20
		CLARITY-3 Results Expected	1Q21
Pimavanserin	Negative Symptoms of Schizophrenia	Initiate ADVANCE-2	Summer 2020
Trofinetide	Rett Syndrome	LAVENDER Results Expected	2021

Finance Update

Elena Ridloff
Chief Financial Officer

4Q19 Financial Highlights

Millions, Except EPS

	4Q19 (GAAP)	4Q18 (GAAP)	YoY Change
Total Revenue	\$98.3 ¹	\$59.6	+65%
Cost of Product Sales, License Fees and Royalties	\$5.3	\$4.4	+21%
R&D	\$57.5	\$48.2	+19%
SG&A	\$91.9	\$74.3	+24%
Net Loss	(\$53.0)	(\$65.5)	-19%
Weighted Average Basic Shares Outstanding	154.5	131.6	+17%
EPS	(\$0.34)	(\$0.50)	+32%

2019 Financial Highlights

Millions, Except EPS	2019 (GAAP)	2018 (GAAP)	YoY Change
Total Revenue	\$339.1	\$223.8	+52%
Cost of Product Sales, License Fees and Royalties	\$19.6	\$18.3	+7%
R&D	\$240.4	\$187.2	+28%
SG&A	\$325.6	\$265.8	+23%
Net Loss	(\$235.3)	(\$245.2)	- 4%
Weighted Average Basic Shares Outstanding	147.2	126.6	+16%
EPS	(1.60)	(1.94)	+18%
Cash Balance 12/31/2019¹	\$697.4		

FY2020 Financial Guidance

FY 2020	Guidance	Commentary
NUPLAZID® Net Sales	\$440 to \$470M	+34% YoY Growth¹
Gross-to-Net	17-18%	<i>Increase in donut hole obligation in 2020</i>
GAAP R&D Expense	\$270 to \$285M	<i>Reflects progression of 4 pivotal studies</i>
GAAP SG&A Expense	\$440 to \$460M	<i>Reflects similar YoY investments in PDP and launch prep for DRP</i>
Non-Cash Stock-Based Compensation Expense	\$90 to \$100M	
Projected 2020 Year-End Cash Balance²	\$470 to \$500M	

¹YoY growth number based on mid-point of the guidance range.

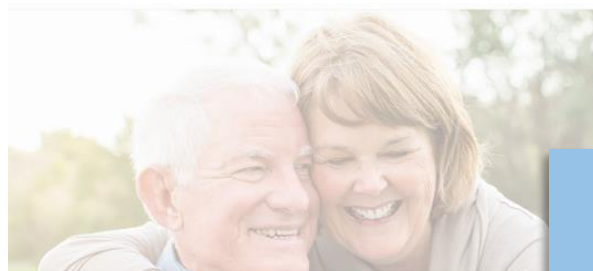
²Projected cash balance includes cash, cash equivalents and investments.

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Closing Remarks

Steve Davis
CEO

ACADIA in 2020 – Building a Leading CNS Platform



DRIVE NUPLAZID®
GROWTH IN PDP

\$440 - \$470M
FY2020 Net Sales Guidance



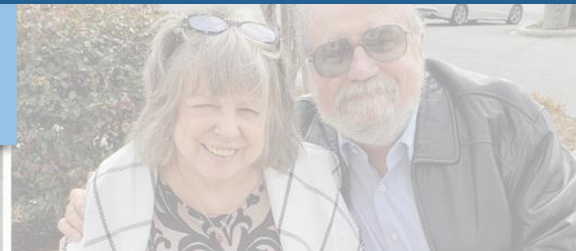
DELIVER DRP OPPORTUNITY
TO THE MARKET

POTENTIAL APPROVAL:
~YE 2020



DEVELOP INNOVATIVE TREATMENTS
FOR UNMET NEEDS

4 PIVOTAL STUDIES TO
ADVANCE IN 2020



2020: A Transformational Year



Q&A

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