

# AXSOME

## THERAPEUTICS

January 2018

# 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 and completion of the trials, interim analyses and receipt of interim results; the timing of and our ability to obtain and maintain U.S. Food and Drug Administration or other regulatory authority approval of, or other action with respect to, our product candidates; the Company’s ability to obtain additional capital necessary to fund its operations; the Company’s ability to generate revenues in the future; 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; the enforceability of the Company’s license agreements; the acceptance by the market of the Company’s product candidates, if approved; 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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# Developing novel therapies for CNS disorders.

Axsome is addressing growing markets, where current treatment options are limited or inadequate, by leveraging well-characterized compounds to create novel therapeutics to meet unmet medical needs and improve the lives of patients.

# Our Candidates and Pipeline

- Four differentiated clinical-stage assets targeting significant and growing markets:
  - AXS-05: novel, oral, fixed-dose combination for multiple CNS indications
  - AXS-02: oral, non-opioid, long-acting, potentially first-in-class therapeutic for chronic pain
  - AXS-07: rapidly-absorbed, new molecular entity for migraine combined with triptan
  - AXS-06: rapidly-absorbed, once-daily, non-opioid, pain therapeutic with a gastroprotectant
- Patent protection to 2034, Worldwide rights.

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-05 (DM + BUP)	Treatment Resistant Depression: Fast Track Granted			Ongoing
	Agitation in Alzheimer's Disease: Fast Track Granted			Ongoing
	Smoking Cessation			Duke University Collaboration
AXS-02 (DZT)	Knee OA with BMLs: SPA Received; Fast Track Granted			Ongoing
	CLBP with MCs			
AXS-07 (MoSEIC™ Mx + Riz)	Migraine			
AXS-06 (MoSEIC™ Mx + Eso)	OA and RA			

Abbreviations: BML = Bone Marrow Lesions; BUP = Bupropion; CLBP = Chronic Low Back Pain; DM = Dextromethorphan; DZT = Disodium Zoledronate Tetrahydrate; Eso = Esomeprazole; MC = Modic Changes; Mx = Meloxicam; OA = Osteoarthritis; RA = Rheumatoid Arthritis; Riz = Rizatriptan; SPA = Special Protocol Assessment.

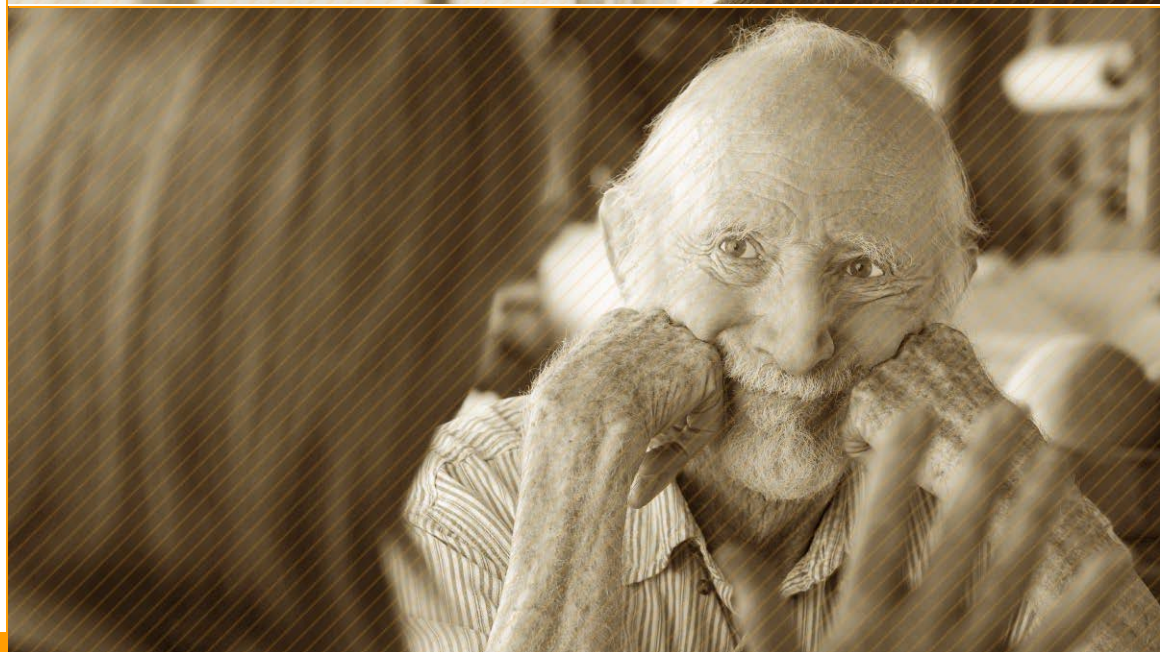


# AXS-05

## Dextromethorphan (DM) + Bupropion (BUP)

Novel therapy for CNS disorders:

- Treatment Resistant Depression (TRD)
- Agitation in Alzheimer's Disease (AD)



# CNS Disorders:

## Mechanisms of Action

### Pharmacodynamic Synergy

Mechanism of Action	AXS-05		
	DM	BUP	DM+BUP
NMDA Receptor Antagonist	✓		✓
Sigma-1R Agonist	✓		✓
Norepinephrine Reuptake Inhibitor	✓	✓	✓
Serotonin Reuptake Inhibitor	✓		✓
Dopamine Reuptake Inhibitor		✓	✓
Nicotinic ACh Receptor Antagonist		✓	✓

DM = Dextromethorphan; BUP = Bupropion.

✓ Present

# CNS Disorders:

## Mechanisms of Action and Relevant Indications

Mechanism of Action	Pharmacodynamic Synergy			Relevant Indications <sup>1</sup>								Related Agents <sup>2</sup>
	DM	BUP	AXS-05 DM+BUP	ADHD	Anxiety	Alzheimer's	Depression	Fibromyalgia	OCD	Pain	Smoking cessation	
NMDA Receptor Antagonist	✓		✓									<ul style="list-style-type: none"> <li>• Ketamine</li> <li>• Memantine (Namenda®)</li> </ul>
Sigma-1R Agonist	✓		✓									<ul style="list-style-type: none"> <li>• Fluvoxamine (Luvox®)</li> <li>• Donepezil (Aricept®)</li> </ul>
Norepinephrine Reuptake Inhibitor	✓	✓	✓									<ul style="list-style-type: none"> <li>• Duloxetine (Cymbalta®)</li> <li>• Venlafaxine (Effexor®)</li> </ul>
Serotonin Reuptake Inhibitor	✓		✓									<ul style="list-style-type: none"> <li>• Escitalopram (Lexapro®)</li> <li>• Fluoxetine (Prozac®)</li> <li>• Sertraline (Zoloft®)</li> </ul>
Dopamine Reuptake Inhibitor		✓	✓									<ul style="list-style-type: none"> <li>• Bupropion (Wellbutrin®)</li> </ul>
Nicotinic ACh Receptor Antagonist		✓	✓									<ul style="list-style-type: none"> <li>• Bupropion (Wellbutrin®)</li> </ul>

DM = Dextromethorphan; BUP = Bupropion.

✓ Present

■ Relevant

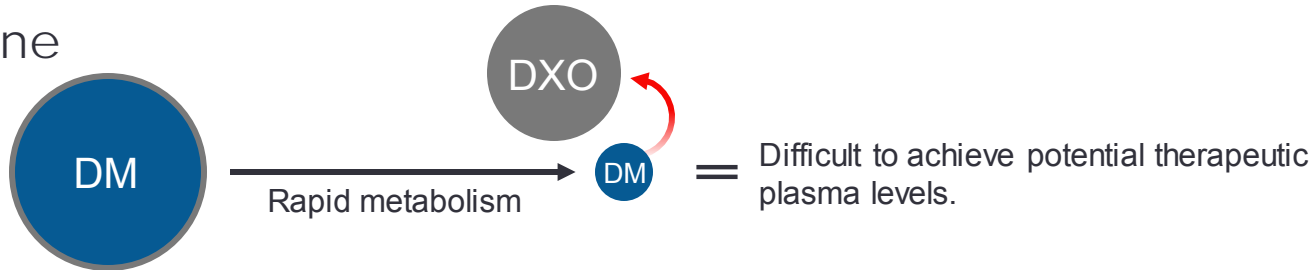
1. Indications listed are associated with the mechanism of action and are not related to either DM or BUP, unless specifically noted.

