

# 34<sup>th</sup> Annual J.P. Morgan Healthcare Conference

January 11, 2016



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SCIENCE.™**  
ACORDA  
THERAPEUTICS

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# Improving Lives of People with Neurological Diseases



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**ACORDA**  
THERAPY



**Multiple Sclerosis**

**Parkinson's disease**

**Post-Stroke Walking Deficits**

**Epilepsy**

**Migraine**

# 2015 Achievements



## **AMPYRA 2015 Growth\***

- Net sales of \$436 Million
- Net sales growth of 19%



## **rHlgM22 Safety Data in MS**

- Positive Phase 1 safety data
- Second Phase 1 enrolling in acute MS relapses



## **Advanced Late Stage Programs**

- CVT-301 in Parkinson's disease
- PLUMIAZ in epilepsy
- Dalfampridine in post-stroke



## **Initiated CVT-427 Program**

- Completed Phase 1 migraine study



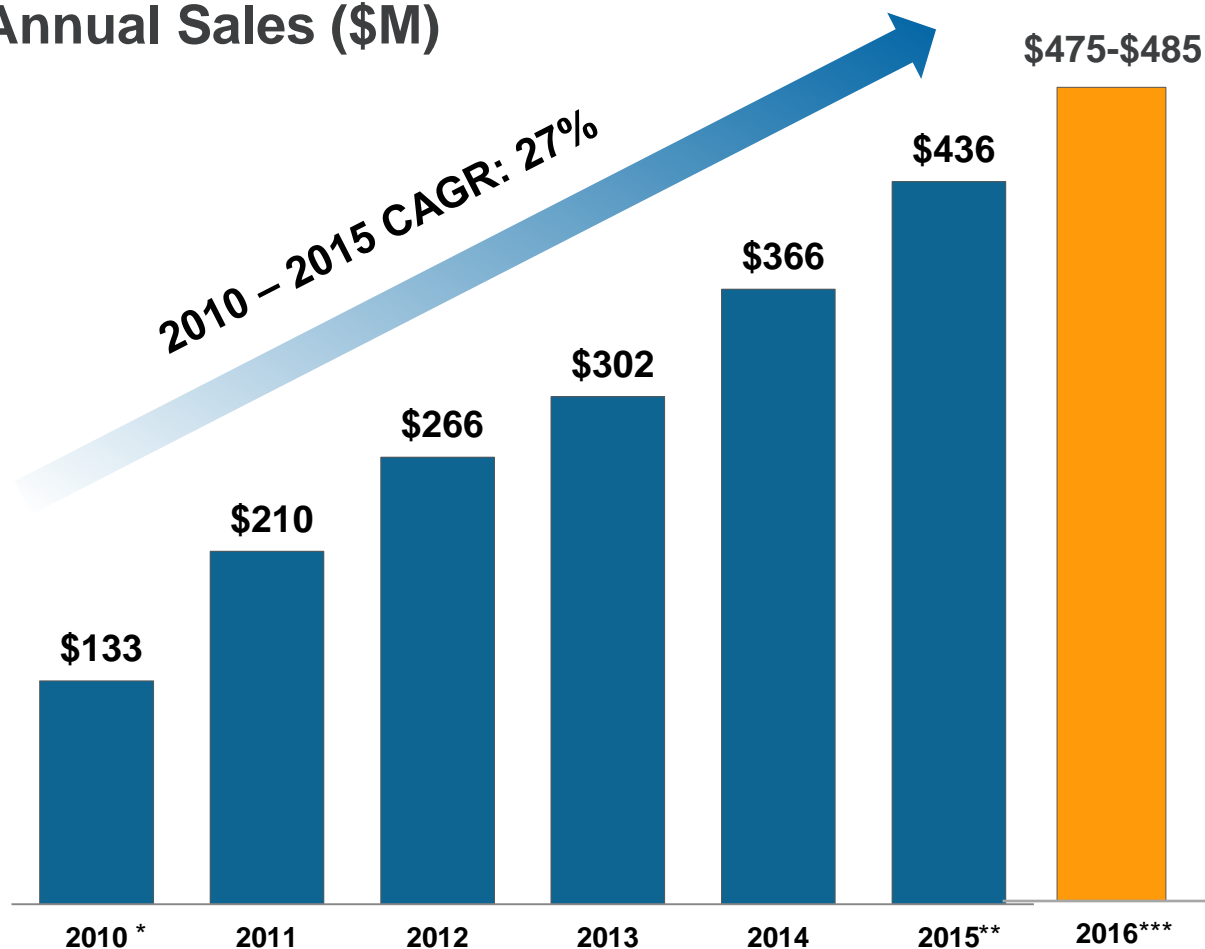
## **IP Defense**

- 3 ANDA filer settlements
- PTAB denial of 2 IPR petitions

# AMPYRA (dalfampridine) for Multiple Sclerosis

## Lead Commercial Asset

### AMPYRA Annual Sales (\$M)



# Clinical Pipeline

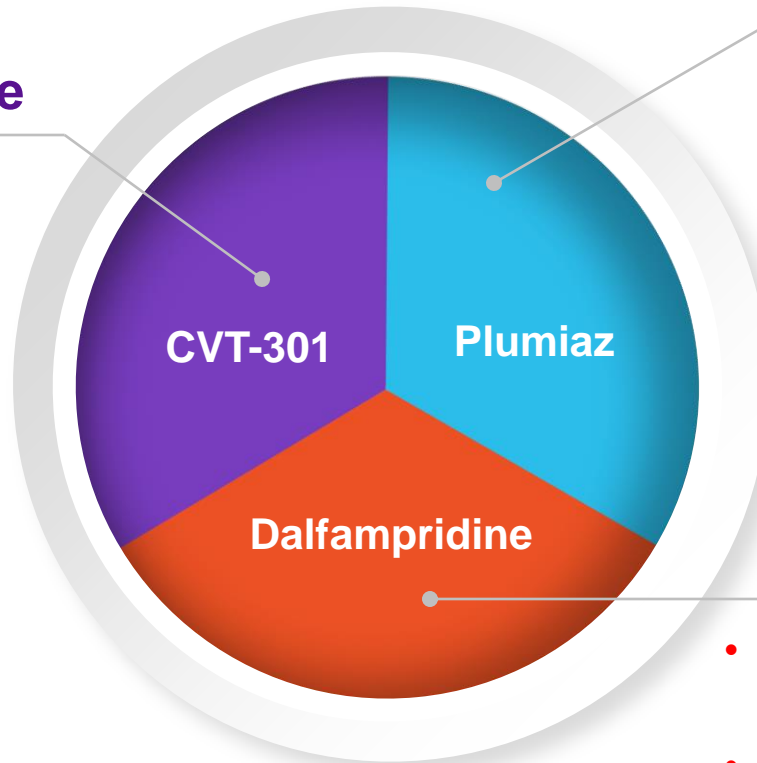
THERAPY	INDICATION	PHASE 1	PHASE 2	PHASE 3
CVT-301	Parkinson's Disease			
PLUMIAZ™ (diazepam) Nasal Spray	Seizure Clusters			
DALFAMPRIDINE	Chronic Post-Stroke Walking Deficits			
CVT-427	Migraine			
rHlgM22	MS			

# Late Stage Pipeline

## Targeting Large Unmet Medical Needs

### Parkinson's Disease

- ~350,000 patients in US with OFF periods
- >\$500M US peak sales



### Epilepsy

- ~175,000 patients in US with seizure clusters
- > \$200M US peak sales

### Post-Stroke Walking Deficits

- ~3.5 million stroke survivors in US with mobility issues
- 3 QD prototypes currently in human PK studies





# CVT-301 in Parkinson's Disease

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# CVT-301 Overview

## Phase 3 Study Ongoing



### Inhaled Levodopa

- Self-administered, inhaled medication
- Utilizes ARCUS® technology to deliver specific doses of dry powder L-dopa



### Positive Phase 2b Efficacy Data

- Results show potential to rapidly treat off periods
- Separation vs. placebo observed at 10 minutes after dosing and was durable for at least an hour
- Clinically important reductions in UPDRS Part III at both tested doses



