

AXSOME

THERAPEUTICS

1Q 2020 Financial Results and Business Update
May 8, 2020

Forward-Looking Statements & Safe Harbor

Certain information contained in this presentation may include “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. We may, in some cases, use terms such as “predicts,” “believes,” “potential,” “continue,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “could,” “might,” “will,” “should” or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. In particular, the Company’s statements regarding trends and potential future results are examples of such forward-looking statements. The forward-looking statements include risks and uncertainties, including, but not limited to, the success, timing and cost of our ongoing clinical trials and anticipated clinical trials for our current product candidates, including statements regarding the timing of initiation, pace of enrollment and completion of the trials (including our ability to fully fund our disclosed clinical trials, which assumes no material changes to our currently projected expenses), futility analyses and receipt of interim results, which are not necessarily indicative of the final results of our ongoing clinical trials and the number or type of studies or nature of results necessary to support the filing of a new drug application for any of our current product candidates; our ability to fund additional clinical trials to continue the advancement of our product candidates; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration (“FDA”) or other regulatory authority approval of, or other action with respect to, our product candidates (including, but not limited to, FDA’s agreement with the Company’s discontinuation of the bupropion treatment arm of the ADVANCE-1 study in accordance with the independent data monitoring committee’s recommendations); the Company’s ability to obtain additional capital necessary to fund its operations; the Company’s ability to generate revenues in the future; the potential for the MOMENTUM clinical trial to provide a basis for approval of AXS-07 for the acute treatment of migraine in adults with or without aura, pursuant to our special protocol assessment; the potential for the ASCEND clinical trial, combined with the GEMINI clinical trial results, to provide a basis for approval of AXS-05 for the treatment of major depressive disorder and accelerate its development timeline and commercial path to patients; the Company’s ability to successfully defend its intellectual property or obtain the necessary licenses at a cost acceptable to the Company, if at all; the successful implementation of the Company’s research and development programs and collaborations; the enforceability of the Company’s license agreements; the acceptance by the market of the Company’s product candidates, if approved; the Company’s anticipated capital requirements, including the Company’s anticipated cash runway; and other factors, including general economic conditions and regulatory developments, not within the Company’s control. These factors could cause actual results and developments to be materially different from those expressed in or implied by such statements. Forward-looking statements are not guarantees of future performance, and actual results may differ materially from those projected. The forward-looking statements are made only as of the date of this presentation and the Company undertakes no obligation to publicly update such forward-looking statements to reflect subsequent events or circumstance.