2. Agents do not contain DM or BUP, unless specifically noted.

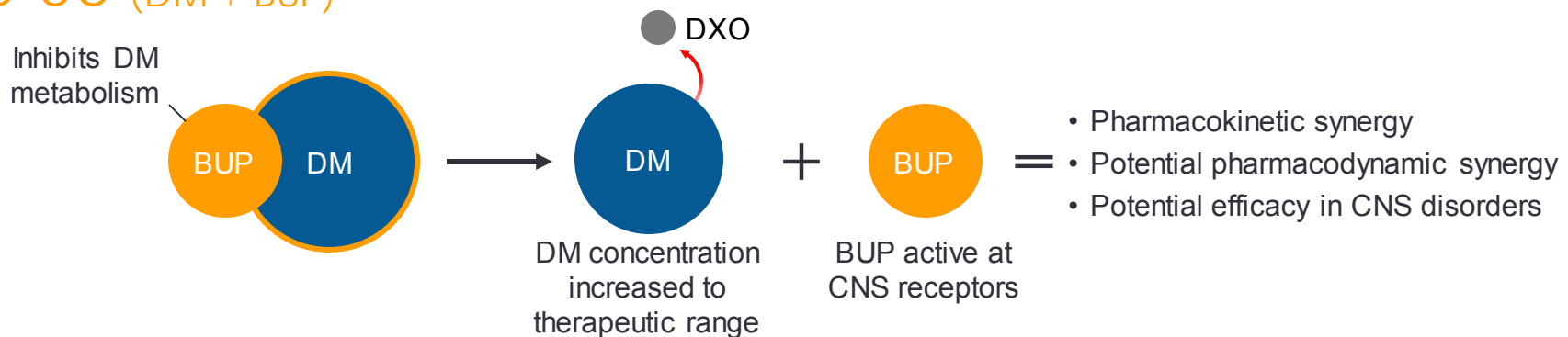
# CNS Disorders:

## Novel Therapy for CNS Disorders

DM Alone



AXS-05 (DM + BUP)



DM = Dextromethorphan; DXO = Dextrorphan; BUP = Bupropion.

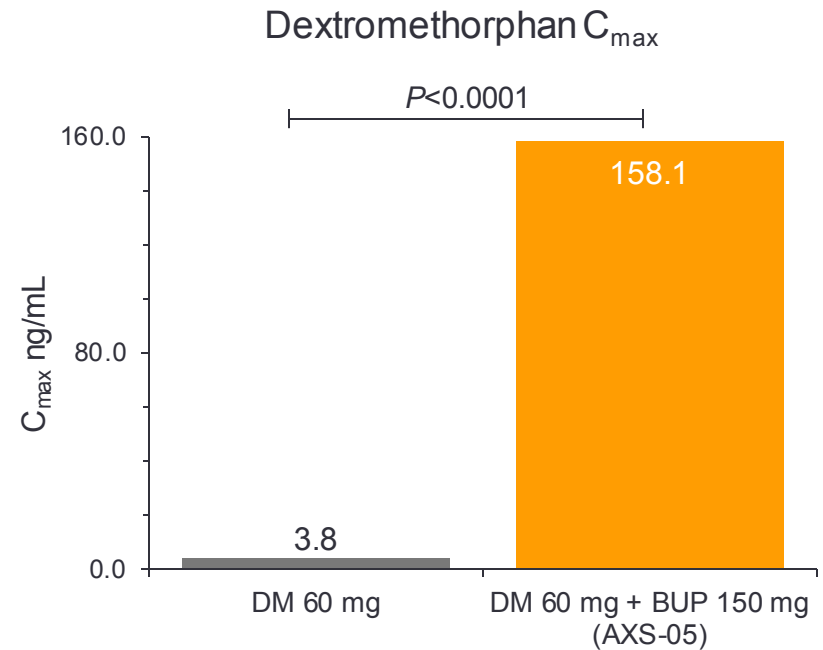
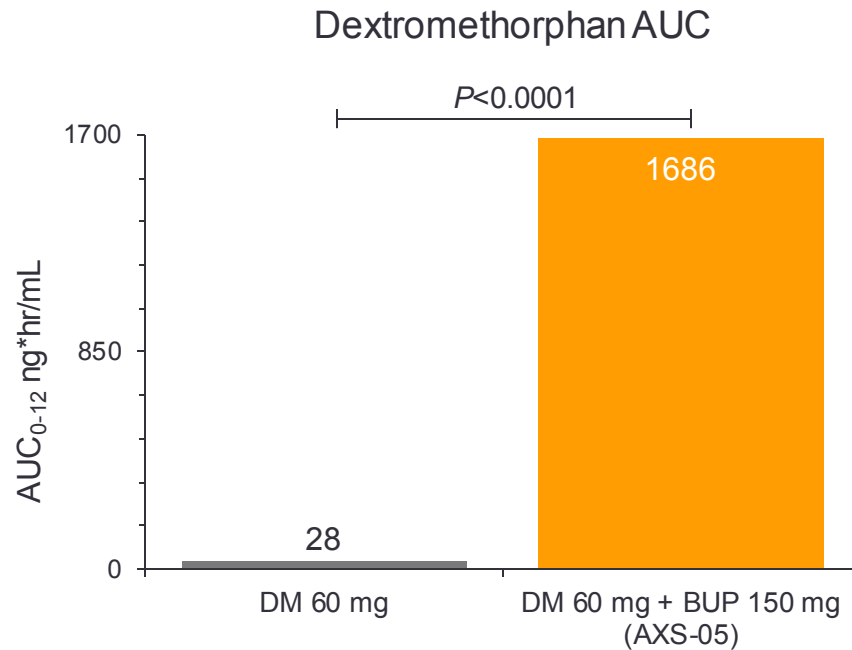
- Phase 1 trials with AXS-05 completed:
  - Significant increase in DM plasma levels.
- Phase 3 trials in TRD and AD Agitation initiated.

### IP Overview

- 22 issued patents – protection through 2034.