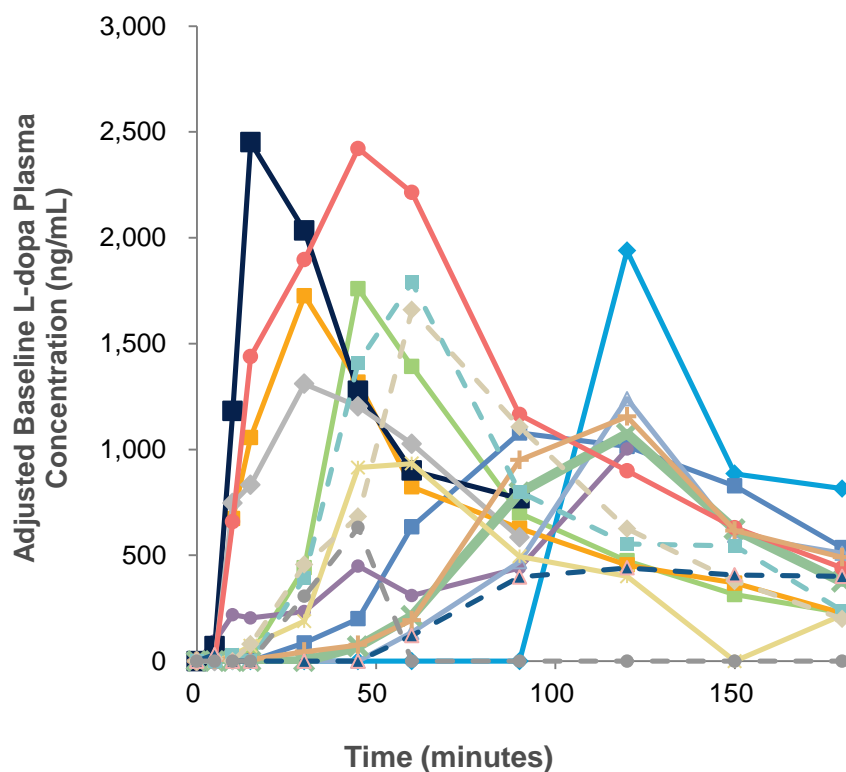
### Phase 2b Safety Profile

- No treatment-associated AEs on lung function
- No serious AEs overall
- Well tolerated; no increase in dyskinesia during at-home use