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Axsome Therapeutics 1Q 2020

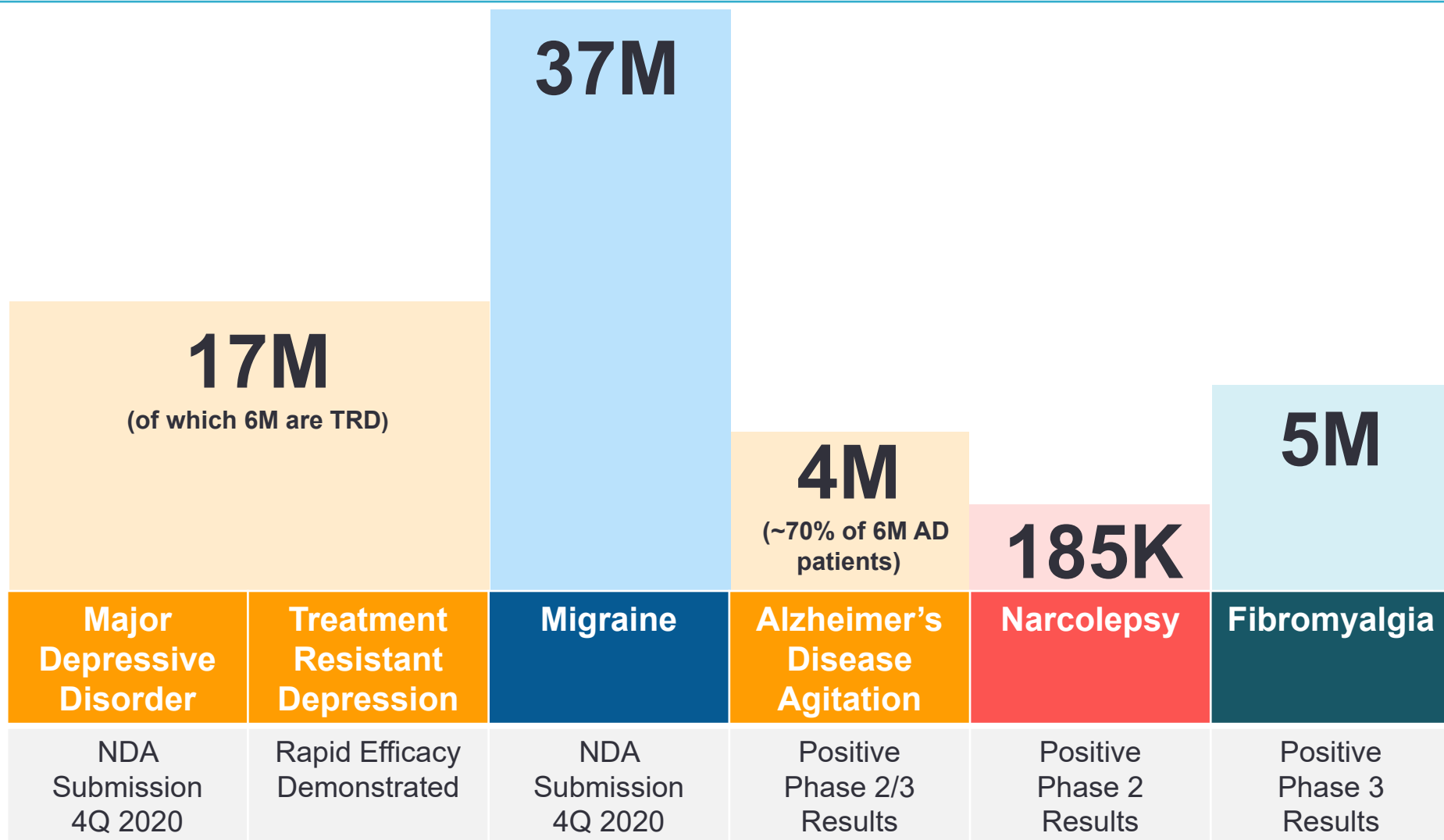
Financial Results and Business Update

Introduction	Mark Jacobson , Chief Operating Officer
Business Update	Herriot Tabuteau, MD , Chief Executive Officer
Financial Results	Nick Pizzie , Chief Financial Officer
Q&A	Presenters Dave Marek , Chief Commercial Officer Cedric O’Gorman, MD , Sr VP Clinical Development & Medical Affairs
Concluding Remarks	Herriot Tabuteau, MD , Chief Executive Officer

Axsome Therapeutics 1Q 2020

- Clinical successes highlight Axsome's accelerated evolution into a leading CNS company
- Positive efficacy results in 5 significant CNS indications with 4 differentiated product candidates advance Axsome's broad late-stage pipeline
- Positive pivotal Phase 2/3 results for AXS-05 in Alzheimer's disease agitation further deepen Axsome's innovative pipeline
- Two NDA submissions, for AXS-05 in MDD and AXS-07 in migraine, are on track for 4Q 2020
- Pre-commercialization activities are underway for our potentially first-in-class or best-in-class CNS therapies

Our late-stage portfolio has generated positive data in conditions that affect >60M U.S. patients