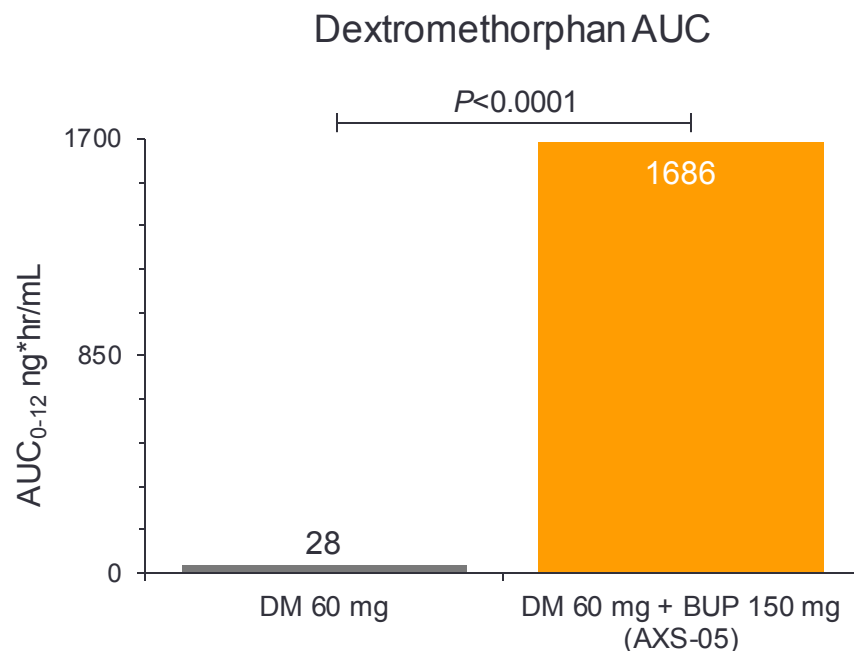
# CNS Disorders: Phase 1 Results



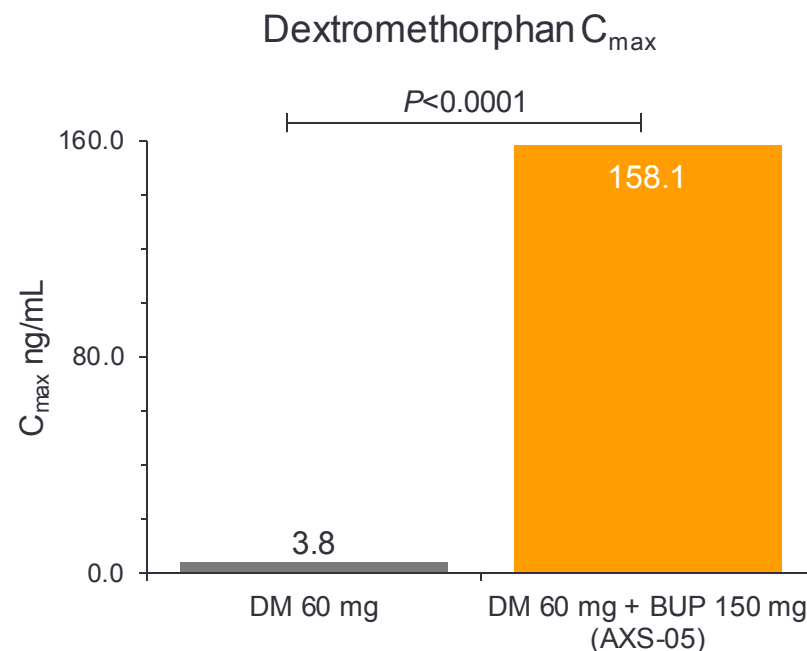
Axsome data on file.

†DM, Dextromethorphan; BUP, Bupropion.

# CNS Disorders: Phase 1 Results



Dose <sup>†</sup>	AUC <sub>0-12</sub> ng*hr/mL
DM 20 mg + Q 10 mg	525
DM 30 mg + Q 10 mg	883



Dose <sup>†</sup>	C <sub>max</sub> ng/mL
DM 20 mg + Q 10 mg	53
DM 30 mg + Q 10 mg	85

Axsome data on file.

<sup>†</sup> Nuedexta® NDA 021879, FDA Clinical Pharmacology Review.

DM, Dextromethorphan; Q, Quinidine; BUP, Bupropion.

# CNS Disorders:

## TRD Overview

- Major Depressive Disorder (MDD) is a leading cause of disease burden in the US.<sup>4</sup>
- 63% and 44% of MDD patients have inadequate response to initial therapy and second line therapy, respectively.<sup>2</sup>
- Only 1 approved drug for TRD = unmet medical need.
- AXS-05 combines the MOA of 4 distinct anti-depressant drug classes into one novel oral therapeutic.
- DM antidepressant effects demonstrated preclinically and clinically.
- Phase 3 ongoing.



3M patients  
in the U.S.<sup>1-3</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-05 (DM + BUP)	Treatment Resistant Depression: Fast Track Granted			Initiated

Abbreviations: DM = Dextromethorphan; BUP = Bupropion.

1. Marcus SC, Olfson M. *Arch Gen Psychiatry* 2010;67:1265-1273.  
2. Rush AJ, et al. *Am J Psychiatry* 2006;163:1905-1917.

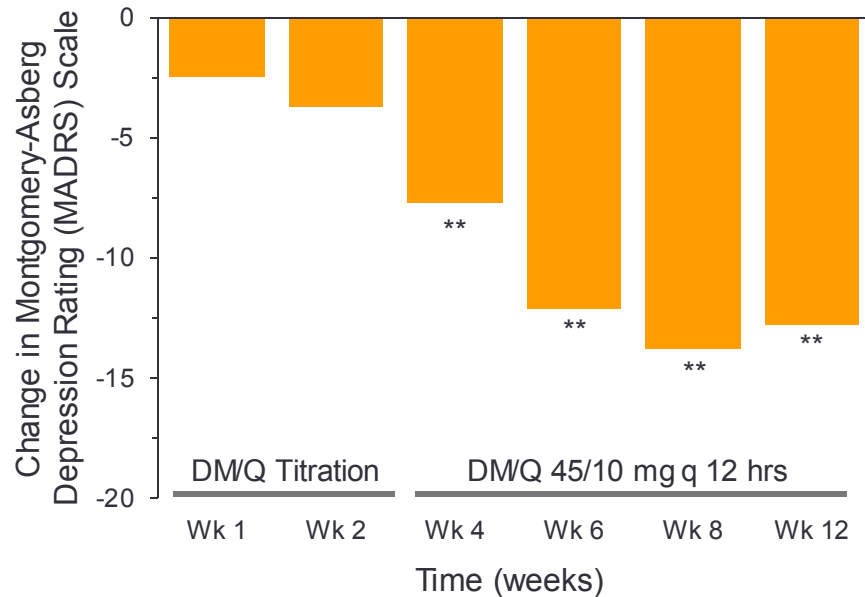
3. U.S. Census Bureau, Population April 1, 2010 to July 1, 2013.  
4. Mathers CD, *PLoS Med* 2006; 3(11): e442.

# CNS Disorders:

## TRD Clinical Rationale

- DM and metabolic inhibitor reduce depressive symptoms in TRD and in AD.

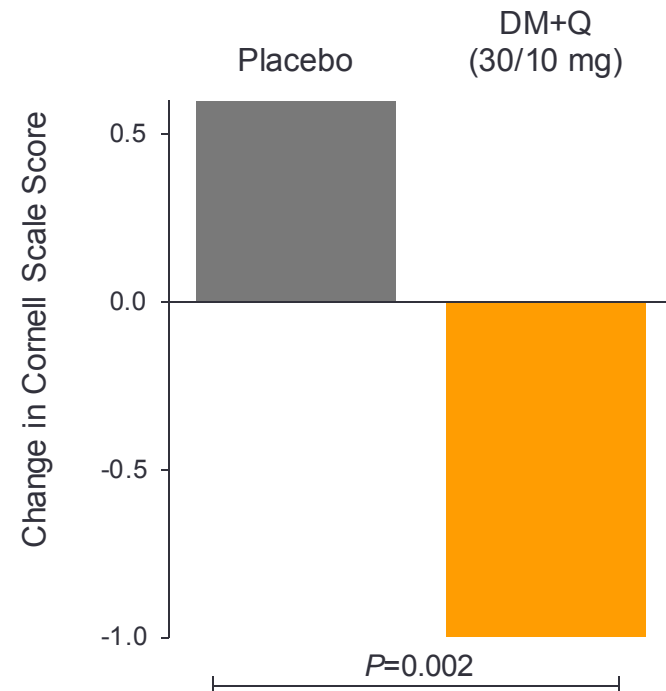
Symptom Reduction in TRD Patients  
Treated with DM and Metabolic Inhibitor<sup>1</sup>



- Failed 2 to 10 prior treatments
- 45% of patients had  $\geq 50\%$  reduction in MADRS

\*\*  $P < 0.01$  versus baseline

Depressive Symptom Reduction in AD Agitation  
Patients Treated with DM and Metabolic Inhibitor<sup>2</sup>



1. Murrough J, et al. *J Affect Disord.* 2017;218:277-283.

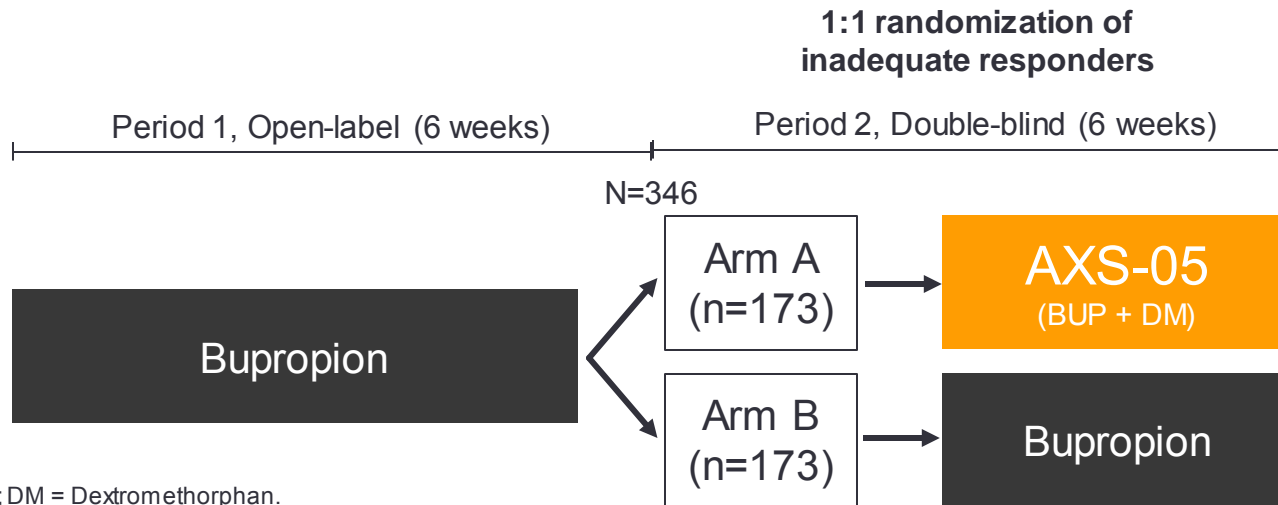
2. Cummings J, et al. *JAMA.* 2015;314:1242-1254.