# L-Dopa Pharmacokinetics

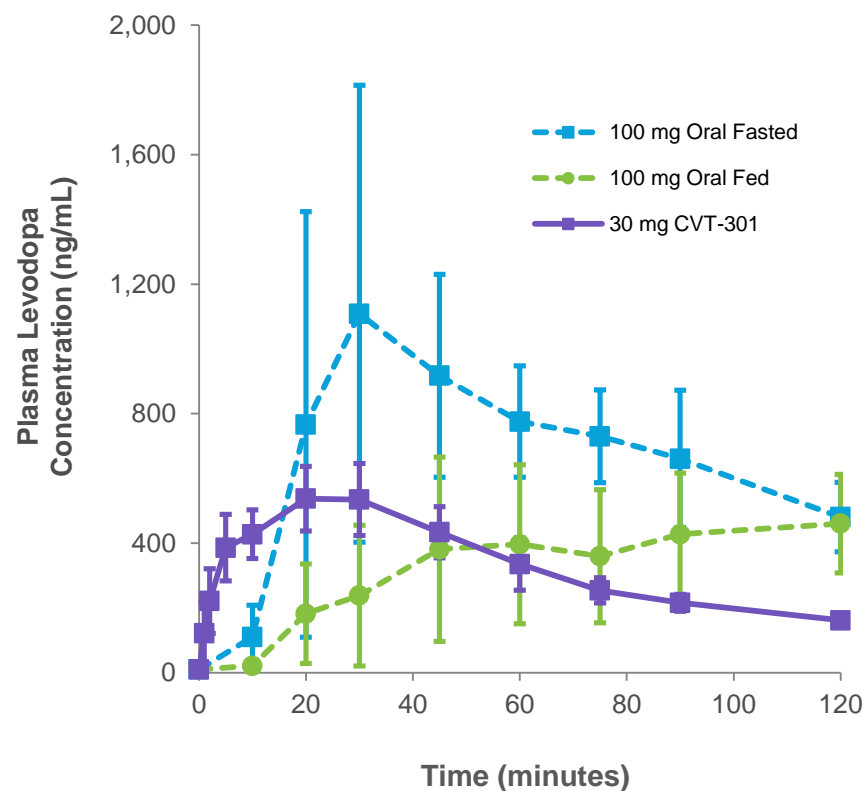
## Current Oral Standard of Care

Data from Phase 2a in fasted PD patients



## CVT-301 Profile

Data from Phase 1 trial in healthy volunteers

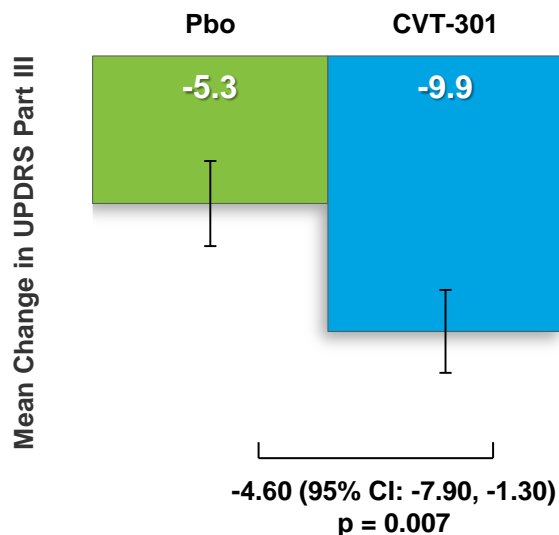


# Phase 2b Study Achieved Primary Endpoint

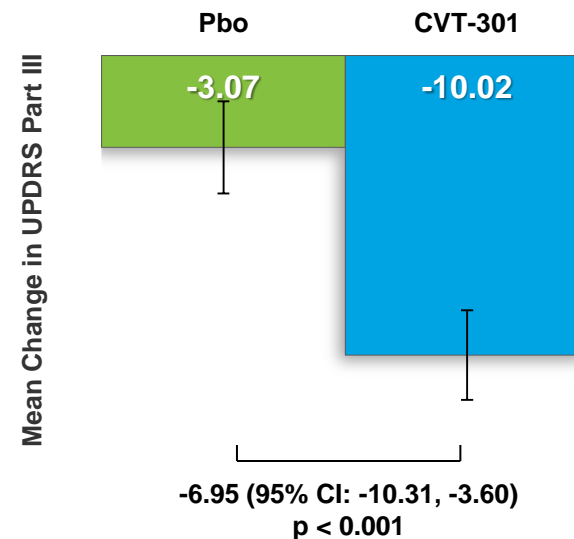
## UPDRS Part III

Clinically important reductions at both tested doses

Visit 4: CVT-301 35mg or Pbo



Visit 6: CVT-301 50mg or Pbo

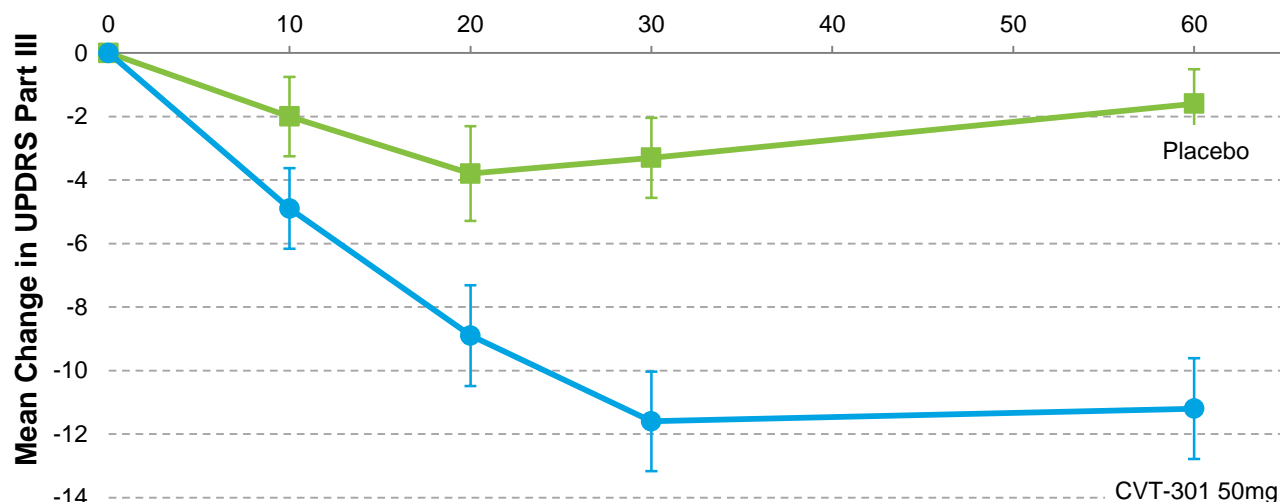


# Phase 2 Data Presented at MDS 2015

## Separation vs. Placebo Observed – 10 Minutes

### Visit 6 – CVT-301 50mg dose

Time (minutes)



#### UPDRS Part III Clinically Important Differences (CID)\*:

2.5pts = Minimal CID

5.2pts = Moderate CID

10.8pts = Large CID

	10 min	20 min	30 min	60 min
Diff vs Pbo Mean (SEM)	-3.56 (1.62)	-5.68 (2.04)	-8.43 (1.90)	-9.59 (1.83)
p-value	0.0309	0.0068	<0.0001	<0.0001