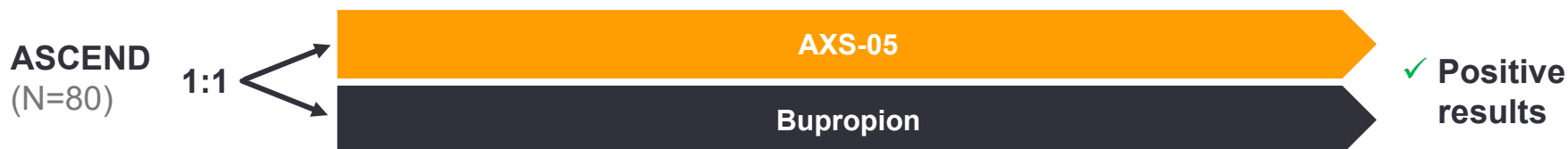
Late-Stage Indications with Potential Total U.S. Peak Sales of Up to \$9B

Program/ MOA	Indication	Launch Year Est.	Est. Peak U.S. Sales	Key Highlights
AXS-05 NMDA receptor antagonist with multimodal activity	MDD	2021	\$1B - \$2B	<ul style="list-style-type: none"> Novel, oral, NMDA receptor antagonist Rapid and substantial effect, as early as week 1 Breakthrough Therapy Designation
	TRD	2023	\$0.5B - \$1B	<ul style="list-style-type: none"> Symptom improvement in resistant population Rapid and substantial effect, as early as week 1 Improvement in cognitive function observed
	Alzheimer's Disease Agitation	2023	\$1.5B - \$3B	<ul style="list-style-type: none"> Rapid and substantial effect, as early as week 2 Not associated with cognitive impairment or sedation Currently no approved products for AD agitation
AXS-07 MoSEIC™ COX-2 pref. inhibitor + 5-HT _{1B/1D} agonist	Migraine	2021	\$0.5B - \$1B	<ul style="list-style-type: none"> Superior efficacy vs. gold standard in patients with history of inadequate response Prevention of pain progression with early treatment; rapid relief
AXS-12 Highly selective NE reuptake inhibitor	Narcolepsy	2023	\$0.5B - \$1B	<ul style="list-style-type: none"> Improved cataplexy, EDS, cognitive function Daytime dosing; well tolerated Not expected to be scheduled
AXS-14 Highly selective NE reuptake inhibitor	Fibromyalgia	2023	\$0.5B - \$1B	<ul style="list-style-type: none"> Reduced pain and improved function Effect on fatigue, a difficult-to-treat symptom Only 3 approved treatments

AXS-05 Depression Franchise

MDD NDA-Enabling Efficacy Studies:

Status:



TRD Efficacy Study:



Open Label Safety Study:



AXS-07 Migraine Program

NDA-Enabling Efficacy Studies:

Status:

MOMENTUM
(N=1594)

2:2:2:1

AXS-07

Rizatriptan

MoSEIC™ Meloxicam

Placebo

✓ Positive results

INTERCEPT
(N=302)

1:1

AXS-07

Placebo

✓ Positive results

Open Label Safety Study:

AXS-07

1

6 months, N=300

1 year, N = 100

- Completion expected 3Q 2020

AXS-05 Alzheimer's Disease Agitation

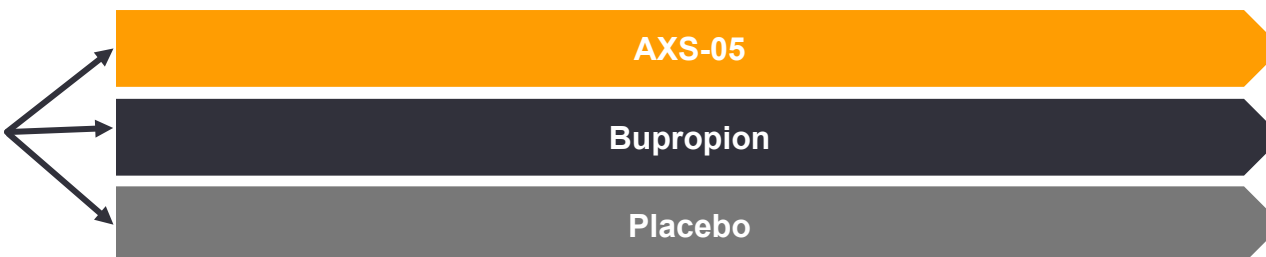
NDA-Enabling Efficacy Studies:

Status:

ADVANCE-1

(N=366)

Randomization



✓ Positive results

ADVANCE-2

(N=TBD)

1:1



• Initiation 2H 2020



Financial Update

Nick Pizzie

AXSOME THERAPEUTICS

CHIEF FINANCIAL OFFICER
AXSOME THERAPEUTICS, INC.

Key Financial Information

(in millions)

Cash and Cash Equivalents

<u>1Q '20</u>	<u>4Q '19</u>
\$ 197.3	\$ 220.0

Research & Development

<u>1Q '20</u>	<u>1Q '19</u>
\$ 27.5	\$ 7.6

General & Administrative

\$ 5.0	\$ 2.8
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Interest Expense

\$ -	\$ 0.2
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Net Loss

\$ 32.5	\$ 10.6
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One-time Expense - Pfizer License Agreement

\$ 10.2	\$ -
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Pro-forma Net Loss

\$ 22.3	\$ 10.6
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- **Financial guidance:** Cash anticipated to fund operating requirements for at least two years.



Q&A

Concluding Remarks

Herriot Tabuteau, MD

CHIEF EXECUTIVE OFFICER
AXSOME THERAPEUTICS, INC.

Our CNS Candidates and Pipeline

- Four differentiated clinical-stage CNS assets targeting significant and growing markets
- Patent protection to 2034-2036, worldwide rights for most product candidates

Product Candidate	Phase 1	Phase 2	Phase 3	NDA
AXS-05 (DM + BUP)	Major Depressive Disorder: Breakthrough Therapy Designation			
	Treatment Resistant Depression: Fast Track Designation			
	Alzheimer's Disease Agitation: Fast Track Designation			
	Smoking Cessation			
AXS-07 (MoSEIC™ Mx + Riz)	Migraine			
AXS-12 (Reboxetine)	Narcolepsy: U.S. Orphan Designation			
AXS-14 (Esreboxetine)	Fibromyalgia			

Abbreviations: BUP = Bupropion; CNS = Central Nervous System; DM = Dextromethorphan; Mx = Meloxicam; Riz = Rizatriptan;

Our Clinical and Regulatory Milestones

Product Candidate	Indication	2020
AXS-05 (DM + BUP)	MDD	<ul style="list-style-type: none"> ● NDA submission (4Q)
	TRD	<ul style="list-style-type: none"> ✓ STRIDE-1 Phase 3 topline results ● Phase 3 trial start (3Q)
	AD Agitation	<ul style="list-style-type: none"> ✓ ADVANCE-1 Phase 3 topline results ● ADVANCE-2 trial start (2H)
	Smoking Cessation	<ul style="list-style-type: none"> ● FDA meeting (2H)
AXS-07 (MoSEIC™ Mx + Riz)	Migraine	<ul style="list-style-type: none"> ✓ INTERCEPT Phase 3 topline results ● NDA submission (4Q)
AXS-12 (Reboxetine)	Narcolepsy	<ul style="list-style-type: none"> ● Phase 3 trial start (2H)
AXS-14 (Esreboxetine)	Fibromyalgia	<ul style="list-style-type: none"> ● FDA meeting (2H)

Abbreviations: AD = Alzheimer's Disease; BUP = Bupropion; DM = Dextromethorphan; MDD = Major Depressive Disorder; Mx = Meloxicam; Riz = Rizatriptan; TRD = Treatment Resistant Depression

✓ Accomplished milestone

● Upcoming milestone

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Thank you.

For more information, please contact

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