# CNS Disorders:

## TRD Phase 3 Design



A Phase 3 trial to assess the efficacy and safety of  
**AXS-05** in the treatment of TRD.



BUP = Bupropion; DM = Dextromethorphan.

- **Primary Endpoint:** Change in depression score from randomization to end of study, measured using the Montgomery-Asberg Depression Rating Scale (MADRS).
- **Key Inclusion Criteria:**
  - Male or female 18-65 years old
  - History of inadequate response to 1 or 2 adequate antidepressant treatments

# CNS Disorders:

## Agitation in AD Overview

- Agitation and aggression seen in approximately 45% of AD patients during 5-year period.<sup>3</sup>
- Characterized by emotional distress, aggressive behaviors, disruptive irritability, disinhibition, and caregiver burden.<sup>4</sup>
- Associated with<sup>4,5</sup>:
  - Accelerated cognitive decline
  - Earlier nursing home placement
  - Increased mortality
- No approved medication = unmet medical need.
- Proof of concept: DM plus metabolic inhibitor reduced agitation in AD patients.
- Phase 2/3 ongoing.



2M patients  
in the U.S.<sup>1,2</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-05 (DM + BUP)	Agitation in Alzheimer's Disease: Fast Track Granted			Initiated

Abbreviations: DM = Dextromethorphan; BUP = Bupropion.

1. Ryu, SH, et al. *Am J Geriatr Psychiatry*. 2005;13:976-983.

2. Hebert, LE, et al. *Neurology*. 2013;80:1778-1783.

3. Steinberg M, et al. *Int J Geriatr Psychiatry*. 2008;2:170-177.

4. Antonsdottir IM, et al. *Expert Opin Pharmacother*. 2015;11:1649-1656.

5. Rabins PV et al. *Alzheimers Dement*. 2013; 9:204-207.

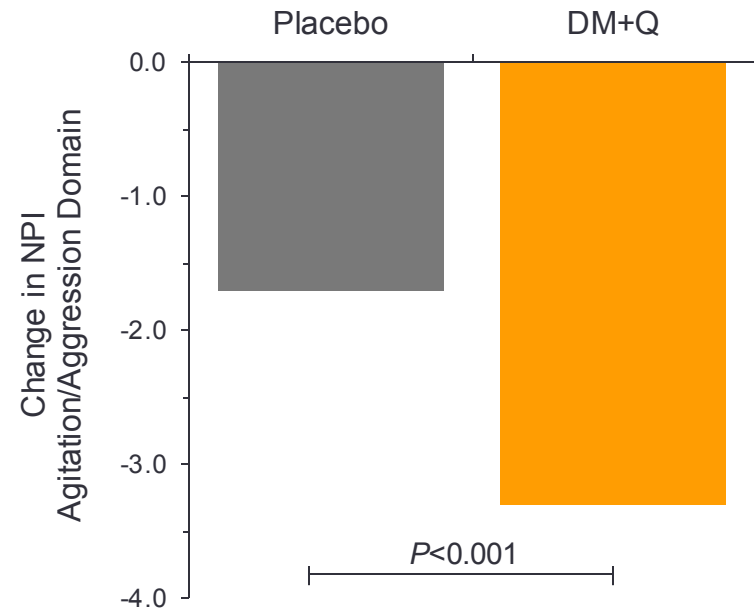


# CNS Disorders:

## Agitation in AD Clinical Rationale

- Randomized, double-blind, placebo-controlled, two-stage trial.
  - Placebo (n=125), 30 mg DM + 10 mg quinidine (Q) (n=93), for stage 1.
- DM+Q treatment reduced agitation/aggression in AD by 46% vs. 24% for placebo ( $P<0.001$ )—primary endpoint.
- Statistically significant improvement in multiple secondary endpoints.
- DM plasma levels achieved with AXS-05 in target therapeutic range.
- Potential for additional contribution from bupropion component of AXS-05.

Change in Agitation/Aggression Scores in AD with DM and Metabolic Inhibitor Quinidine (Q)



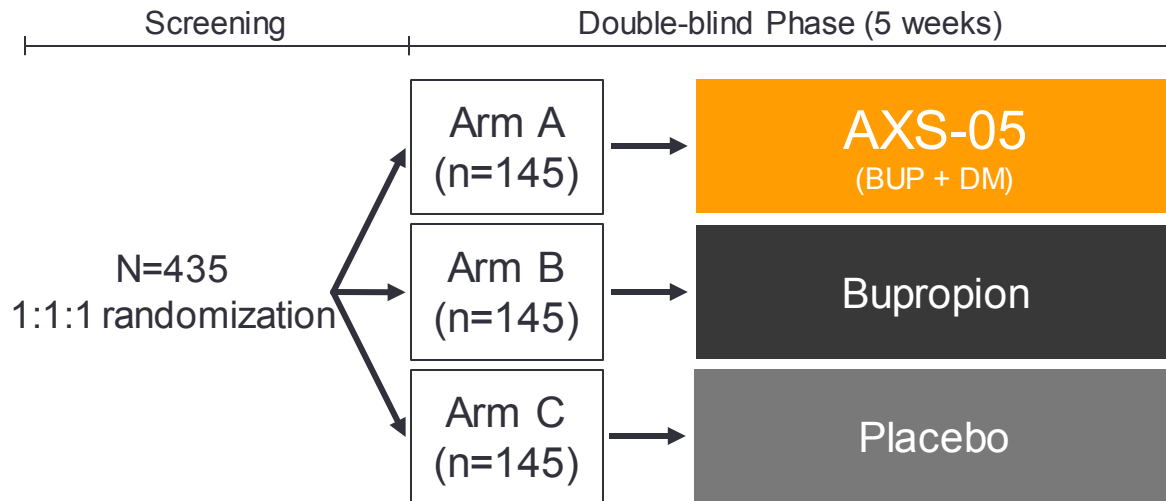
Cummings J, et al. *JAMA*. 2015;314:1242-1254.

# CNS Disorders:

## Agitation in AD Phase 2/3 Design

**ADVANCE**  
STUDY

A Phase 2/3 trial to assess the efficacy and safety of  
**AXS-05** in the treatment of Agitation in AD.



BUP = Bupropion; DM = Dextromethorphan.

- **Primary Endpoint:** Cohen-Mansfield Agitation Inventory (CMAI).
- **Key Inclusion Criteria:**
  - Diagnosis of probable Alzheimer's disease
  - Clinically significant agitation
- Interim analysis planned.

# CNS Disorders:

## Smoking Cessation Overview

- Smoking is single largest cause of preventable death in the U.S.<sup>1</sup>
- 70% of smokers want to quit and only 3-5% who attempt to quit without assistance are successful for 6-12 months.<sup>2</sup>
- DM component of AXS-05 significantly reduced nicotine self-administration in nicotine-dependent rats.
- Bupropion component of AXS-05 has been found to be effective for smoking cessation in clinical trials.
- Axsome entered into a research collaboration with Duke University to evaluate AXS-05 in a Phase 2 clinical trial in smokers attempting to quit.
- Phase 2 controlled trial initiation anticipated in 1Q 2018.



40M patients  
in the U.S.<sup>1</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-05 (DM + BUP)	Smoking Cessation			Duke University Collaboration

Abbreviations: DM = Dextromethorphan; BUP = Bupropion.

1. U.S. Department of Health and Human Services. The Health Consequences of Smoking: 50 Years of Progress. A Report of the Surgeon General. 2014.