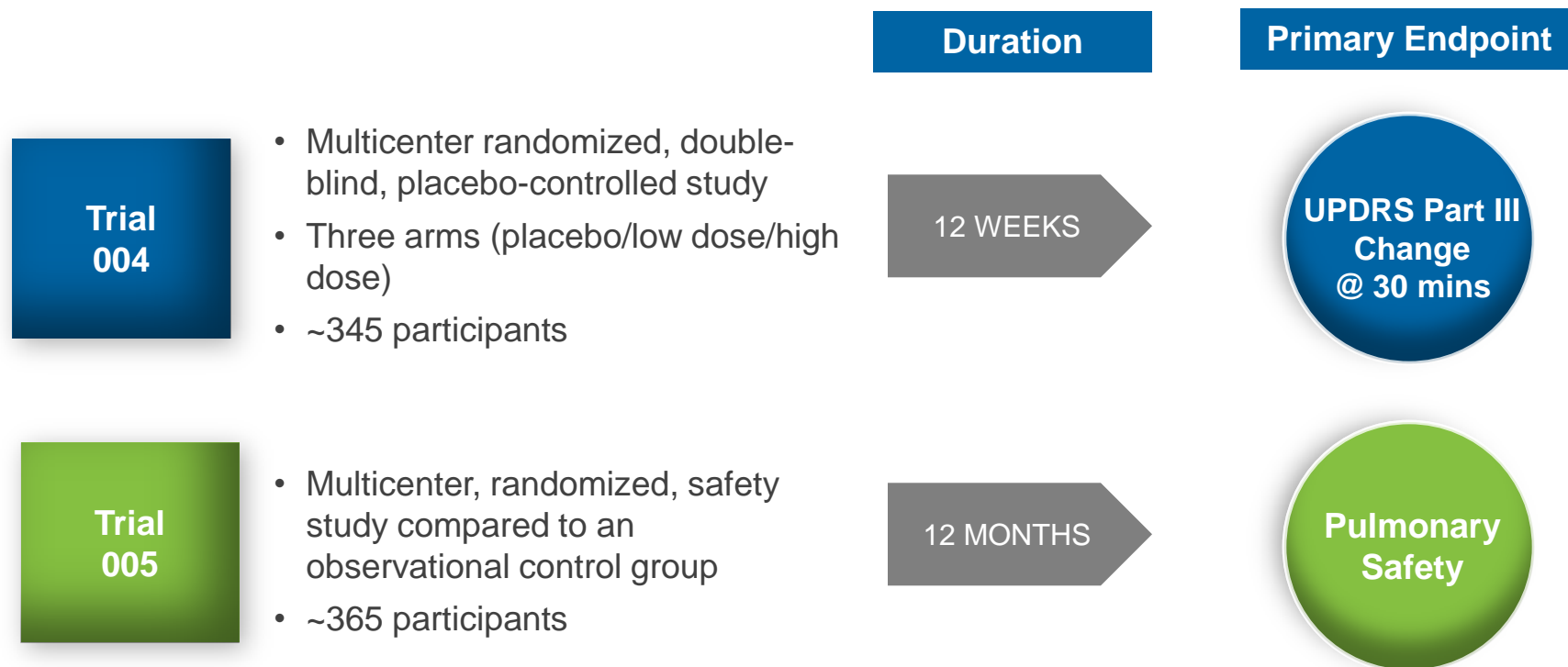


# CVT-301 Phase 2b Safety Profile

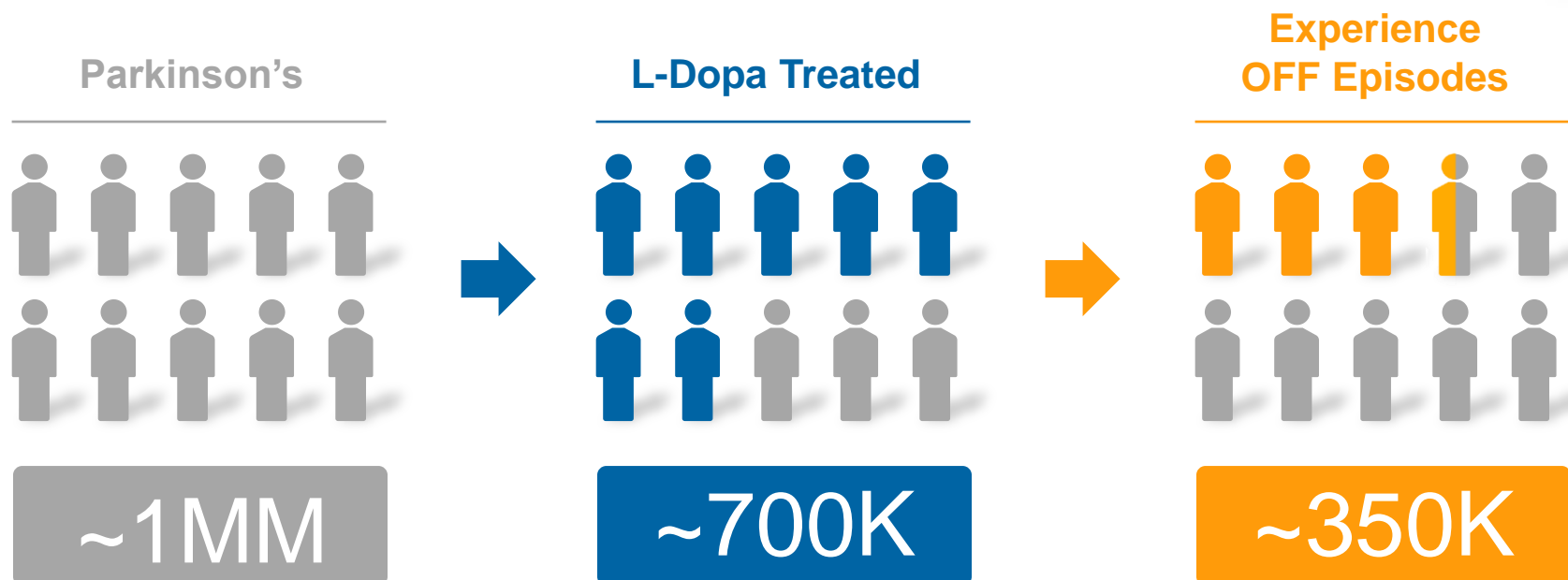
Treatment-Emergent Adverse Event, n (%)	Placebo Group (n=43)	CVT-301 Group (n=43)
Dizziness	2 (5)	3 (7)
Cough	1 (2)	3 (7)
Nausea	0	3 (7)
Headache	2 (5)	2 (5)
Peripheral edema	1 (2)	2 (5)
Anxiety	0	2 (5)
Discolored sputum	0	2 (5)