2. Hughes JR, et al. *Addiction*. 2004;99(1):29-38.

# AXS-02

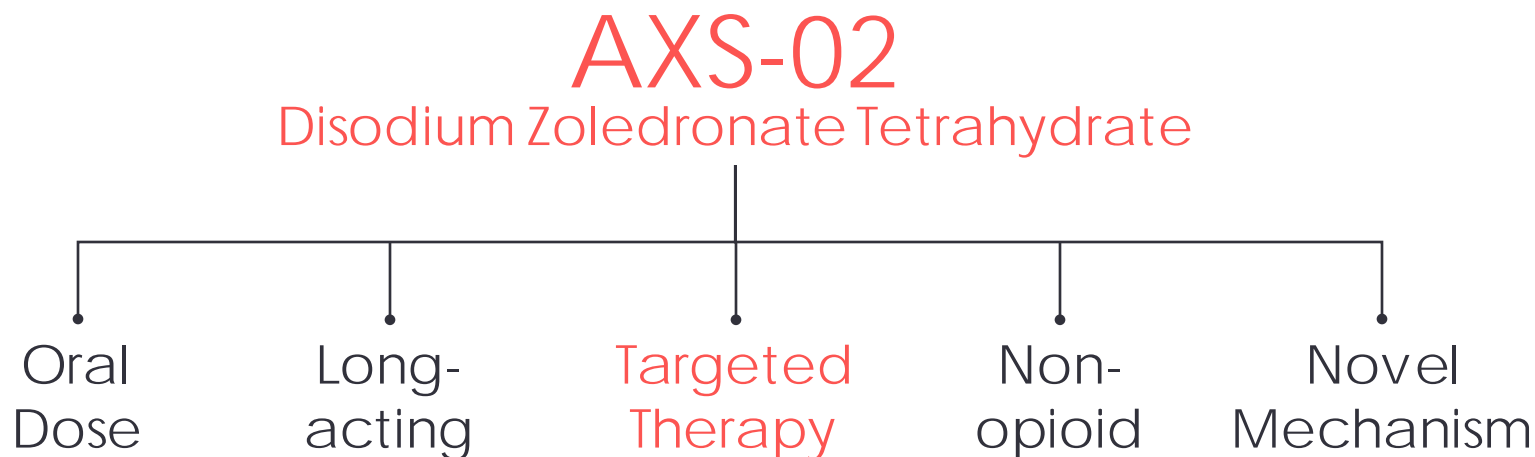
## Disodium Zoledronate Tetrahydrate

Novel therapy for chronic pain:

- Knee Osteoarthritis (OA) with Bone Marrow Lesions (BMLs)
- Chronic Low Back Pain (CLBP) with Modic Changes (MCs)



# Chronic Pain: Differentiated Therapy



## IP Overview

- 61 issued patents\* – protection through 2034.
- Drug delivery, pharmacokinetic, composition of matter, and method of use claims.

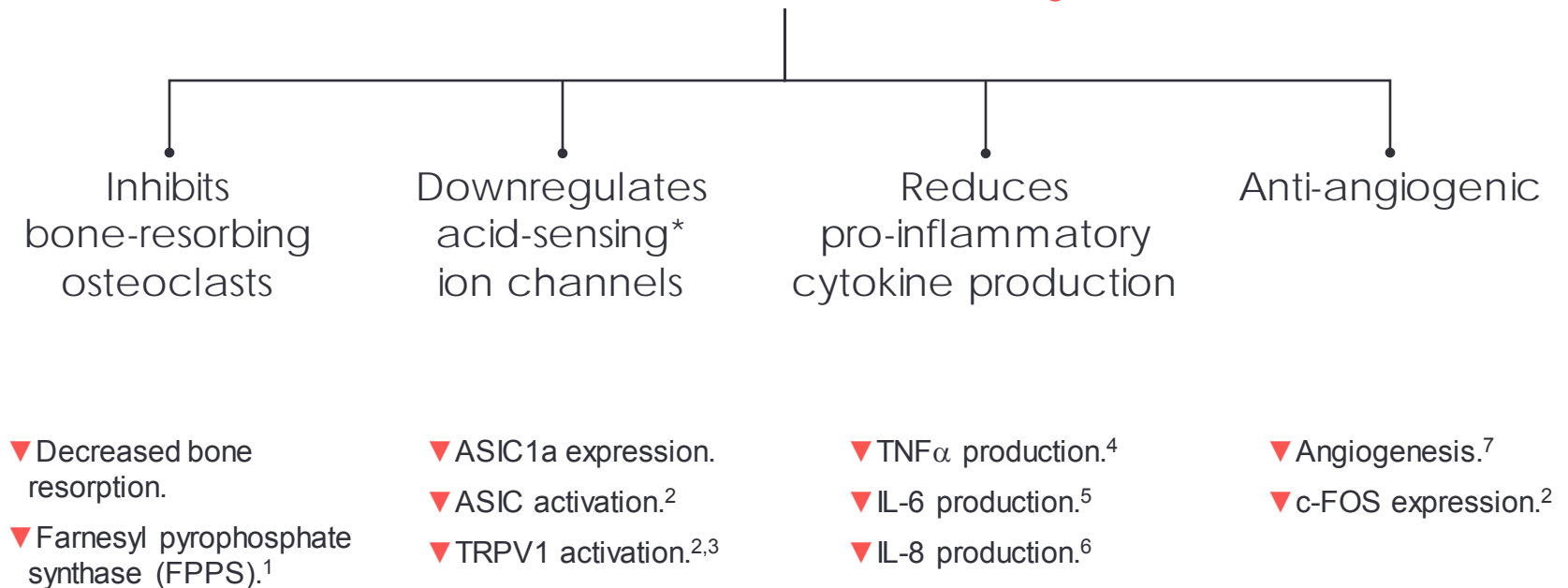
\*Claims cover AXS-02 and related substances and disease indications.

# Chronic Pain:

## Therapy via Multiple Mechanisms of Action

### AXS-02

#### Disodium Zoledronate Tetrahydrate



\* Acid is a well known cause of pain.

1. Green JR, Rogers MJ. *Drug Dev Res*. 2002;55:210-24.

2. Nagae M, et al. *Bone*. 2006;39:1107-15.

3. Abe Y, et al. *J Bone Miner Metab*. 2015;33:125-134.

4. Wolf AM, et al. *Haematologica*. 2006;91:1165-71.

5. Derenne S, et al. *Bone Miner Res*. 1999;14:2048-56.

6. Stathopoulos GT, et al. *Am J Respir Crit Care Med*. 2008;178:50-9.

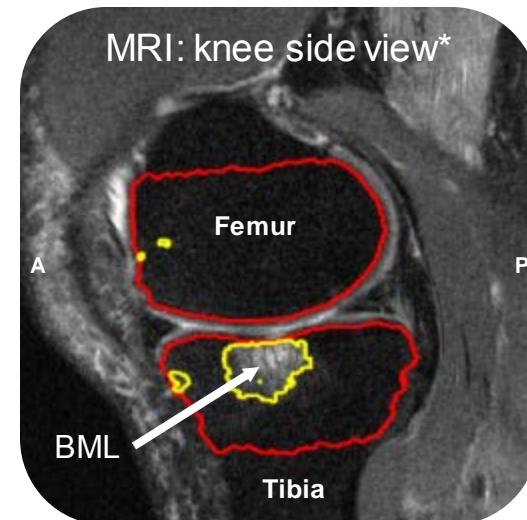
7. Misso G, et al. *Cancer Biol Ther*. 2012;13:1491-500.



# Chronic Pain:

## Knee OA with BMLs Overview

- Bone marrow lesions (BMLs) on MRI are associated with pain in knee osteoarthritis (OA).<sup>1</sup>
- BMLs are regions of increased bone turnover, and reduced mineral density.<sup>2,3</sup>
- Zoledronic acid inhibits bone resorption and increases mineral density.
- Phase 3 trial initiated based on positive Phase 2 results with IV zoledronic acid.
- Phase 3 interim analysis: IDMC recommended continuation to full enrollment



7M patients  
in the U.S.<sup>4-9</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-02 (DZT)	Knee OA with BMLs: SPA Received; Fast Track Granted			Initiated

Abbreviations: DZT = Disodium Zoledronate Tetrahydrate.

\* MRI showing BML in medial tibia from Driban, et al. *Arthritis Res Ther.* 2013;15:R112.

1. Driban JB, et al. *Arthritis Res Ther.* 2013;15:R112.

2. Hunter DJ, et al. *Arthritis Res Ther.* 2009;11:R11.

3. Kazakia GJ, et al. *Osteoarthritis Cartilage.* 2013;21:94-101.

4. Lawrence RC, et al. *Arthritis Rheum.* 2008;58:26-35.

5. Zhang Y, Jordan. *JM Clin Geriatr Med.* 2010;26:355-69.

6. Tanamas SK, et al. *Rheumatology.* 2010;49:2413-19.

7. Guermazi A, et al. *BMJ.* 2012;345:e5339.

8. Jensen OK, et al. *Spine J.* Feb. 14, 2014;pii:S1529-9430(14)00214-9.

9. U.S. Census Bureau, Population April 1, 2010 to July 1, 2013.

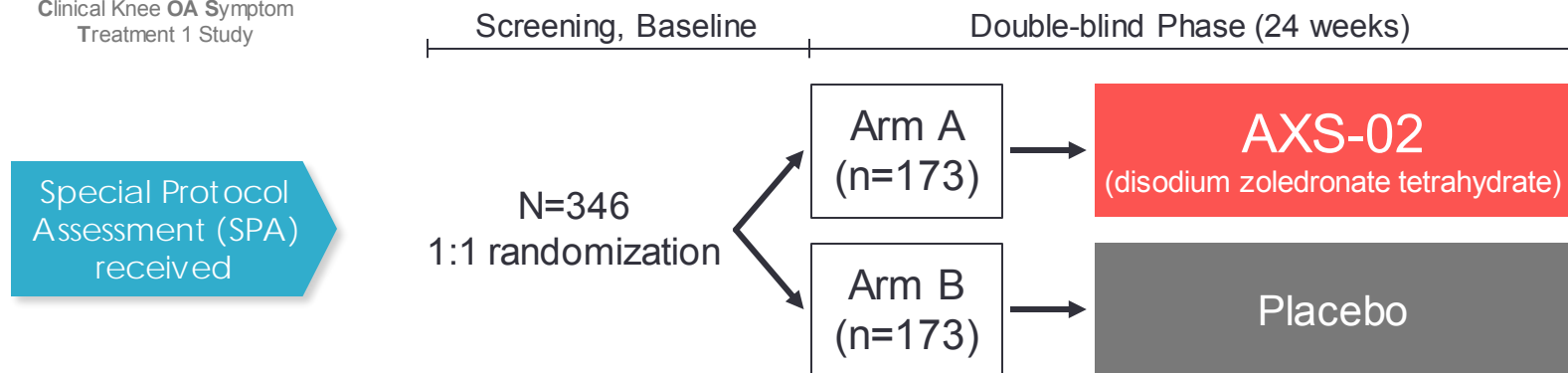
# Chronic Pain:

## Knee OA with BMLs Phase 3 Design

# coast-1

Clinical Knee OA Symptom  
Treatment 1 Study

A Phase 3 trial to assess the efficacy and safety of  
**AXS-02** in the treatment of pain of knee OA associated with BMLs.

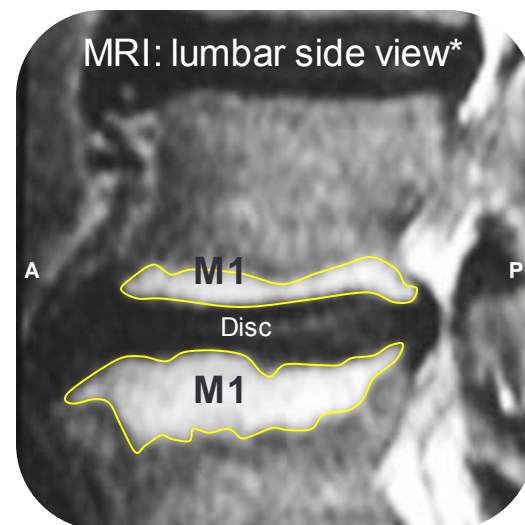


- **Primary Endpoint:** Change in pain intensity from baseline to week 24, measured using the 0-10 Numerical Rating Scale (NRS).
- **Key Inclusion Criteria:**
  - Male at least 50 years of age or postmenopausal female, with knee OA and BMLs
  - Moderate or worse knee pain
- **Dosage:** Once per week for six weeks; no drug for remainder of double-blind phase.

# Chronic Pain:

## CLBP with MCs Overview

- Modic changes (MCs) type 1 (M1) on MRI are associated with chronic low back pain (CLBP).<sup>1</sup>
- Increased bone turnover on bone scan is seen in M1 lesions.<sup>2</sup>
- Increased pro-inflammatory cytokines, and vascular density seen in M1 lesions.<sup>3</sup>
- Zoledronic acid reduces bone turnover, suppresses the production of inflammatory mediators, and is anti-angiogenic.
- Phase 2 results: Zoledronic acid reduced pain in patients with CLBP.
- FDA clearance received for IND for Phase 3 trial – initiation planned following readouts from CREATE-1 and STRIDE-1.
- Issued U.S. patents: protection into 2034 – uses of oral zoledronic acid for low back pain.



1.6M patients  
in the U.S.<sup>4-7</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-02 (DZT)	CLBP with MCs			

Abbreviations: DZT = Disodium Zoledronate Tetrahydrate.

\* MRI showing modic type 1 lesions from Luoma K, et al. *European Congress of Radiology (ECR)*. 2014;Poster B-0458.

1. Zhang Y, et al. *Eur Spine J*. 2008;17:1289-1299.

2. Järvinen J, et al. *Spine: ISSLS Society Meeting Abstracts*. Oct. 2011;Volume Suppl, Abstract GP127.

3. Rahme R, Moussa R. *Am J Neuroradiol*. 2008;29:838-42.

4. Lawrence RC, et al. *Arthritis Rheum*. 2008;58:26-35.

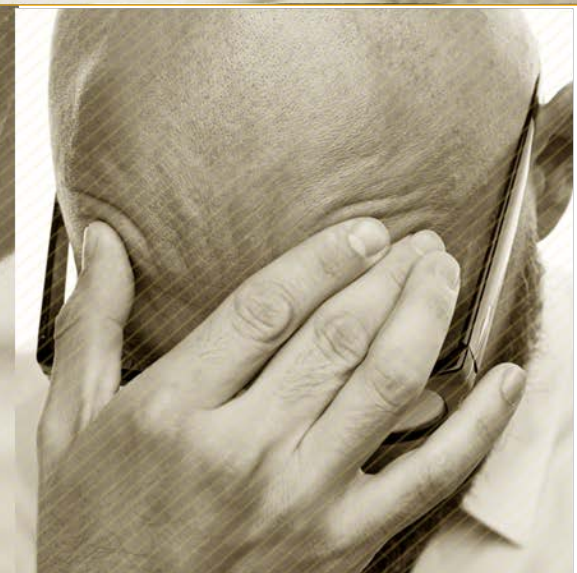
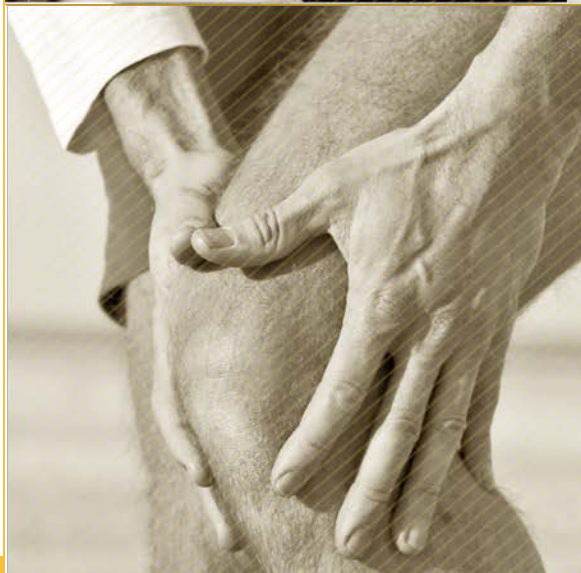
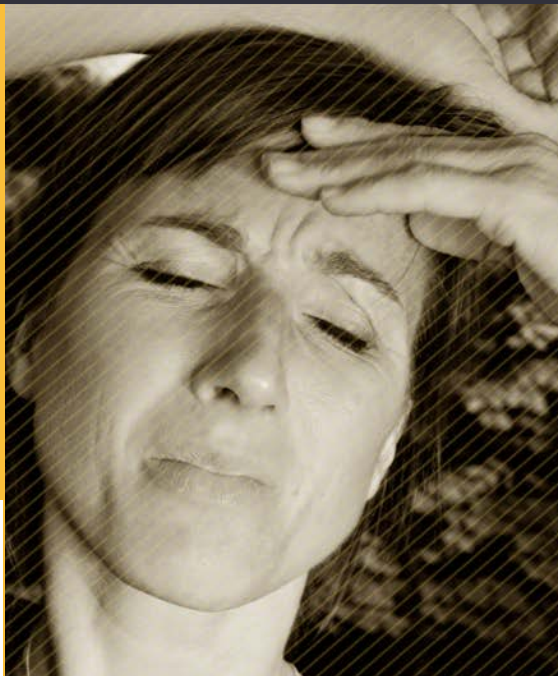
5. Zhang Y, Jordan. *JM Clin Geriatr Med*. 2010;26:355-69.