Source: Poster from MDS June 2015

# Phase 3 Program – Target NDA Filing 1Q 2017



# CVT-301 U.S. Market Opportunity



**Projected U.S. Peak Sales in Excess of \$500 million**



# PLUMIAZ™ (diazepam nasal spray) in Epilepsy

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# PLUMIAZ Development Overview



## Diazepam Nasal Spray for Seizure Clusters

- Unmet need
  - Only approved treatment is rectal gel
- Nasal administration is more practical and socially acceptable



## Three Clinical Studies

- Bioavailability: PLUMIAZ vs Diastat
- Long term safety study
- Dose proportionality study expected to begin 1Q16

# PLUMIAZ: On Track for 1Q17 NDA Refiling

**2.8MM** People with  
Epilepsy (US)

**~175K** Experience  
seizure clusters\*

**Patients continue to  
experience seizure  
clusters even though they  
are on stable AEDs**



**Six month review & orphan status**



**Leverages commercial  
organization**

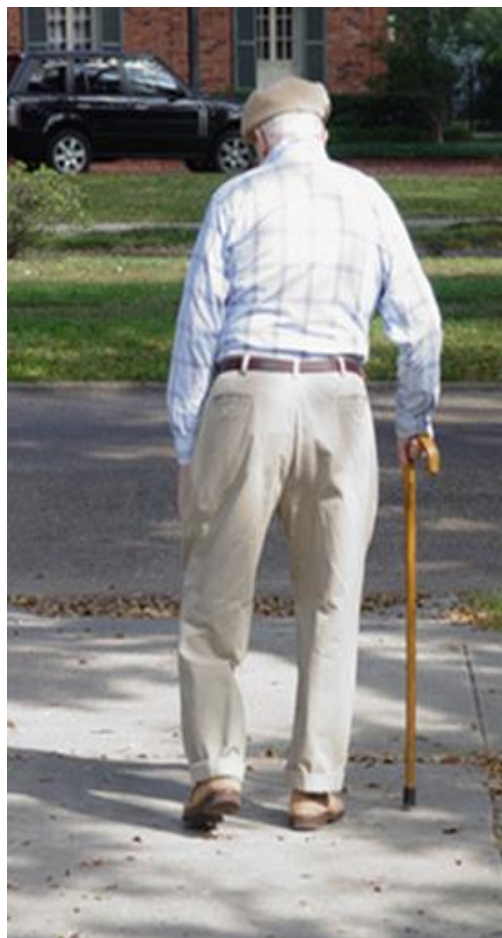


**Potential peak sales of >\$200M**



# Dalfampridine in Chronic Post-Stroke Walking Deficits (PSWD)

# Dalfampridine in Development for PSWD



## No drug therapy indicated for people with chronic PSWD

- ~7 million people in U.S. have had a stroke
- ~3.5 million have mobility issues