6. Jensen OK, et al. *Spine J*. Feb. 14, 2014;pii:S1529-9430(14)00214-9. 7. U.S. Census Bureau, Population April 1, 2010 to July 1, 2013.

# MoSEIC™ Meloxicam

Novel therapies:

- AXS-07 – Migraine
- AXS-06 – OA and RA



# Migraine, OA and RA:

## MoSEIC™ Meloxicam Overview

- MoSEIC meloxicam is a potent, oral, rapidly-absorbed, once-daily, non-opioid, COX-2 preferential, pain therapeutic.
- Standard meloxicam has an extended  $T_{\max}$  (4-6 hours) which delays its onset of action.<sup>1,2</sup>
- Axsome's MoSEIC (Molecular Solubility Enhanced Inclusion Complex) technology substantially increases the rate of absorption of meloxicam while maintaining its approximately 20-hour half-life.
- Phase 1 results: 9 times faster  $T_{\max}$ , higher  $C_{\max}$  and similar half-life, compared to Mobic®.
- Potential utility for migraine, and the signs and symptoms of OA and RA.
- AXS-07 is a fixed-dose combination of MoSEIC meloxicam and rizatriptan.
- AXS-06 is a fixed-dose combination of MoSEIC meloxicam and esomeprazole (to reduce risk of NSAID-associated ulcers).

### IP Overview

- 1 issued patent – protection through 2036.
- Pharmacokinetic patents
- 14 pending U.S. and international applications.

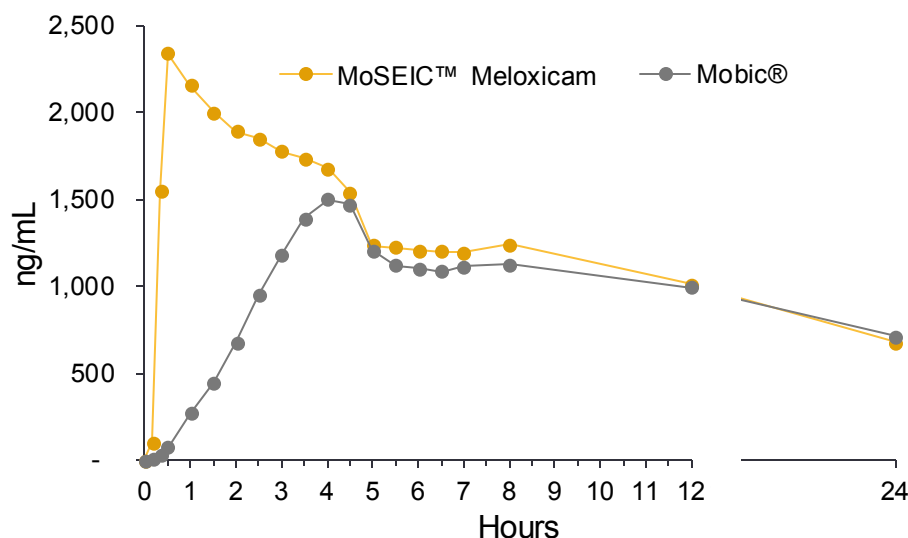
1. Mobic® (meloxicam) FDA Package Insert.

2. Euler-Ziegler et al., *Inflamm Res* 50, Supplement 1 (2001) S5–S9.

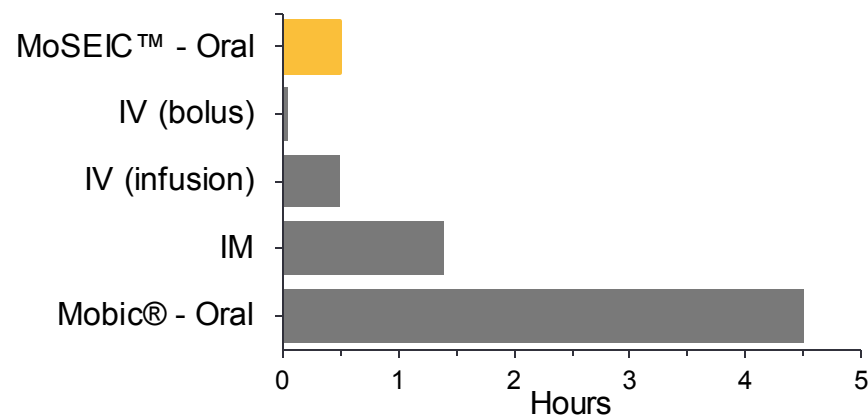
# Migraine, OA and RA:

## MoSEIC™ Meloxicam Phase 1 Results

Mean Meloxicam Concentrations



Meloxicam  $T_{max}$  after 15 mg Dose



- MoSEIC meloxicam  $T_{max}$  9 times faster than Mobic® (0.5 hour versus 4.5 hours, respectively,  $p < 0.0001$ ).
- Therapeutic plasma levels achieved within 15 minutes of oral dosing of MoSEIC meloxicam.
- MoSEIC meloxicam had higher mean  $C_{max}$  ( $p = 0.0018$ ), faster time to therapeutic plasma concentration ( $p < 0.0001$ ), and time to half-maximal plasma concentration ( $p < 0.0001$ ) as compared to Mobic®.
- Terminal half-lives were approximately 20 hours for MoSEIC meloxicam and 22 hours for Mobic®.

Sources: Axsome data on file. IV and IM data from Euler-Ziegler et al., *Inflamm Res* 50, Supplement 1 (2001) S5–S9.



# AXS-07: MoSEIC™ Meloxicam + Rizatriptan for Migraine

- Meloxicam is a new molecule for migraine—not currently approved or used for this indication due to prolonged  $T_{\max}$
- MoSEIC delivery enables its use in abortive treatment of migraine
  - Rapid  $T_{\max}$  of MoSEIC meloxicam is ideal for migraine treatment
  - Extended half-life of MoSEIC meloxicam should lead to lower symptom recurrence
- AXS-07 combines unique PK of MoSEIC meloxicam with proven efficacy of rizatriptan
- FDA Pre-IND written guidance received
- Phase 3 initiation anticipated in 2018



37M patients  
in the U.S.<sup>1</sup>

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-07 (MoSEIC™ Mx + Riz)	Migraine			

Abbreviations: Mx = Meloxicam; Riz = Rizatriptan.