## Successful dalfampridine proof-of-concept study



## Enrollment in Phase 3 study ongoing; interim analysis in 2016

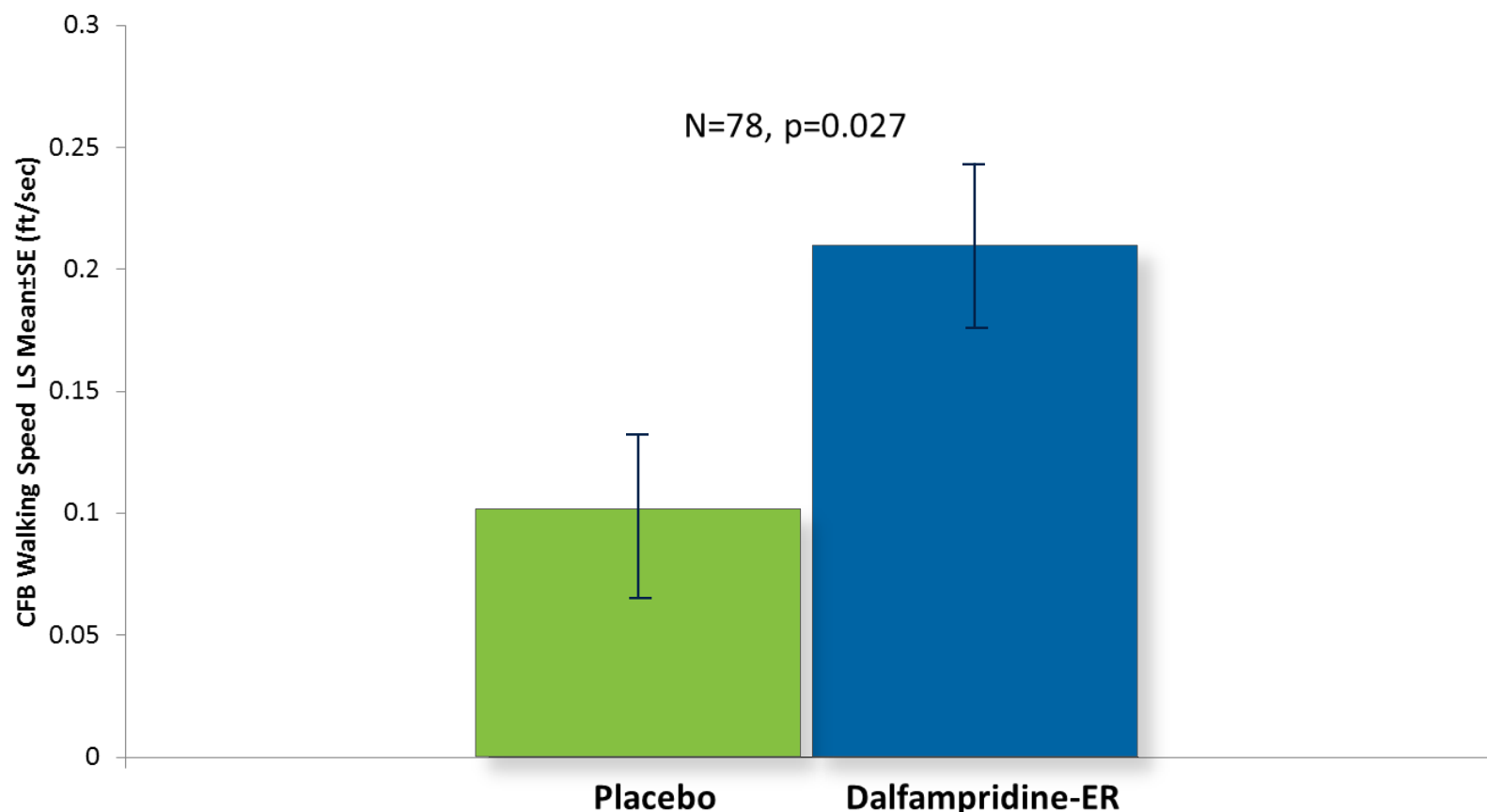


## QD formulation

- Three prototypes currently in Phase 1 PK studies
- Data expected in 1Q 2016

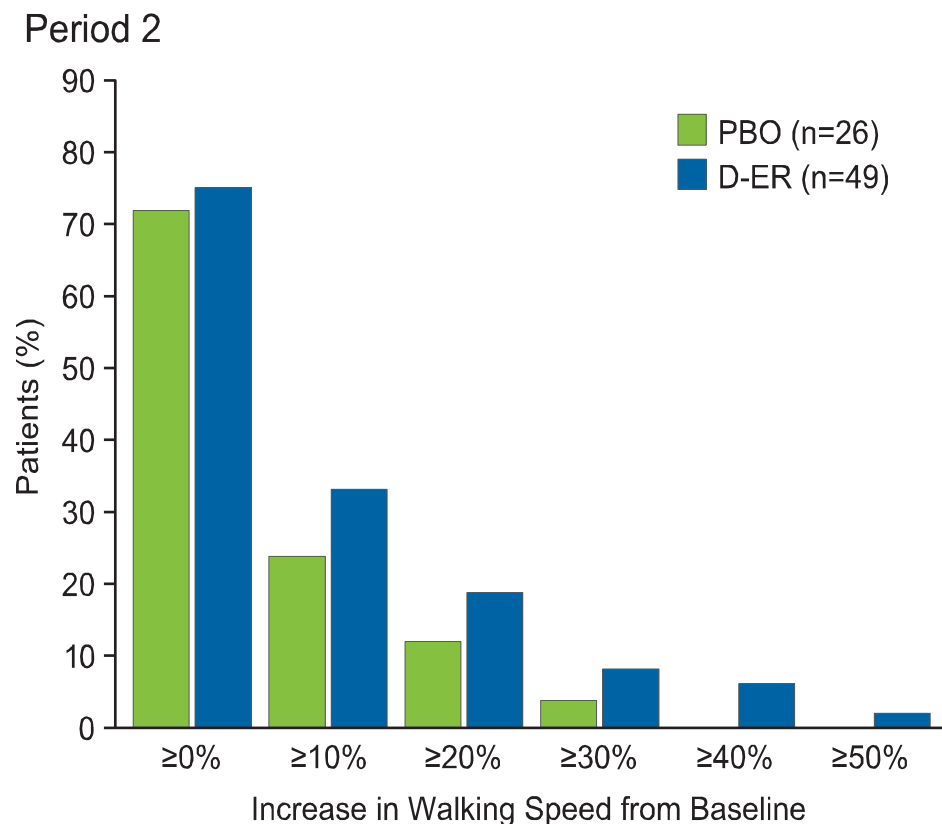
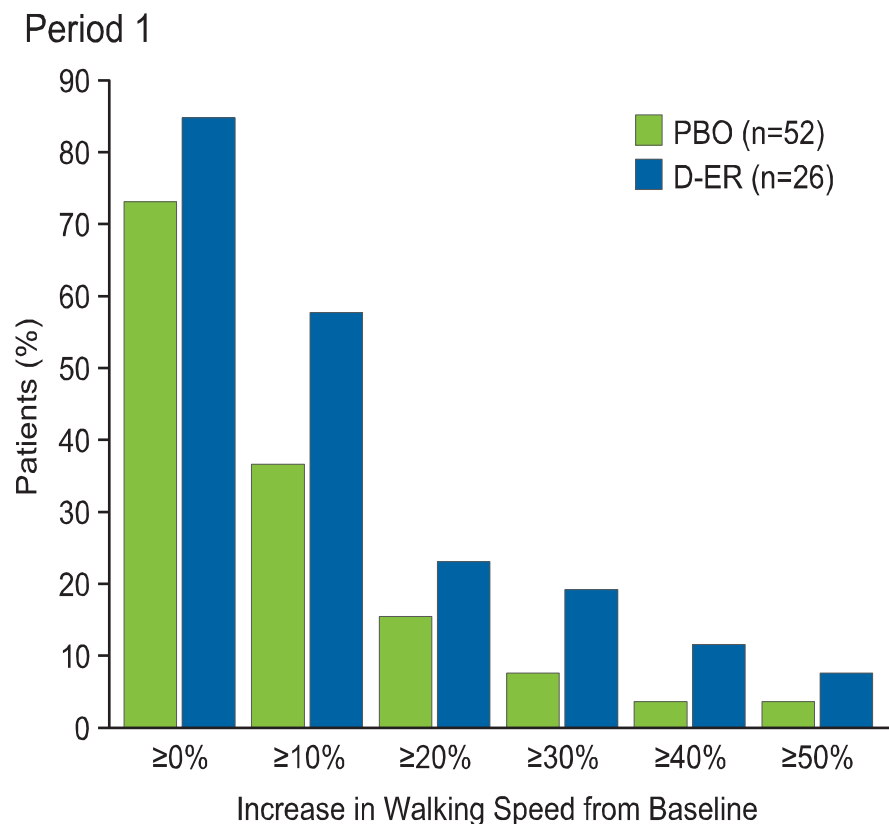


# Phase 2 POC Study: Overall Timed Walk Result



Note: p value from a mixed statistical model with sequence, period, visit and treatment as fixed effects and subject as a random effect.

# Percentages of Patients Who Reached Threshold Change from Baseline in Walking Speed by Period



# Early Stage Pipeline



## CVT-427 (migraine)

- Phase 1 study complete
- Data to be presented at medical meeting



## rHlgM22 (multiple sclerosis)

- Phase 1, single ascending dose study in acute MS relapses currently enrolling patients
- Study completion expected 1H17



## Cimaglermin alfa (heart failure)

- Topline data from second Phase 1 (on clinical hold - Hy's Law case)
- Safety profile consistent with first Phase 1; inconclusive efficacy data
- Data analyses ongoing; plan to meet with FDA to discuss data/evaluate next steps



# 2016 Guidance and Milestones

# 2016 Financial Guidance



**AMPYRA**  
\$475 – \$485  
million\*



**R&D**  
\$165 – \$175  
million



**SG&A**  
\$195 – \$205  
million

# Key Clinical Milestones

Single dose PK/safety update Phase 1 CVT-427 in migraine	1Q 2016
Single dose PK results from QD formulation (dalfampridine)	1Q 2016
Interim analysis of dalfampridine Phase 3 post-stroke trial	3Q 2016
Complete CVT-301 Phase 3 in Parkinson's (LPO)	4Q 2016
File NDA for CVT-301 in Parkinson's	1Q 2017
File NDA for PLUMIAZ for seizure clusters	1Q 2017
Complete Phase 1 M22 in acute MS relapses	1H 2017



# 2016 Priorities

**Advance  
Pipeline**

**Continue to  
Grow Ampyra**

**Business  
Development**

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