1. Fleis JR, et al., *Summary health statistics for U.S. adults: National Health Interview Survey, 2009. National Center for Health Statistics. Vital Health Stat 10(249). 2010.*

# AXS-07:

## Differentiated Clinical Profile for Migraine

- **Rapid absorption and onset of action**
  - Based on rapid absorption of MoSEIC meloxicam and expected additive effect of AXS-07 components
- **Strong and consistent pain relief**
  - Potential for superior efficacy as compared to current treatments based on expected additive effect of AXS-07 components
- **Sustained pain relief**
  - Based on extended MoSEIC meloxicam half-life and expected additive effect of AXS-07 components
- **Pharmacoeconomic benefits**
  - Potentially superior efficacy expected to result in reduced use of medication and medical services, reduced absenteeism and loss of productivity

# AXS-06:

## MoSEIC™ Meloxicam + Esomeprazole for OA and RA

- AXS-06 is a fixed-dose combination of MoSEIC™ meloxicam and esomeprazole
- Being developed to treat OA and RA, and to reduce the risk of NSAID-associated upper GI ulcers
- Potentially best-in-class NSAID profile:
  - Oral administration with IV-like onset of action
  - Long half-life for sustained effect and once-daily dosing
  - Improved GI safety from esomeprazole component
- Positive Phase 1 results: therapeutic meloxicam concentrations within 15 mins, gastroprotective esomeprazole concentrations
- FDA Pre-IND written guidance received
- AXS-06 is Phase 3-ready

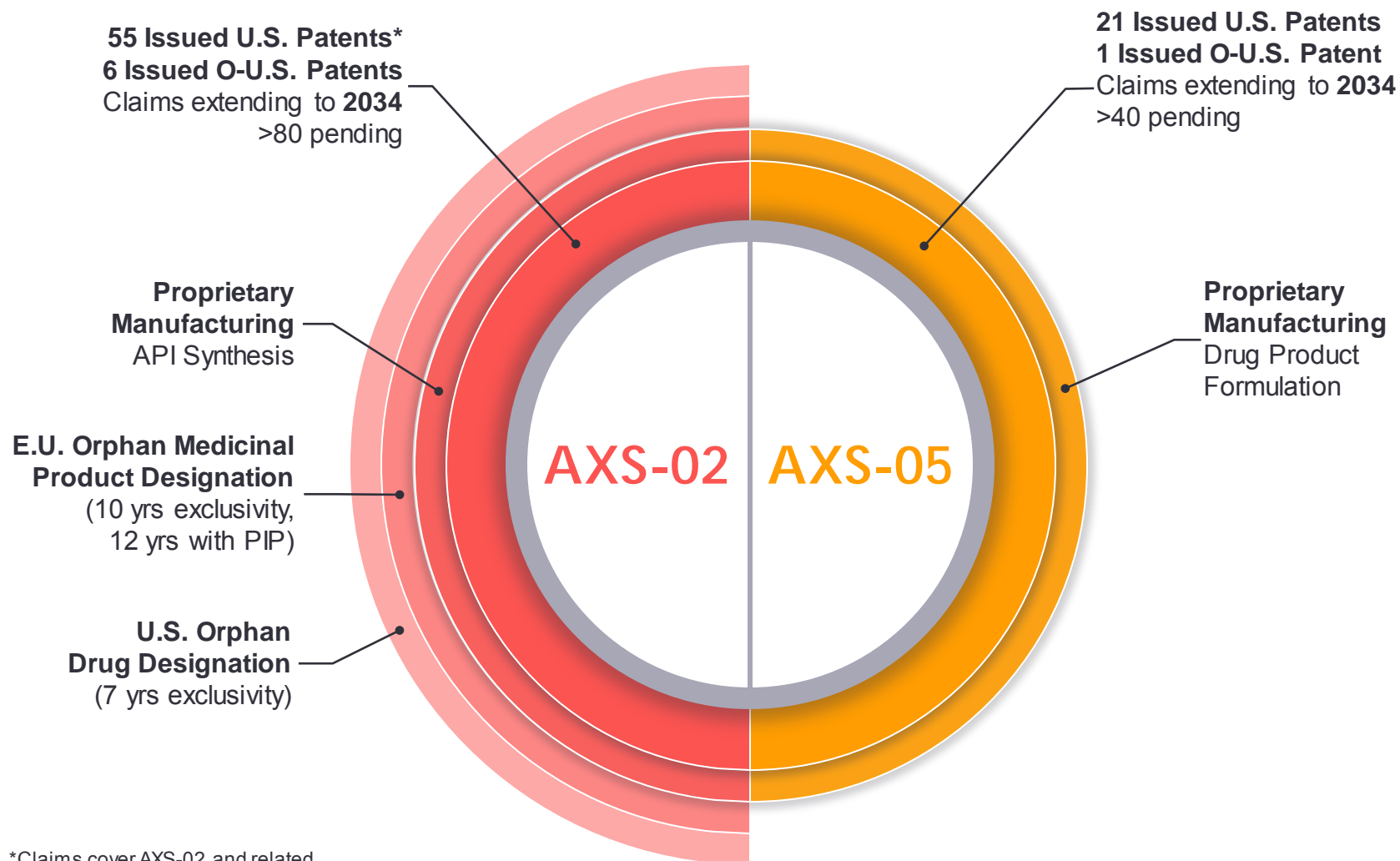


**120M** NSAID TRx  
per year  
in the U.S.

Product Candidate	Preclinical	Phase 1	Phase 2	Phase 3
AXS-06 (MoSEIC™ Mx + Eso)	OA and RA			Phase 3 ready

Abbreviations: Eso = Esomeprazole; Mx = Meloxicam; OA = Osteoarthritis; RA = Rheumatoid Arthritis.

# Barriers to Entry



\*Claims cover AXS-02 and related substances and disease indications.

# Our Team

## Management

Herriot Tabuteau, MD  
**Founder & CEO**

John Golubieski, MBA  
**CFO**

Cedric O’Gorman, MD, MBA  
**SVP, Clinical Development & Medical Affairs**

Mark Jacobson, MA  
**SVP, Operations**

Robert Niecestro, PhD  
**VP, Clinical & Regulatory**



**AXSOME THERAPEUTICS**

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## Board of Directors

Roger Jeffs, PhD  
 Former President, Co-CEO, Director  
**United Therapeutics Corp.**  
 Prior positions at Amgen and Burroughs Wellcome

Myrtle Potter  
 Former President, COO  
**Genentech**  
 Prior positions at Bristol-Myers Squibb and Merck

Mark Saad  
 Former CFO  
**Bird Rock Bio, Inc.**  
 Former COO of the Global Healthcare Group at UBS

Mark Coleman, MD  
 Medical Director  
**National Spine and Pain Centers**  
 Diplomat of the American Board of Anesthesiology

Herriot Tabuteau, MD  
 Chairman

# Key Financial Information

	As of September 30, 2017 <sup>1</sup>
Cash:	\$40.7 Million
Debt (Face Value) <sup>2</sup> :	\$10.0 Million
Common Shares Outstanding:	25.5 Million
Options and Warrants Outstanding <sup>3</sup> :	4.5 Million

- **Financial guidance:** Cash anticipated to fund operating requirements into the fourth quarter of 2019.

1. Pro-Forma to include the effect of the equity capital financings completed in 4<sup>th</sup> Quarter 2017.

2. Book value of \$10.1 million.

3. Consists of 2.4 million options and 2.1 million warrants.



# Clinical Milestones

Product Candidate	Indication	2017	2018	2019
AXS-05 (DM + BUP)	<b>TRD</b>	✓ <b>Fast Track</b> designation	● <b>STRIDE-1</b> top-line results (2H 2018/1H 2019)	
	<b>AD Agitation</b>	✓ <b>Fast Track</b> designation ✓ <b>Ph 2/3</b> trial start		● <b>ADVANCE-1</b> top-line results (2H 2019/1H 2020)
	<b>Smoking Cessation</b>	✓ <b>Duke University</b> collaboration	● <b>Ph 2</b> trial start (1H 2018)	
AXS-02 (DZT)	<b>Knee OA</b>		✓ <b>COAST-1</b> interim analysis	
	<b>CLBP</b>	✓ <b>Ph 3 IND</b> FDA clearance		
AXS-07 (MoSEIC™ Mx + Riz)	<b>Migraine</b>	✓ <b>Ph 3</b> FDA written guidance	● <b>Ph 3</b> trial start	● <b>Ph 3</b> top-line results
AXS-06 (MoSEIC™ Mx + Eso)	<b>OA and RA</b>	✓ <b>Ph 1</b> trial results ✓ <b>Ph 3</b> FDA written guidance		

Abbreviations: AD= Alzheimer's Disease; BUP = Bupropion; CLBP = Chronic Low Back Pain; DM = Dextromethorphan; DZT = Disodium Zoledronate Tetrahydrate; Eso = Esomeprazole; ; Mx = Meloxicam; OA = Osteoarthritis; RA = Rheumatoid Arthritis; Riz = Rizatriptan; TRD = Treatment Resistant Depression.

✓ Accomplished milestone.

● Upcoming milestone.

# AXSOME

## THERAPEUTICS

Thank you.

For more information, please contact

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